



THANK YOU to our Speakers, Panelists and Moderators



David Sibbald,
Chief Executive Officer



Rodrigo Barnes,
Chief Technology Officer



Gideon Giacomelli,
FAIR Product Owner



Laura Shishodia,
Workspaces Product
Owner



Kara Lasater,
Project Manager

Aridhia

Aridhia's mission is to enable large scale data science programs for biomedical research and innovation. Our platform provisions relevant datasets, data management pipelines and analysis methods to scale biomedical research programs within healthcare systems. The company has worked over the past eight years to develop, release and support a Digital Research Environment (DRE) -a hybrid PaaS/SaaS platform to address many of the common challenges associated with achieving biomedical data science scale and sustainability. Our proposed solution helps to deliver those outcomes and aligns to best practice principles from the Global Alliance for Genomics and Health (GA4GH) Framework for Responsible Sharing of Genomic and Health Related Data, the FAIR guiding principles for scientific data management and the extensive use of open-source software for reproducible analytics.

Karim Azer, Ph.D., is head of Systems Biology & Discovery at Axcella, where he leads data and discovery sciences. Prior to Axcella, Karim led quantitative sciences efforts at the Bill & Melinda Gates Medical Research Institute. His work focused on leveraging the spectrum of computational sciences, including bioinformatics/systems biology, QSP and Pharmacometrics modeling approaches, and data science, to address research and development needs of the institute. Karim's efforts are also dedicated to the development of supportive computational and mathematical approaches and pipelines to enable robust in-silico simulations and visualizations of models and model outputs. Previously at Sanofi, he formed and headed the quantitative systems pharmacology group, supporting programs across several disease areas, including immunology, rare diseases, cardiovascular and oncology. Moreover, as part of the leadership of the integrated pharmacometrics program, he took part in the development and application of pharmacometric disciplines across the development organization, and building bridges across the multiple quantitative disciplines. He also led the development of technical capabilities in support of model development, calibration, qualification and simulation, in collaboration with academic partners and Institutes. He has applied modeling to many discovery and development programs throughout his career in pharma and non-profit and has been involved in associated interactions with regulatory agencies.



Jeff Barrett, Ph.D., F.C.P. is Senior Vice President at C-Path and serves as the lead for its Rare Disease Cure Accelerator-Data and Analytics Platform (RDCA-DAP) initiative. As SVP, Dr. Barrett guides all operational and scientific activities for RDCA-DAP. These activities include continuing outreach and collaboration with the rare disease community to optimize data usability and availability, as well as transforming such data into actionable knowledge to advance drug development for rare and orphan conditions. He was previously Senior Advisor 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. Prior to coming to C-Path, he was Head of Quantitative Sciences at the Bill & Melinda Gates Medical Research Institute. In this role he was responsible for implementing model-based drug development, employing PK/PD modeling, statistics, and clinical trial simulations to advance the discovery and development of new medicines and vaccines. Prior to MRI, he was Vice President, of Translational Informatics at Sanofi Pharmaceuticals. He led various aspects of model-

based decision-making spanning and provided leadership for Sanofi's cloud-based, high-performance computing and "big data" initiatives. Jeff spent more than 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 Philadelphia. He has co-authored over 175 manuscripts, is 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 was a past acting chair of the FDA Advisory Committee for Pharmaceutical Science and Clinical Pharmacology; a voting member of the committee for eight years.

Alexandre Bétourné, Ph.D., Pharm.D., is Scientific Director for the Rare Disease Cures Accelerator-Data and Analytics Platform initiative. Dr. Bétourné works with the RDCA-DAP team to expand its reach into new diseases areas accessing new data and enhancing C-Path's relationships within the rare disease community. Dr. Bétourné holds both a PhD and a PharmD from the University of Toulouse in France. He holds three patents and has written multiple papers that intersect with several different rare disease areas. Before joining C-Path, he led a team of senior U.S. scientists, CMC and regulatory consultants at a small company developing therapies for amyotrophic lateral sclerosis (ALS).



Terry Jo Bichell, Ph.D., worked as a documentary filmmaker in the early days of videotape, then became a public health nurse-midwife after filming a difficult birth in West Africa. When her youngest child, Lou, was diagnosed with Angelman syndrome in 2000, she quickly switched focus to help move bench science into the first clinical trials for Angelman syndrome and to help design natural history studies. Dr. Bichell earned a PhD in neuroscience from Vanderbilt University in 2016 in an effort to find Angelman treatments. Along the way, she studied gene-environment interactions in Huntington disease as well as circadian aspects of Angelman syndrome. After graduating, she was the Founding Director of the Angelman Biomarkers and Outcome Measures Alliance from 2016-2018. Dr. Bichell founded a new non-profit in 2019, COMBINEDBrain (Consortium for Outcome Measures and Biomarkers for Neurodevelopmental Disorders), to assist other rare and ultra-rare non-verbal neurogenetic disorders with clinical trial preparations. She also serves as the Vice Chair of the Tennessee Rare Disease Advisory Council, writes a column for Bionews, and leads a course in Translational Neuroscience at Vanderbilt University.

Amanda J. Borens, M.S., is Executive Director of Data Science at C-Path and has over 20 years of development, analytics and scientific experience in academia, hospital labs, government, as well as healthcare informatics and biotech companies, and brings a wealth of practical experience and leadership to the C-Path Data Collaboration Center. Her early career was focused upon development, support, and implementation of electronic health record (EHR) systems in hospitals around the world. After graduate education in life science, Ms. Borens shifted to research and analytics with a geospatial and biostatistical component. Work in federal, state, and local governments included advanced spatial analyses of Big Data sources, where she developed new technologies and algorithms to facilitate answers to scientific questions. This experience in the emerging field of data science led to work in medical diagnostics, where Ms. Borens was part of a device development team that successfully achieved FDA clearance and CE Mark. Throughout all this work, there was a common thread of providing Big Data solutions to solve scientific problems and acting as the interface between life scientists and technology innovation



Megan Cala, Ph.D., is a postdoctoral fellow of pharmacometrics within the Quantitative Medicine Program at the C-Path. She is a chemical engineer by training from the University of Pittsburgh where her research was focused on developing a multiphase and multiscale computational model of blood coagulation. At C-Path, she is developing quantitative solutions such as disease progression models, placebo response models, and model-based clinical trial simulators using pharmacometrics methods to advance drug development. Her efforts are primarily focused on developing models and simulations to describe and quantify the biological variability of disease progression in Friedreich's Ataxia and bronchopulmonary dysplasia. She also provides quantitative analytical expertise to support the development of the Rare Disease Cures Accelerator Data and Analytics Platform (RDCA-DAP).

Michelle Campbell, Ph.D., is the Sr. Clinical Analyst for Stakeholder Engagement and Clinical Outcomes for the Office of Neuroscience (ONs), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA). 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 inpatient-focused drug development and the use of patient experience data in the regulatory setting. Prior to joining the FDA, Dr. Campbell spent over 10 years in the academic-clinical setting with research experience including the use of both qualitative and quantitative methods to develop instruments, surveys, program evaluation and the application of various study designs including clinical trials. 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.





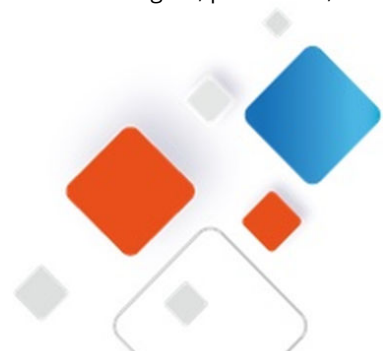
Arthur Leonard Caplan, Ph.D., is a Drs. William F. and Virginia Connolly Mitty Professor and founding head of the Division of Medical Ethics at NYU Grossman School of Medicine in New York City. Prior to starting at NYU, Dr. Caplan was the Sidney D. Caplan Professor of Bioethics at the University of Pennsylvania Perelman School of Medicine in Philadelphia, where he created the Center for Bioethics and the Department of Medical Ethics. He has also taught at the University of Minnesota, where he founded the Center for Biomedical Ethics; the University of Pittsburgh; and Columbia University. He received his PhD from Columbia University and has served on several national and international committees including as chair of the National Cancer Institute Biobanking Ethics Working Group; chair of the Advisory Committee to the United Nations on Human Cloning; and chair of the Advisory Committee to the Department of Health and Human Services on Blood Safety and Availability.

Dr. Caplan has served since 2015 as a chair of the Compassionate Use Advisory Committees (CompAC), independent groups of internationally recognized medical experts, bioethicists, and patient representatives that advise Johnson & Johnson's Janssen Pharmaceuticals on requests for compassionate use of its investigational medicines. Dr. Caplan is the recipient of many awards and honors including the McGovern Medal of the American Medical Writers Association and the Franklin Award from the City of Philadelphia. He was a USA Today 2001 "Person of the Year" and was described as one of the ten most influential people in science by Discover magazine in 2008. He has also been honored as one of the fifty most influential people in American health care by Modern Health Care magazine, one of the ten most influential people in America in biotechnology by the National Journal, one of the ten most influential people in the ethics of biotechnology by the editors of Nature Biotechnology, and one of the 100 most influential people in biotechnology by Scientific American magazine. In 2016, the National Organization for Rare Disorders (NORD) honored him with its Rare Impact Award; that year he also received the Food and Drug Law Institute's Distinguished Service Leadership Award and the American Society for Bioethics and Humanities' Lifetime Achievement Award. In 2019, he was honored by the Reagan-Udall Foundation for the FDA with its Innovation Award. Dr. Caplan holds seven honorary degrees from colleges and medical schools.

Joanne M. Donovan, M.D., Ph.D., has been Chief Medical Officer at Edgewise Therapeutics, a biotechnology company focused on rare muscle disorders, since April 2021. Dr. Donovan has been deeply involved in clinical development for Duchenne muscular dystrophy for the last decade in her role as Chief Medical Officer and Senior Vice President, Clinical Development at Catabasis Pharmaceuticals. Since 1989, she has been a staff physician at the VA Boston Healthcare System and is currently an Associate Clinical Professor of Medicine at Harvard Medical School. From 1998 to 2011, Dr. Donovan served in positions of increasing responsibility, ultimately as vice president of clinical development, at Genzyme. Dr. Donovan holds a Ph.D. in medical engineering and medical physics from the Massachusetts Institute of Technology, an M.D. from Harvard Medical School and an S.B. from the Massachusetts Institute of Technology. She completed residency training in internal medicine and a fellowship in gastroenterology at the Brigham and Women's Hospital.



Billy Dunn, M.D., is the Director of the Office of Neuroscience at the U.S. Food and Drug Administration's Center for Drug Evaluation and Research. The Office of Neuroscience includes the Division of Anesthesiology, Addiction Medicine, and Pain Medicine, the Division of Neurology I, the Division of Neurology II, the Division of Psychiatry, and the Division of Pharmacology and Toxicology for Neuroscience. The Office of Neuroscience is responsible for the regulation and review of Investigational New Drug (IND) applications and marketing applications for drug and biologic products for the treatment of conditions such as Alzheimer's disease, stroke, Parkinson's disease, Huntington's disease, epilepsy, migraine and other headaches, muscular dystrophy, amyotrophic lateral sclerosis, multiple sclerosis, dementia, narcolepsy, neurological medical countermeasures, traumatic brain injury, inner ear disorders, multiple sclerosis, neuroimmunologic disorders, neuromuscular disorders, neurogenetic and rare neurological disorders, bipolar disorder, schizophrenia, schizoaffective disorder, major depressive disorder, attention deficit hyperactivity disorder, obsessive-compulsive disorder, panic attacks, posttraumatic stress disorder, generalized anxiety disorder, autism spectrum disorder, insomnia and sleep disorders, acute pain, chronic pain, addiction, and patient comfort and ease of treatment in surgical, procedural, and critical care settings.





Jennifer Farmer is the Chief Executive Officer of the Friedreich's Ataxia Research Alliance (FARA). Jennifer has a Master's degree in Genetic Counseling and prior to joining FARA she worked at the University of Pennsylvania as a genetic counselor, clinical research coordinator and Director of the Division of Medical Genetics. As a genetic counselor, Jennifer developed a special interest in neurogenetic conditions and then went on to establish and coordinate clinical and research programs for individuals and families diagnosed with Friedreich Ataxia (FA) and other neurodegenerative diseases.

As the Coordinator for the Friedreich Ataxia Clinical Outcome Measure study, a multi-center natural history study in FA, she has been responsible for all activities related to study administration, clinical site training, recruitment and enrollment, data management and publications.

Having established relationships with the families who founded FARA and sharing in their vision to treat and cure FA she joined the organization full time in 2006. Jennifer has led FARA's efforts to establish clinical

research infrastructure and clinical trial readiness, grown the research grant program from funding <1M annually to >6M annually, led efforts to engage bio-pharma industry in FA drug discovery and development, and ensured highly efficient and transparent organizational growth and development. In her current role at FARA as CEO, she helps to carry out the strategic mission of the organization through leading FARA's research and partnership initiatives.

Prashant Goel serves as the Vice President of Information Technology at the National Organization for Rare Disorders (NORD). In this role, he is responsible for all aspects of the department, including people, processes and technology that enable NORD to fulfill its mission in serving the rare disease community.

An accomplished leader with over 25 years of experience, Prashant brings to this role a wealth of knowledge and a well-established background in information technology. Prior to joining NORD, he led the Imperium and data quality teams at Dynata, housing one of the world's largest collections of permissioned first-party data. As Senior Vice President of Technology, Prashant led the data quality team while serving the largest global market research companies with the Imperium suite of products. Before Dynata, Prashant built Imperium from the ground up as Chief Technology Officer. He created several fraud prevention and data quality services for the market research industry that have become the gold standard, processing billions of transactions every year.



Prashant has held several tech leadership positions at United HealthCare, Wipro Systems, and Allied Signal. At United HealthCare, he managed the technology team in charge of member enrollment and renewals. He led the effort to migrate the Oxford Health Plan members to United Healthcare, rewrote parts of the claims system and set up mandated HIPAA EDI transactions.

Prashant has worked with a wide range of technologies, including relational/analytical databases, modern languages/frameworks, cloud technologies, web/mobile development, and machine learning/AI. He has direct experience with a wide range of compliance and regulatory requirements such as HIPAA, PHI, PCI, COPPA, GDPR and CCPA.



Paul Howard, Ph.D., is Director of Public Policy at Amicus Therapeutics, where he is responsible for ensuring consistent and appropriate implementation of overarching public policy initiatives and messages within the US that help shape the legislative, reimbursement, and regulatory environment for the development and marketing of products for the treatment of rare and genomic disorders. Previously, he was Senior Advisor to the Commissioner of the U.S. Food and Drug Administration (2017-19) on regulatory policy, strategic innovation initiatives, and promoting competition to efficiently advance public health and safety.

Prior to joining the U.S. FDA, he was a Senior Fellow and Director of Health Policy at the Manhattan Institute, where he wrote on a wide variety of medical-policy issues, including FDA reform, biopharmaceutical innovation, consumer-driven health care, and Medicare and Medicaid modernization.

David Lynch, M.D., Ph.D., is a graduate of Yale University majoring in Molecular Biophysics and Biochemistry, and obtained his MD and PhD at the Johns Hopkins University School of Medicine, graduating in 1988. His PhD thesis was on processing of endogenous opioid peptides. He completed internship and neurology residency at the University of Pennsylvania, followed by a fellowship in molecular pharmacology, neurogenetics and movement disorders at the same institution. He was appointed to the faculty in 1995 as an Assistant Professor in Neurology and Pediatrics. He was promoted and received tenure in 2004 and promoted to Full Professor in 2010. He has published > 250 scientific papers, mainly on NMDA receptors, antiNMDA receptor encephalitis, and Friedreich Ataxia, with greater than 23,000 citations. He has been continuously funded by the NIH since 1994 for basic science, clinical science and translational projects.



Rajnikanth (Raj) Madabushi, Ph.D., received his Ph.D. degree in Pharmaceutical Sciences from Birla Institute of Technology and Sciences (BITS), Pilani, India. Following his Ph.D., he did a post-doctoral fellowship with Prof. Hartmut Derendorf at University of Florida, Gainesville. He joined the Pharmacometrics Group at FDA in 2005. As a pharmacometrics reviewer, he was predominantly involved in the application of quantitative clinical pharmacology approaches for regulatory decision making and addressing various drug development issues in the areas of Cardio-Renal, Hematology and Endocrinology drug products. In 2009, he became the Team Leader in the Division of Clinical Pharmacology I, specifically focusing the area of Cardiovascular, Renal and Hematology products. In 2016, Dr. Madabushi became the Team Lead for the newly formed Guidance and Policy Team in the Immediate Office of the Office of Clinical Pharmacology. Since then, he has been involved in the drug development, regulation, research and policy from a clinical pharmacology perspective.



Terina N. Martinez, Ph.D., is Executive Director of the Duchenne Regulatory Science Consortium (D-RSC) and the Critical Path to Therapeutics for the Ataxias (CPTA) at C-Path. Terina is a highly motivated neuroscientist with experience in science communication, research program management and leadership, biomarker and drug development in neurodegenerative diseases across diverse sectors including academia, industry, nonprofit foundations, and patient advocacy groups.

Prior to joining C-Path, Terina was a Senior Associate Director, Research Programs at The Michael J. Fox Foundation for Parkinson's Research in New York, New York where she led the Foundation's programs for preclinical tools and animal models, emerging targets, and inflammation. Thereafter, Terina was a field application and collaboration scientist with Taconic Biosciences based out of Cambridge, MA, where she provided expert technical and scientific consultation across all research sectors for preclinical model selection, selection, application, translational and IND-enabling study design, encompassing diverse disease and

therapeutic areas. Terina received her undergraduate degree in Biology from the University of Dallas and earned a Ph.D. in Integrative Biology from the University of Texas Southwestern Medical Center at Dallas, where she studied cellular and molecular neuroscience. She completed her postdoctoral training at The University of Pittsburgh.

Terina is passionate about improving the lives of people living with devastating neurodegenerative disease and is thrilled to lead the D-RSC and CPTA teams in executing C-Path's mission to develop new approaches to advance medical innovation and regulatory science.

Justin Moy is a rising senior at Worcester Polytechnic Institute pursuing a combined bachelor and master's degree in bioinformatics and computational biology. He was born with merosin deficient congenital muscular dystrophy, now known as MDC1A, and served as MDA National Ambassador from 2018 through 2019. Justin also currently serves on the WPI Arts and Sciences Undergraduate Student Advisory Council. In his capacity as an RDCA-DAP beta tester, Justin has been examining the Duchenne muscular dystrophy dataset and has specifically focused on the 6-minute walk distance tests. He is excited to bring an undergraduate student perspective to this conference and hopes that data accessibility will encourage other young adults with disabilities to pursue STEM fields. Outside of school, he enjoys drinking tea and playing piano.



Andrew E. Mulberg, M.D., F.A.A.P., C.P.I., 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. He is currently Senior Vice President, Regulatory Affairs at Neurogene Inc, a company devoted to gene therapy approaches to management of neurodegenerative disorders in children and adults. 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.

Theresa Mullin, Ph.D., serves as CDER's Associate Director for Strategic Initiatives. She oversees areas of strategic interest to external stakeholders. She leads the 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. 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. Before joining CDER in 2007, Dr. Mullin was Assistant Commissioner for Planning in FDA's Office of the Commissioner. Dr. Mullin 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. Dr. Mullin received the Senior Executive Service Presidential Rank Award for Meritorious in 2006 and for Distinguished Service in 2011.



Edward Neilan, M.D., Ph.D., is the Chief Medical and Scientific Officer of the National Organization for Rare Disorders (NORD). He is a physician-scientist and rare disease expert, with prior leadership experience in both academic medicine and the pharmaceutical industry. Ed joined NORD in 2021. He leads our medical and research programs as part of NORD's mission to help the more than 25 million Americans living with rare diseases. He also seeks to encourage and enable institutions and companies to develop innovative approaches and new treatments to help rare disease patients. Prior to joining NORD, Ed worked at Sanofi Genzyme, where he led global medical affairs strategy and execution for the rare neurological diseases portfolio and contributed medical expertise to clinical development efforts across multiple programs. Prior to that, Ed served as the President of the Medical Staff at Boston Children's Hospital. As a staff physician, clinical geneticist, Dr. Neilan directly cared for and studied patients with many genetic diseases. He is an expert in clinical trial design and drug development, who has contributed data that helped support the FDA and global regulatory approvals of five new rare disease therapies, and he has authored or co-authored multiple

rial protocols and safety and regulatory reports to global health authorities. After completing B.S. and M.S degrees in Biology at Yale University, Ed earned his MD and PhD degrees at Stanford University. He then completed residency and fellowship training at Harvard Medical School, where he subsequently also served as a faculty member for more than 12 years. Dr. Neilan is board-certified in pediatrics, clinical genetics, and clinical biochemical genetics. He is a fellow of both the American Academy of Pediatrics and the American College of Medical Genetics and Genomics.

William Roddy, currently leads the Critical Path Institute's data engineering team and develops tooling to support the moving and processing of data from a diversity of data sources with a focus on extracting evidence to improve patient care. Mr. Roddy has a background in neuroscience and experience from a variety of biomedical informatics projects where he led key components of each phase of the research data lifecycle. His previous work has included the technical and scientific design and implementation databases for natural history, interventional, and retrospective studies in the areas of pain management, traumatic brain injury, stress disorders, and limb loss. Currently Mr. Roddy focuses on developing the data pipeline and platform for the RDCA-DAP project and leveraging data in the areas of rare diseases and complications of premature birth.



Christian Rummy, Ph.D., is an independent data scientist and biostatistician with more than 20 years of experience in academic, pre-competitive and industrial settings. Track record in creative analysis, communication, and translation of natural history data into improved clinical trials. Expert in Friedrich's ataxia, rating scales, and clinical endpoint development. Extensive experience in rare/neuromuscular diseases such as muscular dystrophy, ALS, MS and Parkinson's disease.



Peter L. Saltonstall has been President and CEO of the National Organization for Rare Disorders (NORD) since 2008. Under his leadership, NORD has maintained the integrity of the Orphan Drug Act while forging new relationships between the patient community and the White House, Congress, HHS, FDA, NIH, Social Security Administration and CMS; as well as with drug and device companies, and with the medical, academic and investment communities.

Peter is committed to globalization of the rare disease patient community, as diseases do not recognize geographical boundaries and research can be expedited when patients from many countries are involved. He has helped establish collaborative programs with patient communities throughout Europe, Australia, Asia and South America. He has overseen the expansion of NORD's US-based patient services program, which works with manufacturers and patients to provide financial assistance to patients in need. He has also played a major role in the building of the NORD longitudinal natural history system that is recognized by the FDA as one of the tools of choice for patient organizations collecting data on their disease.

Before joining NORD, Peter held senior positions with several major academic medical centers and organizations, including Harvard's Brigham and Women's Hospital, Tufts-New England Medical Center and St. Elizabeth's Medical Center of Boston. He helped launch the Harvard Risk Management Foundation's startup venture, Risk Management Strategies, and the University of Pittsburgh Medical Center's private equity arm, Strategic Business Initiatives.

Peter was the co-founder and CEO of SafeCare Systems, LLC, which developed one of the country's first patient safety management systems. He also played an active role on Capitol Hill in the development of the Patient Safety Act of 2005, which dramatically improved the reporting of events that adversely affect patients.

Theresa Strong, Ph.D., is a co-founder and Director of Research Programs at the Foundation for Prader-Willi Research (FPWR, fpwr.org), a nonprofit organization that supports research to advance the understanding and treatment of the rare neurodevelopmental disorder Prader-Willi syndrome (PWS). Theresa has a PhD in genetics and performed postdoctoral work at the University of Michigan. Prior to joining FPWR full time, she was a faculty member at the University of Alabama at Birmingham, working primarily in the area of cancer gene therapy, and she remains a Volunteer Adjunct Professor of Genetics at UAB. Theresa also serves on the steering committee of the Clinical Trials Transformation Initiative (CTTI), as well as the PWS Clinical Trial Consortium (pwsctc.org), is principal investigator for the Global PWS Registry (pwsregistry.org/), and is currently a member of the FDA Patient Engagement Collaborative. She has four children, including a young adult son with PWS.



Victoria (Vicki) Theurer Crider is an experienced project manager with more than 20 years of combined project management and data analysis experience. She is currently Senior Project Manager for the Data Collaboration Center (DCC) at C-Path and has been with the organization for three years. With her impressive background in software development, Vicki leads a diverse and talented platform development team for RDCA-DAP where she has spearheaded an intense beta testing project that brought together a diverse group of stakeholders for platform optimization. Along with leading beta testing experiences, Vicki manages all operations and project deliverables for her many grant-funded projects including ADDI Pilot and Prevent HAARM

Ramona Walls, Ph.D., received a Bachelor's degree in Environmental Resource Management and Horticulture at Penn State and a Ph.D. in Ecology and Evolution from Stony Brook University. After a post-doc at the New York Botanical Garden, she joined the iPlant Collaborative, now CyVerse, where she led initiatives related to integrating, managing, and publishing big data. In 2018, Walls became an Assistant Research Professor in the Bio5 Institute at the University of Arizona. Her research focuses on ontology design, ontology-based data integration, and management of large and dispersed datasets. Walls is currently Associated Director of Data Science at the Critical Path Institute, where she leads the Semantics, Ontologies, and Metadata, team. Walls is a board member and active contributor to the Genomics Standards Consortium. She is a founding member of the OBO Foundry Operations Committee and has contributed to multiple OBO ontologies.

