CRITICAL PATH INSTITUTE

NORD®
National Organization for Rare Disorders

RARE DISEASE CURES ACCELERATOR

DATA AND ANALYTICS PLATFORM LAUNCH

September 17, 2019
ROCKVILLE, MD

#RDCADAP
#rarecures

c-path.org/rdca-dap

Today, you'll have an opportunity to be a part of what we believe will be a paradigm shift in research for new treatments and cures. We encourage you to engage with our expert speakers, panelists and colleagues.

Please offer your thoughts, comments and questions about the project to this dialogue. And please share your experience via LinkedIn or Twitter using the hashtags **#RDCADAP** and **#rarecures**, so that we can collectively spread the word within our large and connected community.

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Messages from C-Path and NORD Leaders

JOSEPH SCHEEREN

Welcome to the Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP) launch meeting — we're excited you are all here. Your participation demonstrates your commitment to our collective quest to accelerate the advancement of treatments and cures for rare diseases. The RDCA-DAP program aims to do this through areas in which C-Path has proven expertise: collaboration and data standardization, aggregation, analysis and sharing. Our goal is to improve the lives of people living with rare diseases around the world — this is only the beginning of our path to discovering treatments and cures. With a shared vision, we will work in close collaboration with our colleagues from the FDA and the National Organization for Rare Disorders® (NORD) to be the critical force in accelerating therapeutic development for rare diseases.

RDCA-DAP is part of a larger initiative led by the FDA to give patients and their loved ones hope that a cure is on the horizon. We will soon announce another part of this initiative, the Rare Disease Cures Accelerator-Clinical Outcome Assessments (RDCA-COA), also in collaboration with the FDA and NORD. These initiatives will only succeed with the full support of us all, fostering transparent, collaborative data sharing. Data will come from a variety of sources and will be diverse. By optimizing the usability of these data through standardization and combination across diseases, we will be able to extract new insights and potential solutions using analytic tools that will be accessible to the global community.

The RDCA-DAP launch is one milestone on our journey to bring relief for patients. We welcome you to continue your participation in the global rare disease community all year round. As the project develops, we will need the input and expertise of all community stakeholders, and we hope you will contribute your knowledge and data. Each day, somewhere across the globe, members of this community are taking the next step, creating new knowledge, and joining together to collectively move forward in our mission to advance life-changing therapies for the patients who inspire us. Your presence and commitment, make this extraordinary progress possible. Thank you all from the bottom of my heart for your continued support.

Sincerely,

Joseph Scheeren, PharmD
President and CEO
Critical Path Institute

PETER L. SALTONSTALL

For more than 35 years, the National Organization for Rare Disorders has committed to improving the lives of the over 25 million Americans living with rare diseases. At NORD, we believe uniting the entire rare disease community and its stakeholders is imperative. Together, we can partner to drive innovation, research and new therapies for those millions of people whose lives are challenged by rare diseases. Today, there is no question about the power and possibility of data. Patient registries and longitudinal natural history studies are critical tools that can align stakeholders around a shared mission to accelerate scientific discovery and therapeutic product development for the 90% of rare diseases that remain without an FDA-approved treatment.

We at NORD are especially proud to partner with the Critical Path Institute on the Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP), a project funded by the FDA that we believe has the potential to change lives.

RDCA-DAP is a resource and a solution. The platform will help stakeholders navigate well-known data challenges, allowing researchers and drug developers to access and understand data about rare diseases and how they progress, and creating opportunities for innovative use cases that drive new insights. Optimizing tools and methodologies to empower the rare disease community and create efficiencies in clinical trial design will lead to faster, less expensive development of new treatments. The input and participation of patients, and the support that NORD will provide through its research and registry program, will be integral parts of the collaboration, providing opportunities for the community to not only share their experience and expertise, but also to have a real role in moving towards life-saving cures.

As always, our work is guided by our principles of authentic community engagement, inclusivity, transparency and integrity. I am excited about today's launch and thrilled you are able to join us for what I know will be a momentous start to a ground-breaking and transformative collaboration.

Sincerely,

Peter L. Saltonstall
President and CEO
National Organization for Rare Disorders

RDCA-DAP

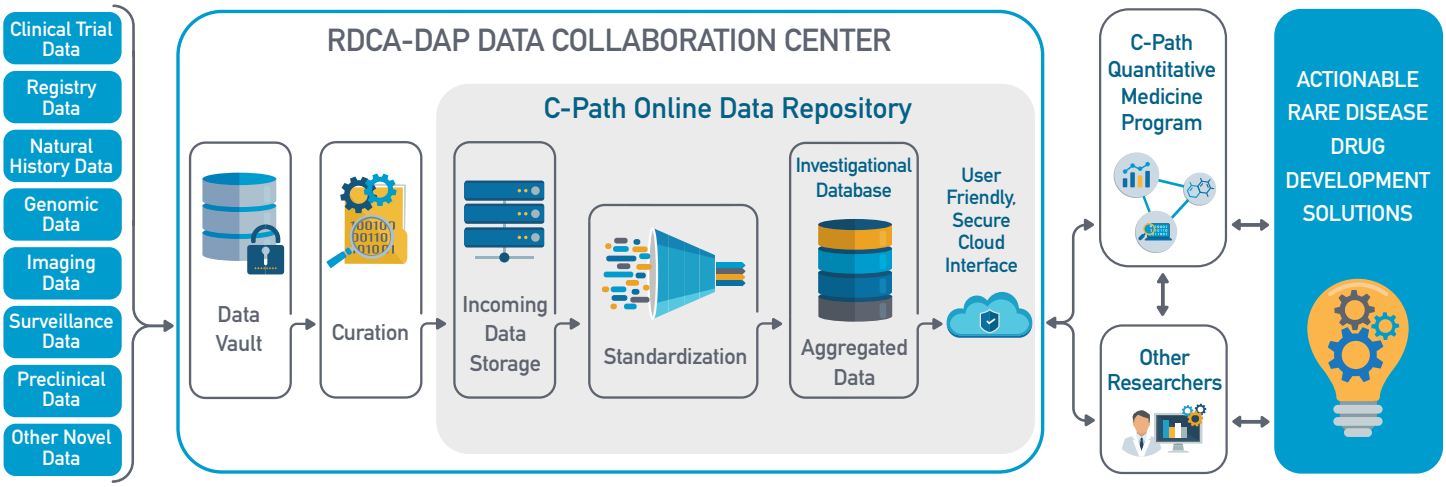
A NEW DATA AND ANALYTICS PLATFORM

The Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP) is an integrated database and analytics hub that is designed to be used in building novel tools to accelerate drug development across rare diseases. It is being developed by the Critical Path Institute (C-Path) and the National Organization for Rare Disorders® (NORD) through a collaborative grant from the FDA [Critical Path Public-Private Partnerships Grant Number U18 FD005320 from the US Food and Drug Administration].

RDCA-DAP will promote the sharing of existing patient-level data and encourage the standardization of new data collection. By integrating such data in a regulatory-grade format suitable for analytics, RDCA-DAP will accelerate the understanding of disease progression (including sources of variability to optimize the characterization of subpopulations), clinical outcome measures and biomarkers, and facilitate the development of mathematical models of disease and innovative clinical trial designs. RDCA-DAP will be positioned to generate solutions to drug development bottlenecks. As such, the utility of the patient-level data will be maximized and used to develop tools that will be accessible to the community in order to optimize and accelerate drug development across rare disease states.

HOW RDCA-DAP WORKS

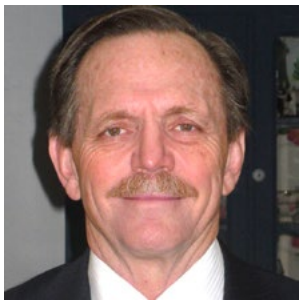
The RDCA-DAP will house integrated patient-level data from diverse sources, including clinical trials, longitudinal observational studies, patient registries and real-world data (e.g. electronic health records) across a multitude of rare diseases. Data will be contributed from different organizations and companies around the world. C-Path has extensive experience in building such integrated databases for many diseases, including existing rare disease databases (in Duchenne muscular dystrophy, Huntington's disease, Friedreich's ataxia and polycystic kidney disease). NORD will leverage their IAMRARE™ registry platform and extensive expertise to identify the data contributors, establish contacts with the contributing organizations and help to negotiate data contribution and use agreements to allow patient-level data to be transferred to the RDCA-DAP. From there the data can be standardized and integrated with other contributed data and made available to the degree agreed to by the data contributors to researchers.



RDCA-DAP Launch Agenda

7:30 AM - 8:30 AM	**Breakfast & Registration**	
8:30 AM - 8:40 AM	**Welcome**	Joseph Scheeren (C-Path) Peter Saltonstall (NORD)
8:40 AM - 8:55 AM	**Opening Remarks**	Janet Woodcock (CDER/FDA)
8:55 AM - 9:30 AM	**RDCA: What is it? and What are its Core Components?**	Theresa Mullin (CDER/FDA)
9:30 AM - 9:50 AM	**Coffee Break**	
9:50 AM - 11:00 AM	**Moderated Panel Discussion: Current Challenges in Rare Disease Drug Development**	*Moderator:* Kathleen Donohue (CDER/FDA) *Panelists:* Thomas Blättler (Orphazyme) Rosángel Cruz (Cure SMA) Tanya Fischer (Sanofi) Josh Lehrer (Global Blood Therapeutics) Jeff Sherman (Horizon Therapeutics)
11:00 AM - 12:30 PM	**Lunch**	
12:30 PM - 1:00 PM	**Accelerating Drug Development for Rare Diseases: Establishing a Cornerstone Through Data Sharing**	Samantha Budd Haeberlein (Biogen)
1:00 PM - 1:30 PM	**Overview of NORD IAMRARE™ Registry**	Vanessa Boulanger (NORD)
1:30 PM - 2:15 PM	**Overview of C-Path Data Integration and Analytics Approaches**	Klaus Romero (C-Path)
2:15 PM - 2:45 PM	**Coffee Break**	
2:45 PM - 4:15 PM	**Case Studies of Data Integration in Rare Diseases, How They Fit the Vision for RDCA-DAP and Next Steps**	*Co-Moderators:* Jane Larkindale (C-Path) Pamela Gavin (NORD) *Panelists:* Ron Bartek (FARA) Alexandra Kruse (PDSA) Kevin Krudys (CDER/FDA) Theresa Strong (FPWR) Michael Yeaman (Guthy-Jackson)
4:15 PM - 4:30 PM	**Closing Remarks**	Billy Dunn (CDER/FDA)
4:30 PM	**Adjourn**	

THANK YOU to our
Speakers, Panelists and Moderators



Ronald (Ron) Bartek, MA, is Co-founder/President, Friedreich's Ataxia Research Alliance; Board of Directors/past Chairman, National Organization for Rare Disorders; Board of Directors, Alliance for a Stronger FDA and Alliance for Regenerative Medicine; Member, NIH/NCATS National Advisory Council; Vice Chair, NCATS Cures Acceleration Network Review Board; member, FDA/CTTI Patient Engagement Collaborative; 4-year member, NIH Neurological Institute National Advisory Council; former partner/president, business development/government affairs firm; twenty years in federal executive and legislative branches in defense, foreign policy and intelligence, (six years on House Armed Services Committee staff; four years at U.S. State Department, one year on U.S. Delegation to Intermediate-Range Nuclear Forces Treaty talks, Geneva; six years as CIA analyst, including a year as Intelligence Community representative to U.S. arms control committees; after graduation from United States Military Academy, West Point, four years as Army officer, as company commander in Korea and Infantry and Military Intelligence officer in Vietnam; Master's Degree, Russian Area Studies - Georgetown University.

Thomas Blättler, MD, is Chief Medical Officer at Orphazyme A/S. Thomas is a neurologist who, for fifteen years, has assumed roles with increasing strategic responsibility in the biopharmaceutical industry. He has overseen clinical development programs from phases 1-3 in neurological and psychiatric diseases, most recently with a focus on rare diseases with high unmet medical needs.

After 12 years in "big pharma" at Novartis, Bristol Myers Squibb, and Roche, Thomas joined Orphazyme as Chief Medical Officer in 2016. Here, he has expanded the development team to 40+ employees managing four rare disease, late-stage programs in Niemann-Pick disease Type C, Amyotrophic Lateral Sclerosis, sporadic Inclusion Body Myositis, and (neuronopathic) Gaucher disease. Moving from relatively high-prevalence disease areas to rare diseases, Thomas' clinical research interests are focused on gathering information on disease progression patterns, analyzing factors that influence those patterns, as well as understanding how to measure disease progression in the rare disease space. Thomas earned his medical degree from the University of Zurich, Switzerland.

Vanessa Boulanger, MSc, is the Director of Research at the National Organization for Rare Disorders (NORD). Vanessa oversees the management, growth, and implementation of NORD's research activities. Prior to joining NORD in 2017, 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. In 2018, Vanessa was appointed as a member of the Patient-Centered Outcomes Research Institute (PCORI) Advisory Panel on Rare Diseases. She holds an MSc degree in Global Health and Population from Harvard University and a BA in International Development and Social Change from Clark University.

Samantha Budd Haeberlein, PhD, is the Vice President, Late Stage Clinical Development Unit Head, at Biogen. Samantha Budd Haeberlein has nearly twenty years of biopharmaceutical industry experience across Research, Translational Medicine and Clinical Development. Based in Cambridge, MA Samantha currently leads the Late Stage Unit accountable for the Clinical Development of Biogen's Alzheimer's disease, dementia & Movement Disorders portfolios. Prior to Biogen Samantha was at AstraZeneca where she held roles in the US, Canada and Sweden as Vice President of Translational Science & Global Program Lead in Alzheimer's disease.

Samantha's discovery and development experience spans multiple therapeutic modalities and multiple Neurological indications including Alzheimer's disease, dementias, movement disorders, pain and ophthalmology. Samantha is recognized internationally for her leadership in Alzheimer's disease having led and developed programs through early and late clinical stages, and in doing has been passionate about advancing the science on biomarkers, patient selection, diagnostics and disease stage. For her leadership in this space Samantha was a recipient of the 2017 Fierce Women in Biotech Award.

Samantha is a member of the World Dementia Council, and an executive member of the Board of The Boston Home. Samantha has a BSc and a PhD in Biochemistry from the University of Dundee in Scotland. She was a Wellcome Trust Fellow and Instructor at Harvard Medical School at Children's Hospital and Brigham & Women's Hospital in Boston and conducted research at The Burnham Institute in San Diego before joining the biopharmaceutical industry.

Rosángel Cruz, MS, is a Director of Research and Clinical Affairs at Cure SMA who brings over two decades of experience spanning the Business, Non-Profit, and Academic/Research sectors. In her current role, Rosángel provides strategic direction and operational oversight for an SMA Industry Collaboration (IC) that works together to share information, ideas, and data to address clinical, scientific and regulatory topics critical to advancing drug development in SMA and goals important to the community. Prior to joining Cure SMA, Rosángel successfully managed Industry and Investigator-Sponsored clinical trials, and natural history trials, involving children with neuromuscular disorders (Spinal Muscular Atrophy, Becker/Duchenne Muscular Dystrophy, and Centronuclear/Myotubular Myopathy (MTM). Rosángel is well-versed in protocol development, clinical trials' design and execution, PFDD, patient-reported outcomes, and FDA regulatory compliance; she brings five years of experience as Quality Manager & Consultant at J.P. Morgan Chase and Merrill Lynch. Rosángel holds a Bachelor of Science (Magna Cum Laude, Phi-Beta Kappa) and a master's degree in Psychology, with a concentration in Behavioral Neuroscience.

Kathleen Donohue, MD, MS, graduated from medical school at Virginia Commonwealth University. She completed her residency in Internal Medicine and fellowships in Allergy & Immunology and the Genetics of Complex Diseases 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 translational molecular 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 drug development for patients with rare genetic disorders.

Billy Dunn, MD, is the Director of the Division of Neurology Products (DNP) at the U.S. Food and Drug Administration's Center for Drug Evaluation and Research. The Division of Neurology Products 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 lateral sclerosis, multiple sclerosis, cerebral palsy, dementia, narcolepsy, Lennox-Gastaut syndrome, and insomnia.

Tanya Fischer, MD, PhD, serves as Global Project Head at Sanofi in the Multiple Sclerosis, Neurology and Gene Therapy Therapeutic Area. She is responsible for leading the flagship global project teams in clinical development (Phase 2 and Phase 3) for a rare genetic form of Parkinson's Disease and related rare neurodegenerative diseases (such as Gaucher disease (type 3), as well as genetic forms of ophthalmology. Moreover, her team is responsible for bringing forward the first industry sponsored clinical trial in a genetic form of Parkinson's Disease. As a physician-scientist, Dr. Fischer also works closely between research and clinical development for internal and external programs in both the Neurology and the Rare Disease therapeutic areas.

Dr. Fischer is a board certified neurologist, with clinical subspecialties in multiple sclerosis/demyelinating diseases and chronic neuropathic pain. Dr. Fischer did her Neurology residency at Yale New Haven Hospital. While an Associate Professor at Yale University in Neurology, her VA-supported academic research focused on genetic and acquired forms of pain (ion channels (especially in Nav 1.7) and diabetic neuropathy) with a variety of peer-reviewed paper. She also was awarded the prestigious Presidential Early Career Award for Scientists and Engineers (PECASE) Award in 2011. The PECASE Awards are intended to recognize some of the finest scientists and engineers who, while early in their research careers, show exceptional potential for leadership at the frontiers of scientific knowledge during the twenty-first century.

The Awards foster innovative and far-reaching developments in science and technology, increase awareness of careers in science and engineering, give recognition to the scientific missions of participating agencies, enhance connections between fundamental research and national goals, and highlight the importance of science and technology for the nation's future.

The PECASE Award is the highest honor bestowed by the U.S. government on outstanding scientists and engineers beginning their independent careers.

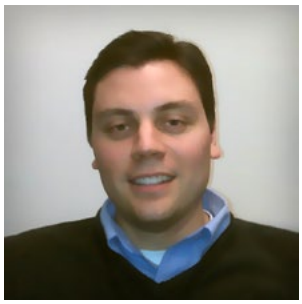
Dr. Fischer currently represents Sanofi on the international scientific advisory board (IASB) for the Parkinson's Progression Marker Initiative (PPMI) and on the GBA-PD committee of the Michael J. Fox Foundation (MJFF); she also represents Sanofi at the Critical Path for Parkinson Consortium (CPP). Moreover, she is the co-chair for AMP-PD since it was first started in January 2018.

Pamela Gavin, MBA, currently serves as Chief Strategy Officer at the National Organization for Rare Disorders (NORD). Her primary concerns in this position include NORD's membership programs; patient support services, strategic business development and general operations. She joined NORD in 2010 after 13 years in positions focused on health care safety where she was chiefly responsible for implementing complex, multi-stakeholder programs aimed at improving patient-centric quality of care and mitigating risk of harm. As a consultant to the federal government, she received Special Citations from FDA's Commissioner and CFSAN Director for outstanding leadership and teamwork implementing a new web-based portal for the reporting of pre-market and post-market safety data to FDA and NIH.

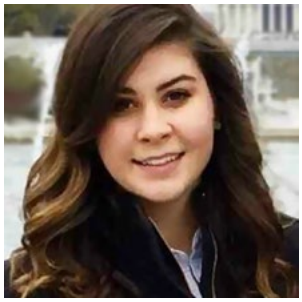
As Senior Director at the University of Pittsburgh Medical Center Pamela worked in an equity partner business unit, bringing new concepts and emerging technologies to market utilizing the UPMC enterprise to test, evaluate and develop products designed to improve healthcare delivery. Pamela provided executive oversight for several strategic business initiatives, including a clinical trial enrollment and adverse event reporting system, a multi-biomarker assay for the early detection of ovarian cancer, a medical simulation training system and a clinical decision support system for infection control and antibiotic management.

Pamela co-founded SafeCare™ Systems, LLC, which developed one of the country's first patient safety management systems and served as director for RMF Strategies, a division of the Risk Management Foundation of the Harvard Medical Institutions responsible for the commercialization of data driven risk management solutions.

Pamela earned an undergraduate degree in biology from Smith College and an MBA, with a concentration in health care management, from Northeastern University.



Kevin Krudys, PhD, is a Senior Clinical Analyst for Quantitative Analysis and Modeling in the Division of Neurology Products at the FDA. He previously served as Team Leader in the Division of Pharmacometrics in the Office of Clinical Pharmacology at the FDA. His team was responsible for reviewing the application of model informed drug development in the therapeutic areas of neurology, psychiatry, anesthesia, analgesia and addiction. He also previously served as the Scientific Lead of the QT Interdisciplinary Review Team and is currently a member of the Pediatric Review Committee. Prior to joining the FDA he was a fellow in the Clinical Pharmacokinetics Modelling and Simulation group at GlaxoSmithKline. He earned his Ph.D. in Bioengineering from the University of Washington.



Alexandra Kruse is the Research Coordinator for the Platelet Disorder Support Association, overseeing research and advocacy initiatives for patients with Immune Thrombocytopenia (ITP). She created and manages the ITP Natural History Study Patient Registry in collaboration with NORD and support from the U.S. Food and Drug Administration. She holds certificates in Registry Studies, Biomedical Investigators Informational Privacy and Security, Good Clinical Practice HIPAA, and Advanced Methods in Clinical Effectiveness Research. A promoter of patient-powered research, she reshaped PDSA's grant guidelines with guidance from the Patient-Centered Outcomes Research Institute, encouraging patient involvement in promising ITP research and oversees the PDSA Research Program. Prior to her work at PDSA, Alexandra worked in clinical research at Weill Cornell / New York Presbyterian. Alexandra is currently also an MD Candidate at Tulane University School of Medicine.

Jane Larkindale, DPhil, is the Executive Director for the Duchenne Regulatory Sciences Consortium (D-RSC) at the Critical Path Institute in Tucson, Arizona. She launched D-RSC in 2005 and has been its leader since inception. 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. In the laboratory, she did research in areas as diverse as molecular biology, biochemistry, genomics, plant science, medical physics, marine biology, and industrial chemistry. In the course of this research, she published numerous original research papers and review articles in several disciplines. Her experience in drug development and neuromuscular diseases started at the Muscular Dystrophy Association, an international non-profit covering over forty neuromuscular diseases, where she ended as Vice President for Research. Dr. Larkindale was instrumental in the start-up of MDA Venture Philanthropy (MDA's drug development arm), which invested in 21 drug development projects, of which 10 entered clinical trials, and several of which have been licensed by large pharmaceutical companies. After leaving MDA, Dr. Larkindale started a consulting company in the area of drug development for rare neuromuscular diseases and worked for the Friedreich's Ataxia Research Alliance, developing biomarker and patient reported outcomes programs and working on a new patient registry.

Josh Lehrer, MD, Senior Vice President, Development, joined GBT in October 2013 as medical director and was appointed senior vice president, development in July 2017. At GBT, he has overseen the clinical development organization and has led program strategy and execution for GBT440, bringing it through early stage studies into a pivotal Phase 3 program in sickle cell disease. Prior to GBT, Dr. Lehrer led clinical development programs through proof of concept at Genentech in multiple indications and held key roles in business development and overseeing cardiovascular safety. Dr. Lehrer has served as adjunct faculty in the Division of Cardiology at Stanford School of Medicine. Dr. Lehrer holds an AB from Harvard College in Biochemistry and an M.Phil. in Biological Sciences from Cambridge University. Dr. Lehrer completed medical school and training in internal medicine at the University of California San Francisco (UCSF) and cardiology specialty training and postdoctoral research at Stanford University School of Medicine.

Theresa Mullin, PhD, 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.

Klaus Romero, MD, MS, FCP is a clinical pharmacologist and epidemiologist by training, with 15 years combined experience in academic clinical research. Dr. Romero has been with C-Path since December of 2007, and during his tenure, he has helped lead clinical pharmacology, pharmacoepidemiology and modeling and simulation projects in Alzheimer's disease, polycystic kidney disease, tuberculosis, type 1 diabetes, Parkinson's disease, Duchenne muscular dystrophy, kidney transplantation and Huntington's disease, achieving major milestones such as the first regulatory endorsement by FDA and EMA of a clinical trial simulation tool for mild and moderate Alzheimer's Disease and the qualification of an imaging prognostic biomarker for PKD. He is a fellow of the American College of Clinical Pharmacology and the American Society for Clinical Pharmacology and Therapeutics.

Peter L. Saltonstall is the President and CEO of the National Organization for Rare Disorders (NORD). He joined NORD in 2008 after having served for more than 30 years as a senior executive in both for-profit and not-for-profit healthcare environments.

Under his leadership, NORD has maintained the integrity of the Orphan Drug Act while forging new relationships between the patient community and the Executive branch, 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. His efforts to build collaborations stems from his view that advances for the rare disease patient can be achieved best through joint efforts. Today he continues to be one of the countries lead voices on rare disease issues to Industry, FDA, Congress and the Government.

Peter is also 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 established collaborative programs with patient communities throughout Europe, Australia, Japan, Asia and South America and jointly built and launched with EURORDIS (the NORD of Europe) the first global, rare disease-specific web portal enabling patients and clinicians to connect with one another and share information.

Under Peter's leadership, NORD also has built a US-based Patient Assistance Network, which works with manufacturers and patients to provide medication assistance to patients in need of medications they cannot afford. He is also responsible for

building the NORD Longitudinal Natural History System that is recognized by the FDA as the tool 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 Harvard Risk Management Foundation's startup venture, Risk Management Strategies, and the University of Pittsburgh Medical Center's private equity arm, Strategic Business Initiatives. In addition, 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.

Peter serves on the Humana Cares Advisory Board, the FDA Cellular, Tissue & Gene Therapies Advisory Committee (CTGTAC), the NIH's NeuroNEXT External Oversight Board (EOB), the Child Neurology Foundation Board of Directors, the CTSA Consortium Coordinating Center External Advisory Board. Peter is also a member of the IRDiRC (International Rare Diseases Research Consortium) Executive Committee is a founding and current board member of the Medical Device Innovation Consortium (MDIC) and also serves on the Board of Directors of Trio Health and MIT's NEWDIGS Executive Committee.

Joseph Scheeren, PharmD, is President and CEO of the Critical Path Institute. Dr. Scheeren is a global regulatory affairs leader with a distinguished 36-year career in the pharmaceutical industry. His work has supported the approval of more than fifteen drugs or devices.
Dr. Scheeren studied pharmacy at the University of Leiden and started his pharmaceutical industry career in 1982 with Servier in Paris, responsible for Regulatory Affairs Northern and Eastern Europe, and Clinical Development. He was subsequently appointed head for Worldwide Regulatory Affairs at Serono in Geneva, the Global Regulatory Affairs department of Roussel UCLAF in Paris, and the Global Marketed Product Regulatory Affairs Department of Hoechst Marion Roussel in Bridgewater. After the merger with Rhone Poulenc Rorer in 2000, he was nominated to a similar position. Dr. Scheeren joined Bayer Pharmaceuticals as Senior Vice President, Head of Global Regulatory Affairs Pharma in 2004, responsible for development in the US, and in 2009 became Site Head US in Montville, NJ. In 2012, he assumed in addition to his role as Head Global RA Pharma, the position of Head of Development Asia in Beijing, and in 2015, was appointed Head of Global Regulatory Affairs Pharma and Consumer Care of Bayer, Basel. In January 2018, he was appointed Senior Advisor R&D, Bayer AG in Berlin. Dr. Scheeren is the immediate past Chairman of the Board of Directors for the Drug Information Association (DIA). He serves on advisory boards at the Center for Innovation in Regulatory Science, the Regulatory Affairs track at Yale University, and the Center of Regulatory Excellence in Singapore. He is also a foreign member of the Academie Nationale de Pharmacie, France, a lecturer at Yale University, and an Adjunct Professor for Regulatory Sciences at Peking University in the Department of Clinical Research.

Jeffrey (Jeff) W. Sherman, MD, FACP, is Chief Medical Officer and Executive Vice President at Horizon Therapeutics in Lake Forest, Illinois. Jeff has more than 25 years of experience in the pharmaceutical industry at IDM Pharma, Takeda Global Research and Development, NeoPharm, Searle/Pharmacia, and Squibb/Bristol-Myers Squibb. He also serves on the Board of Directors of Strongbridge Biopharma and Xeris Pharmaceuticals.

Jeff is a Past President of the Drug Information Association (DIA) and Board of Directors. He also serves as the DIA liaison to the FDA Clinical Trial Transformation Initiative (CTTI) Steering

Committee. Jeff in addition serves on the Board of Advisors of the Center for Information and Study on Clinical Research Participation (CISCRP). He is also a member of the Global Genes Medical and Scientific Advisory Board and actively involved with the National Organization for Rare Diseases (NORD), the European Organization for Rare Diseases (EURORDIS), and the Rare Diseases Forum.

Theresa Strong, PhD, is a co-founder and Director of Research Programs at the Foundation for Prader-Willi Research (FPWR, www.fpwr.org), a nonprofit organization that supports research to advance the understanding and treatment of the rare neurodevelopmental disorder Prader-Willi syndrome (PWS). Theresa received her B.S. from Rutgers University, a Ph.D. in Medical Genetics from the University of Alabama at Birmingham (UAB) and performed postdoctoral work at the University of Michigan. Prior to joining FPWR full time, she was a faculty member at UAB, working primarily in the area of cancer gene therapy, and remains an Adjunct Professor of Genetics at UAB. She has four children, including a young adult son with PWS.

Janet Woodcock, MD, is Director of the Center for Drug Evaluation and Research (CDER), at the Food and Drug Administration (FDA). Dr. Woodcock first joined CDER in 1994. For three years, from 2005 until 2008, she served FDA's Commissioner, holding several positions, including as Deputy Commissioner and Chief Medical Officer, Deputy Commissioner for Operations, and Chief Operating Officer. Her responsibilities involved oversight of various aspects of scientific and medical regulatory operations. Before joining CDER, Dr. Woodcock served as Director, Office of Therapeutics Research and Review, and Acting Deputy Director in FDA's Center for Biologics Evaluation and Research. Dr. Woodcock received her M.D. from Northwestern Medical School and completed further training and held teaching appointments at the Pennsylvania State University and the University of California in San Francisco. She joined FDA in 1986.

Michael Yeaman, PhD, earned doctoral degrees in immunology and medical science from the University of New Mexico School of Medicine and the University of California, Los Angeles School of Medicine, and he completed NIH and AHA Fellowships in Infectious Diseases and Molecular Immunology at UCLA. He is tenured Professor of Medicine, Department of Medicine at UCLA, and Vice Chair of Medicine and Chief, Division of Molecular Medicine at Harbor-UCLA Medical Center. His expertise focuses on infection and immunity, with special interests in antibiotic-resistance infections and immunology of rare autoimmune diseases. He is Chair Advisor, Guthy-Jackson Charitable Foundation, on its mission to solve the rare autoimmune disease, NMO. Michael has received many honors, including the National Research Service Award (NIH), NIH Innovation Award, and Distinguished Investigator Award (U.S. Department of Defense). He has published over 200 medical & scientific papers and scholarly works, holds 25 issued patents and over 20 patents pending. He lectures internationally, is an appointed NIH expert in infectious disease and immunology, and serves on editorial boards of premier journals. Michael is also an accomplished musical composer and performer integrating art, science and medicine for health & wellness. His music has appeared in films & multimedia productions, his 15 released albums are available on iTunes® and other leading digital services, and his Pandora® radio station is part of the music genome.

COMMITTED TO THE IDENTIFICATION, TREATMENT AND CURE OF RARE DISEASES

The National Organization for Rare Disorders® is leading the fight to improve the lives of rare disease patients and families. We work together with the rare community to accelerate research, raise awareness, provide valuable information and drive public policy that benefits the over 25 million Americans impacted by rare diseases.

COMMUNITY & EVENTS

POLICY LEADERSHIP

EDUCATIONAL PROGRAMMING

RESEARCH, GRANTS & REGISTRIES

RARE DISEASE REPORTS

PATIENT ASSISTANCE

Madilyn (left) has been battling a rare breathing disorder since birth called Congenital Central Hypoventilation Syndrome (CCHS), or Ondine's Curse.

Join us on this rare journey and help drive innovation for rare diseases.

NORD®
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NORD® Rare Research 30 YEARS and Thriving
Granting Hope for Rare Diseases

Alone we are rare. Together we are strong.®

CRITICAL PATH INSTITUTE

FACETS OF SUCCESS

A gem nestled in the American Southwest's Sonoran Desert, C-Path is making a significant and growing impact globally on the process of developing new therapies.

In the first half of 2019, Tucson, Arizona-based Critical Path Institute (C-Path) made a number of significant moves purposefully designed to further accelerate its mission to act as a catalyst in the development of new approaches to advance medical innovation and regulatory science.

The nonprofit organization recognizes that, for people living with devastating diseases, time is of the essence — so it is expediting the pathway to safe and effective treatments through the development of pioneering tools and processes. The ability to tap into its international presence, people and programs affords C-Path the opportunity to uniquely position itself for maximum impact.

Increased Global Presence

Recently, C-Path ramped up operations at the Critical Path Institute, Ltd. (C-Path, Ltd.) in Ireland. This fully-owned subsidiary will enable C-Path to enhance its many collaborative efforts with other organizations in Europe and to be physically closer to the European Medical Agency (EMA), which reviews and makes decisions concerning the novel methodologies that C-Path develops.

C-Path, Ltd. works with scientists and clinicians from the biopharmaceutical industry, government regulatory agencies, academic institutions and patient groups. These collaborations aim to create new tools and processes to develop and test new therapies.

New Talent in the Mix

In March, C-Path appointed Joseph Scheeren, PharmD, as its new President and Chief Executive Officer. Scheeren brings a distinguished 36-year career in the pharmaceutical industry, having held positions domestically and internationally in drug development and regulatory affairs on three continents—he's committed to continuing C-Path's success and recognition as a change agent in health outcomes.

In July, C-Path welcomed Kristen Swingle, MS, as its new Chief Operating Officer. Swingle brings nearly two decades' worth of experience in the medical and molecular sciences industry to lead the implementation of C-Path's global strategy and goals.

The two new leaders complement C-Path's staff of respected PhDs, MDs and industry experts in the research and regulatory fields, as they work together to make a lasting impact in global health.

New Directions

Deeply committed to education in regulatory affairs, C-Path and the University of Arizona James E. Rogers College of Law earlier this year instituted a fully online Graduate Certificate Program in Regulatory Science to provide specialized training to advance the translation of research into clinical interventions. This certificate, created by academic and industry leaders, enables participants to learn what it takes to develop medical products by learning the ethics, policies, and regulations governing regulatory science to make an impact in the biotech and pharmaceutical industry.

With more than 25 million people in the United States affected by one of more than 7,000 rare diseases, in September, C-Path and the National Organization for Rare Disorders (NORD) formally launched an effort to develop a new Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP). Funded by a cooperative agreement with the U.S. Food and Drug Administration, the goal of the platform is to accelerate the development of therapies from bench to bedside for rare diseases. The platform will provide a mechanism to transform data into actionable knowledge that will better characterize these diseases, aiding in the design of clinical trials for therapies in development.

Commitment to Impacting Global Health

C-Path's big-picture moves thus far in 2019 are sure to propel the regulatory science community toward a future of cutting-edge treatments for devastating diseases. With an expanding global presence and a dedicated team of motivated employees and partners, C-Path is shaping the future, faster.

Leading the Way

An international leader in forming collaborations to advance medical innovation and regulatory science, C-Path has established numerous global consortia; 13 are active today.

- Critical Path for Alzheimer's Disease (CPAD)
- Critical Path for Parkinson's (CPP)
- Critical Path to TB Drug Regimens (CPTR)
- Duchenne Regulatory Science Consortium (D-RSC)
- Electronic Patient-Reported Outcome Consortium (ePROC)
- Huntington's Disease Regulatory Science Consortium (HD-RSC)
- International Neonatal Consortium (INC)
- Multiple Sclerosis Outcome Assessments Consortium (MSOAC)
- Polycystic Kidney Disease Outcomes Consortium (PKDOC)
- Patient-Reported Outcome Consortium (PROC)
- Predictive Safety Testing Consortium (PSTC)
- Transplantation Therapeutics Consortium (TTC)
- Type 1 Diabetes Consortium (T1D)

These consortia currently include more than 1,600 scientists from government and regulatory agencies, academia, patient organizations, disease foundations, and dozens of pharmaceutical and biotech companies.

Each is working to develop new tools, methods or quantitative models to lower the risk and cost of drug development while improving the speed and probability of success for new therapies to treat devastating diseases like Alzheimer's, Parkinson's, diabetes, Duchenne muscular dystrophy, tuberculosis and others.

Notes

CRITICAL PATH INSTITUTE

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