

HD-RSC Kickoff Meeting, November 6-7, 2017

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Eric Bastings, MD

Deputy Director, Food and Drug Administration (FDA)

Dr. Bastings is Deputy Director of the Division of Neurology Products, in the Office of New Drugs, Center for Drug Evaluation and Research, Food and Drug Administration. Dr. Bastings is a neurologist, with subspecialty training in neurological rehabilitation. He received his medical degree from the University of Liège, Belgium. He completed postgraduate training in Neurology at the University of Liège, Belgium, and in Neurological Rehabilitation at Wake Forest University School of Medicine, Winston-Salem, North Carolina.

Dr. Bastings joined the FDA in 2000, as a Medical Officer in the Division of Neurology Products. He subsequently served as Clinical Team Leader in the same division from 2003 to 2008, before being appointed Deputy Director in 2008. Prior to joining FDA, Dr. Bastings was Assistant Professor of Neurology at Wake Forest University School of Medicine, Winston-Salem, North Carolina.

Robi Blumenstein, LLB, MBA

President, CHDI Foundation

In 2002, Robi Blumenstein organized CHDI Management, to provide management services to non-profit organizations engaged in Huntington's disease research. Robi began his career as a lawyer at Torys, a law firm in Toronto, before moving into merchant banking, where he was responsible for structuring and negotiating transactions and supervising investment analysis. He was a principal at First City Capital Corporation, CIBC Capital Partners and MMC Capital. Before all that, when he was still a kid, he built a harpsichord and was a director of Life Times Nine, a short subject film that was nominated for an Academy Award in 1973. Robi graduated from the University of Toronto with a BA (1975) and an LLB (1978), and has an MBA from Harvard Business School (1984).

Juliana Bronzova, MD, DSc

Consultant, uniQure and EHDN

With leadership in all facets of clinical development, clinical safety and business development during her time in Pharma Industry she was given senior roles in both Research and Development Divisions within the organization. She led the initial company biomarker strategy development, early discovery programs in Neuroscience (psychiatry; neurodegenerative disorders and TBI), global clinical development projects, including successful interactions with regulatory authorities- FDA, EMA and PMDA. She served as a member of the company Management Team, a Chair of Joint Clinical teams; Chair of Safety Management Team, Chair of Advisory Boards and member of DSMBs. She was one of the champions of cross-functional and Alliance management, clinical safety strategy and polices development, consulting Business Development in evaluation of numerous of CNS compounds. In 2011 she left pharma industry and founded clinical development consulting company.

At European Huntington's Disease Network (EHDN) she served as a Science Director from January 2012 until January 2017. Currently she is a consultant at EHDN and a consultant Medical Director at uniQure involved in the HD clinical program strategy development.

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Martha Brumfield, PhD

President and Chief Executive Officer, Critical Path Institute

Martha A. Brumfield, PhD, is President and Chief Executive Officer of Critical Path Institute, an Arizona based non-profit (501(c)(3)). In this role, Brumfield leads the institute in its mission to catalyze the development of new tools to advance medical product innovation and regulatory science which is accomplished by leading teams that share data, knowledge and expertise resulting in sound, consensus based science. Brumfield assumes the role of CEO after most recently serving as Critical Path Institute's Director of International & Regulatory Programs. In that position, she helped guide international program development and provided regulatory expertise to consortia. She is also Associate Professor, College of Pharmacy, The University of Arizona.

She also has her own consulting practice (Martha A. Brumfield LLC) focusing on concordance in global regulatory initiatives and regulatory science qualification programs. Other areas of focus in her practice include excellence in clinical trial conduct and pharmacovigilance, facilitation of scientific consortia and programs supporting patient access to medicines.

Brumfield brings 20 years of experience from Pfizer Inc., most recently, as senior vice president of worldwide regulatory affairs and quality assurance. There, she led a global team that supported lifecycle pharmaceutical research, development and commercialization through creation and implementation of regulatory strategies and quality assurance oversight. Brumfield also played a key role in managing the broader company relationships with global regulators, trade associations, academics and others on regulatory policy issues. She served on corporate governance initiatives including the planning and implementation of mergers and acquisitions and led her departments through these periods of significant change.

She recently served as Chair of the Board of Directors for the Regulatory Affairs Professional Society and chairs the Global Curriculum Coordinating Committee with FDA's Office of International Policy, which has developed a curriculum for regulators in developing countries. She is also active with global nonprofits, including the Regulatory Harmonization Institute and GlobalMD, where she delivers educational workshops on regulatory and clinical trial topics in Asia. She has served on and contributed to the Institute of Medicine consensus committees, which were commissioned by U.S. FDA focusing on global regulatory systems and on falsified and substandard drugs. She also serves on the Steering Committee of the Multi-Regional Clinical Trials Center of Brigham and Women's Hospital and Harvard and on the External Advisory Committee to Vivli, a MRCT launched non-profit

Brumfield earned a B.S. and an M.S. in chemistry from Virginia Commonwealth University, a Ph.D. in organic chemistry from the University of Maryland, and served as a post-doctoral fellow at The Rockefeller University.

Jackson Burton, PhD

Associate Program Director, Quantitative Medicine, Critical Path Institute

Dr. Burton received his B.S. and M.S. in mathematics at Montclair State University and his PhD in applied mathematics from the University of Arizona under the advisement of Timothy Secomb. His graduate study research focused on quantitative modeling of biological / population based systems, including infectious disease dynamics, structural pathology of malaria-infected red blood cells, and drug transport in solid tumors. Dr. Burton worked with Takeda Pharmaceuticals in general modeling and statistical analysis for a variety of mission-driven

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open questions in oncology pharmacokinetics and decision science. He joined Fractal Therapeutics where he served as a modeling and simulation project lead for student teams locally and remotely on infectious disease diagnostic projects. Dr. Burton is part of C-Path quantitative medicine team supporting the creation of drug development tools for Alzheimer's and Parkinson's disease.

Jeff Carroll, PhD

Associate Professor, Western Washington University

Dr. Carroll was born and raised in Kent, Washington. Immediately after high school he joined the United States Army, serving in the US, Germany and Kosovo. After his four-and-a-half-year tour in the Army, Jeff studied for his Bachelors of Science at the University of British Columbia. During his undergraduate career, Jeff worked in the lab of Michael Hayden, who also supervised his PhD. After completing his PhD, Jeff moved to Boston to pursue post-doctoral work under the supervision of Marcy MacDonald. Jeff returned home to Bellingham to join the Behavioral Neuroscience program at Western Washington University in September 2010.

In addition to his scientific interest in HD, Jeff has a personal connection to the disease. His mother, Cindy, died after suffering with Huntington's disease, placing her 6 children at 50% risk of inheriting the mutation which will cause the disease. In 2003, genetic testing revealed that Jeff had inherited a mutant copy of the Huntington's disease gene from his mother. In the absence of new treatments, this means that he himself will eventually develop the disease.

Daniela Conrado, BPharm, MS, PhD

Associate Director, Quantitative Medicine, Critical Path Institute

Dr. Conrado is a pharmacist by training with over 10 years of research experience in pre-clinical or clinical pharmacology and pharmacometrics. She obtained her a master's degree in pre-clinical pharmacology (2006) and a doctoral degree in clinical pharmacology and pharmacometrics (2012, University of Florida) with research focusing on neuroscience. During her postdoctoral fellowship in the Pfizer Neuroscience Research Unit, Dr. Conrado developed a disease progression model for Alzheimer's disease using data from the Coalition Against Major Diseases consortium (CAMD, C-Path). Moreover, she conducted a model-based meta-analysis of Phase 1 studies to optimize cardiovascular safety assessment.

As a Clinical Pharmacology Lead at Pfizer, Dr. Conrado worked on Phase 1 through Phase 4 stages supporting model-informed drug development activities. In the Phase 1 stage, she performed exposure-response analysis of cardiovascular safety data to support go/no decision. In the Phase 2 stage, she performed PK/PD modeling to support dose selection in Phase 3 confirmatory trials. In Phase 3, she performed exposure-response modeling to support the FDA approval of drugs to treat inflammatory diseases. In Phase 4, she designed a pediatric pharmacokinetic study in a rare disease program. At Critical Path Institute, she is working on model-based biomarker qualification and model-informed drug development tools to optimize the design of clinical trials for drug candidates to treat diseases with high unmet medical need.

Dr. Conrado has received over 15 scientific awards and fellowships including the American Society for Clinical Pharmacology and Therapeutics (ASCPT) Presidential Trainee Award (2012), the American College of Clinical Pharmacology (ACCP) Wayne A. Colburn Memorial Award (2011, 2012), and the Oak Ridge Institute for Science and

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Education (ORISE) Research Fellowship (2012). She has published over 25 peer-reviewed journal articles and conference proceedings, and is an active member of the International Society of Pharmacometrics (ISoP) and the ASCPT.

Brian Corrigan, BSc, Pharm, PhD

Head of Clinical Pharmacology, Global Products Development at Pfizer

Brian Corrigan is Head of Clinical Pharmacology, Global Products Development at Pfizer, in Groton, Connecticut. In his role, Brian acts as the global head for Clinical Pharmacology for Pfizer and leads late stage clinical Pharmacology and pharmacometrics across sites working across all therapeutic areas.

Brian received his B.Sc, Pharmacy from the University of Alberta, Canada (1989), and Ph.D in Pharmacokinetics from the University of Alberta (1996).

Brian's work has focused on application of clinical pharmacology and pharmacometric approaches to facilitate decision making in all stages of drug development, most notably in the neurosciences and pain field. He has helped in introducing a culture of model informed drug development and decision making within Pfizer in such things as creation of a literature based meta-analysis database for use for Model Based Metanalysis.

Brian has been a long-term advocate for the clinical pharmacology and pharmacometrics community. He served as Treasurer of the Midwest Users Forum for Population Approached to Data Analysis (MUF PADA), and has been a co-organizer of multiple MUF PADA meetings in the late 90s and in the 2000s. He served on the Editorial Advisory Board for the Journal of Pharmacokinetics and Pharmacodynamics. He served as a member on the ASCPT Pharmacometrics Task Force. He has mentored numerous doctoral and post-doctoral students, and served on the thesis committee for PhD students from a number of US and European universities.

Brian has served in a number of roles in the disciplines of Clinical Pharmacology and Pharmacometrics in a number of organizations.

- President of the International Society of Pharmacometrics (ISoP) for 2016. Currently serves as Past-President on the Executive Board, ISoP.
- Programming Chair for the American Conference on Pharmacometrics (ACoP) 2013
- Served as an editorial reviewer for peer-reviewed Journals, including Clinical Pharmacology and Therapeutics, the Journal of the American Society of Clinical Pharmacology and Therapeutics, Alzheimer's and Dementia. Editorial Advisory Board, Clinical Pharmacokinetics and Pharmacodynamics (up to 2015).
- Co-chair of the modeling workgroup of the Coalition against Major Diseases (CAMD), a consortium of industry, FDA, NIH, medical associations, and patient advocacy groups working to develop common understanding of neurological degenerative diseases, 2009-present. This led to the first submissions to the US and EMA for use of a disease model as a program independent drug development tool, and development of the fit-for-purpose regulatory pathway for drug development tools.
- Steering Committee, Quantitative Systems Pharmacology Special Interest Group (QSP SIG), ISOP

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Billy Dunn, MD

Division Director, Division of Neurology Products, Food and Drug Administration (FDA)

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

Rebecca Fuller, PhD

Director, Clinical Outcomes, CHDI Foundation

Dr. Fuller has over 15 years of experience conducting cognitive research with people with movement disorders and psychiatric illness. Prior to joining CHDI Rebecca was a Clinical Scientist at Bracket, a company that supports clinical trials by providing solutions for scale management, electronic Clinical Outcomes Assessments (eCOA), rater training and quality assurance. As a faculty member at the Catholic University of America in Washington D.C., Rebecca taught cognitive psychology and neuroscience courses at the graduate and undergraduate level in addition to conducting independent research and supervising student research. She was appointed by the Governor of Maryland to serve as a commissioner on the Community Services Reimbursement Rate Commission, an independent agency within the Maryland Department of Health and Mental Hygiene which is concerned with issues regarding community services for individuals with psychiatric or developmental disorders, with particular emphasis on topics such as the rates paid to providers, measurement of quality and outcomes, uncompensated care and updating rates. She earned her PhD from the Institute of Neurology, University College London, University of London and completed postdoctoral fellowships at the Department of Psychiatry, University of Iowa Hospitals and Clinics and at the Maryland Psychiatric Research Center, University of Maryland School of Medicine. Rebecca is currently an adjunct associate professor of psychology at the University of Maryland, University College.

Emily Gantman, PhD

Director, CHDI Foundation

Dr. Gantman joined CHDI in 2017. Most recently, she was the Director and Scientific Liaison for Business Development at the New York Genome Center (NYGC). Dr. Gantman completed her PhD and postdoctoral work in Immunology and Molecular Biology at the Rockefeller University. Prior to Rockefeller, Dr. Gantman trained in the laboratory of Cellular and Developmental Biology at the NIH after graduating from the University of Pennsylvania with a BA in Mathematical Biology.

Mark Forrest Gordon, MD

Senior Director, Clinical Development, CNS Movement Disorders, Teva Pharmaceuticals

Dr. Gordon is an accomplished board-certified neurologist and Movement Disorders fellowship-trained subspecialist. He has clinical, research, and industry expertise and leadership in Huntington's disease (HD), Parkinson's disease (PD), Alzheimer's disease, and other disorders.

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As an attending in the Departments of Neurology and Psychiatry at Long Island Jewish Medical Center, Mark directed the Movement Disorders Program, including a busy clinical practice of patients with PD, HD, and other movement disorders. He served as Principal Investigator on numerous clinical trials.

In industry, Mark directed Boehringer Ingelheim's Phase III-IV pramipexole research for PD. He was medical lead on the international drug development project that resulted in market authorization of pramipexole ER for patients with PD in China. In his role as Senior Director in Clinical Development, Movement Disorders, at Teva, Mark is the Global Clinical Project Leader for the development of laquinimod to treat patients with HD.

Mark's key external roles have included Industry Co-Director of the Coalition Against Major Diseases, Advisor in the Critical Path Parkinson, and work package Co-Director in the European Medical Information Framework. He is the Industry Representative on the FDA's Peripheral and Central Nervous System Drugs Advisory Committee.

Manuel Haas, PhD

Head, Office for Central Nervous Systems, Evaluation Division, EMA

Dr. Haas is the Head of the Central Nervous System & Ophthalmology office within the Medicines Evaluation Division at the European Medicines Agency (EMA) since 2009. He trained as a clinical pharmacist and joined the EMA in 2003. His office is responsible for the management of the overall CNS products' lifecycle from initial marketing authorisation application onwards, covering the entire portfolio of products for safety, efficacy and risk management activities. His office also provides scientific support to working parties and scientific advisory groups involved in the development and evaluation of CNS medicines.

Debra Hanna, PhD

Executive Director, CPTR, Critical Path Institute

Dr. Hanna is the Executive Director of Critical Path to TB Drug Regimens (CPTR) initiative led by the Critical Path Institute and funded by the Bill & Melinda Gates Foundation. In her role as Executive Director, Dr. Hanna coordinates this global, multi-sector partnership that is driven by the contributions of 260+ members and partners. She leads the expanding tuberculosis (TB) data collaboration initiative and multiple program teams focused on accelerating the development of novel drug regimens and rapid drug susceptibility tests for TB. This work is accomplished by facilitating the advancement of innovative regulatory science approaches, qualification of novel drug development tools and biomarkers as well as the development of TB clinical trial modeling and simulation tools.

Prior to joining Critical Path Institute, Dr. Hanna spent 11 years in the Antibacterial Research and Development Unit of Pfizer Global Research and Development. She was the Research Project Leader for multiple Antibacterial Drug Development programs and also led an innovative translational research team focused on understanding pharmacodynamics and pharmacokinetic relationships for novel antibacterial agents spanning early discovery to Phase 2 development. She has contributed to 40+ peer-reviewed publications in 16 high-impact journals. Most notably, 3 journal supplements focused on TB drug-development tools, diagnostics and regulatory approaches in *Clinical Infectious Diseases* and *Journal of Infectious Diseases*.

Debra received her doctorate in Microbiology from North Carolina State University, and her Bachelor of Science degree in Microbiology and Immunology from Colorado State University. Dr. Hanna's passion for tuberculosis

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research began with her postdoctoral fellowship at the University of California, San Diego and motivated her to pursue a career in antibiotic drug development. As Executive Director of CPTR, she has expanded the reach of C-Path partnerships with key stakeholders in the global health community and positioned the organization as a global leader in data collaboration and consensus-based science approaches designed to deliver outcomes.

Steve Hersch, MD, PhD

Senior Director, Clinical Development, Voyager Therapeutics

Dr. Hersch is Senior Director for Clinical Development at Voyager Therapeutics, Professor of Neurology at Massachusetts General Hospital and Harvard Medical School, and Director of the Laboratory of Neurodegeneration and Neurotherapeutics within the MassGeneral Institute for Neurodegeneration. He earned his Ph.D. at Boston University School of Medicine (BUSM), completed a postdoctoral fellowship at Harvard Medical School, and earned his MD degree at Boston University. He completed his residency in Neurology and Fellowship in Behavioral Neurology at Emory University School of Medicine where he joined the faculty and advanced to Associate Professor of Neurology. At Emory, he founded the first Huntington's Disease Society of America (HDSA) Center of Excellence and led the development of this program for the HDSA, which supports multidisciplinary care in dozens of academic clinics across the United States. He was recruited in 2001 to establish a translational research laboratory in the MassGeneral Institute for Neurodegeneration and to lead the HD clinical program at MGH. He joined Voyager Therapeutics in 2017 where he works on early clinical development for HD and other pipeline programs

Dr. Hersch's laboratory research has included studying the synaptic organization and molecular pharmacology of the cerebral cortex and basal ganglia; the pathophysiology of HD; the toxic properties of the mutant huntingtin protein; the discovery and validation of potential treatments for HD in preclinical models; and the development of translational and clinical biomarkers. The Hersch laboratory's preclinical studies of creatine, cystamine, and phenylbutyrate in HD mouse models helped provide a basis for clinical trials in HD patients. Work on metal dysregulation in HD models and patients helped provide the rationale for a successful phase II trial of PBT2 (PRANA Biotechnology). Transcriptomic, metabolomic and protein biomarkers for HD have been developed in the Hersch lab that have been deployed in clinical research, including assays for measuring the normal and mutant huntingtin proteins.

Dr. Hersch has authored more than 125 peer-reviewed publications, has served as a reviewer or editorial board member for numerous scientific journals, has served on many institutional, Foundation, and NIH scientific and grant review committees, and is a member of the Advisory Council for the NIH National Center for Complementary and Integrative Health.

Dr. Hersch co-chaired the Huntington Study Group for many years, an International Consortium dedicated to conducting clinical research and therapeutic trials with the goal of developing treatments for HD. He has participated in developing standard clinical instruments for HD and has served as a site investigator, steering committee member, or principal investigator on many NIH or commercially sponsored clinical and observational studies. He was the PI of a series of phase II and phase III studies of creatine, of the first secondary prevention trial conducted in at-risk HD subjects, and the first multicenter trial of an epigenetic therapy for HD (phenylbutyrate).

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He is the academic principal investigator for a therapeutic trial of a novel treatment for irritability sponsored by Azevan Pharmaceuticals and conducted by the NIH NEURONEXT Network and serves on its executive committee.

Karl Kieburtz, MD, MPH

Professor of Neurology at the University of Rochester

Dr. Kieburtz is a Professor of Neurology at the University of Rochester. He was the founding Director of the Center for Human Experimental Therapeutics, which conducts learning phase clinical trials in a wide spectrum of disorders. He was also the initial Robert J. Joynt Professor in Neurology, and served as the Senior Associate Dean for Clinical Research and Director of the Clinical & Translational Science Institute, where he continues to have a senior advisory role. Dr. Kieburtz's primary clinical and research interests are neurodegenerative diseases affecting the basal ganglia. He was the principal investigator (PI) for the NINDS sponsored trials of neuroprotective agents for PD (NET-PD), served as the Chair of the Parkinson Study Group, and directs the Clinical Core for the Fox Foundation sponsored Parkinson Progression Marker Initiative. He has served as the PI for many multicenter clinical trials in Huntington disease (HD), including the first NIH-funded multicenter trial in HD (CARE HD), and the initial dosage ranging trials of Pridopidine. He previously served on and chaired the FDA Advisory Committee on Peripheral and Central Nervous System Disorders. In 2009, he was one of the co-founders of Clintrex LLC, and continues to serve as President of the organization.

Kevin Krudys, PhD

Division of Pharmacometrics, Office of Clinical Pharmacology, FDA

Dr. Krudys is a Team Leader in the Division of Pharmacometrics in the Office of Clinical Pharmacology at the FDA. His team is responsible for reviewing the application of model informed drug development in the therapeutic areas of neurology, psychiatry, anesthesia, analgesia and addiction. He previously served as the Scientific Lead of the QT Interdisciplinary Review Team and is currently a member of the Pediatric Review Committee.

Georg Bernhard Landwehrmeyer, MD, FRCP

Professor of Neurology, Ulm University Hospital, Ulm, Baden-Württemberg, Germany

Dr. Landwehrmeyer is Full Professor of Neurology at Ulm University Hospital, Dept. of Neurology where the Central Coordination of the European Huntington's Disease Network (EHDN) is situated. 2004 he was instrumental in founding EHDN and served as chairman of the Executive Committee until 2014. EHDN serves as a platform for professionals, people affected by Huntington's disease (HD), and their relatives to facilitate working together throughout Europe and conducts large prospective natural history studies in HD, e.g. the REGISTRY study. EHDN and REGISTRY is generously funded by the CHDI Foundation (USA). He received his MD degree and Doctoral Degree from the Albert-Ludwigs-University, Freiburg. He was trained at the Royal Victoria Hospital, Queen's University, Belfast, at the Kantonsspital, Basel and worked as post-Doc from 1993 -1996 at MGH, Harvard Medical School, Boston. From 1995–1999, he was staff member at Albert-Ludwigs-University (Dept. Neurology & Psychiatry). 1999 he received Board Certification in Neurology, 2000 the Venia Legendi and full Professorship ('C3'). He served as Principal Investigator in numerous HD trials and is PI of the CHDI-sponsored Enroll-HD study, a prospective longitudinal observational study on HD and a clinical research platform with a worldwide reach that annually collects phenotypical clinical data and biomaterials.

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Blair R. Leavitt, MDCM, FRCP(C)
University of British Columbia

Dr. Leavitt is currently Interim Director and Senior Scientist at the Centre for Molecular Medicine and Therapeutics and a full Professor in the Department of Medical Genetics & the Department of Medicine, Division of Neurology (Associate) at the University of British Columbia. Dr. Leavitt completed his medical degree at McGill, medical internship at Columbia-Presbyterian, neurology residency at Cornell and Harvard. While in Boston, he completed a basic neuroscience research fellowship at Harvard Medical School and Children's Hospital of Boston. Blair is a consulting neurologist and Director of Research at the UBC Centre for Huntington's Disease. A scientist and physician, Dr. Leavitt's time (both clinical and research) is dedicated to developing new treatments for genetic brain disorders such as Huntington's disease. He also works on other neurodegenerative diseases including amyotrophic lateral sclerosis and Frontotemporal dementia. Dr. Leavitt is currently the Director of the CMMT Transgenic Animal Facility, the Co-Chair of the Huntington's study group and a founding Editor-in-Chief of *The Journal of Huntington's Disease*.

Jeff D. Long, PhD
Professor of Psychiatry and Biostatistics at the University of Iowa

Dr. Long is a Professor in the Department of Psychiatry (primary) and the Department of Biostatistics (secondary) at the University of Iowa. His focus is indexing disease progression in neurodegenerative disorders, especially Huntington's disease (HD). Dr. Long has extensive experience analyzing data from the large observational HD studies of PREDICT-HD, TRACK-HD, TRACK-ON, and Enroll-HD. This work has helped to characterize the natural history of HD and inform the planning of clinical trials.

Amrita Mohan, PhD
Director, Clinical Bioinformatics, CHDI Foundation

Dr. Mohan joined CHDI Foundation in 2013 and is Director, Clinical Bioinformatics. In her current role she oversees a portfolio of computational modeling efforts relying on Huntington's disease clinical datasets. Prior to joining CHDI, Dr. Mohan held several scientific and technical positions of increasing responsibility at OSI Pharmaceuticals/Astellas Pharma.

Dr. Mohan received a PhD in Informatics from Indiana University where she specialized in the application of machine learning and statistical methods to molecular and clinical datasets.

Mike Panzara, MD, MPH
Franchise Lead, Neurology, Wave Life Sciences

Dr. Michael A. Panzara has been Franchise Lead of Neurology at Wave Life Sciences Ltd. since July 2016. Dr. Panzara oversees Wave's portfolio of neurological therapeutic candidates, from target discovery through clinical development, regulatory approval and post-approval activities. Dr. Panzara has more than 16 years of experience in key leadership roles in neurological drug development, regulatory approval and lifecycle management. Dr. Panzara held the role of Group Vice President and Head of Multiple Sclerosis, Neurology and Ophthalmology Therapeutic Areas in Global Development at Sanofi Genzyme. During his time at Sanofi Genzyme, Dr. Panzara oversaw global regulatory approvals of the multiple sclerosis (MS) drugs LEMTRADA (alemtuzumb) and AUBAGIO

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(teriflunomide), managed the neurological and ophthalmological portfolios across Sanofi which included a diverse pipeline of small molecules and biologics, and led global strategy and execution of development plans for neurology and ophthalmology candidates from preclinical development through regulatory approval. Prior to joining Sanofi Genzyme, Dr. Panzara served as Vice President and Chief Medical Officer of Neurology at Biogen. During his time there, he served as the global clinical lead for the development of TYSABRI (natalizumab) for multiple sclerosis, overseeing its clinical program and global approvals. He managed clinical development activities for all late-stage MS products including AVONEX (interferon beta-1a), PLEGRIDY (PEG-interferon beta-1a), and TECFIDERA (oral dimethylfumarate). Dr. Panzara received his undergraduate degree from the University of Pennsylvania and medical degree from Stanford University School of Medicine. He trained in neurology at Massachusetts General Hospital, received his post-doctoral training in immunology and rheumatology at Brigham and Women's Hospital, and received his MPH from the Harvard School of Public Health.

Gerald (Dave) Podskalny, DO, MPHS

Cross Disciplinary Team Leader Division of Neurology Products, FDA

Dr. Podskalny received a Bachelor's of Science degree in Biology from Rutgers University. He went on to receive a second undergraduate degree in Physical Therapy at the State University of New York at Buffalo, a Doctor of Osteopathy (DO) degree from the New York College of Osteopathic Medicine and a Master Degree in Public Health Sciences (MPHS) from Penn State University. Dr. Podskalny completed Neurology Residency and Fellowship in Movement Disorders at Albany Medical College. He was a full-time faculty member at Albany Medical College, the University of Medicine and Dentistry of New Jersey and the Penn State-Hershey College of Medicine. Dr. Podskalny joined the Division of Neurology Products at FDA in March 2007, where he is currently a Cross Disciplinary Team Leader.

Bernard M. Ravina, MD, MS

Chief Medical Officer, Voyager Therapeutics

Dr. Ravina is Chief Medical Officer of Voyager Therapeutics, a leading AAV gene therapy company focused on the treatment of severe neurological disorders. Dr. Ravina has extensive expertise across a number of neurological disorders and more than 15 years of clinical research experience in government, academia, and industry. Dr. Ravina holds an M.D. from the Johns Hopkins University School of Medicine and a Masters in Clinical Epidemiology and Biostatistics from the University of Pennsylvania, where he also completed residency training in neurology and a fellowship in Parkinson's disease and movement disorders. After training, Dr. Ravina moved to NINDS/NIH to become Program Director in the Clinical Trials Group and Investigator in the Neurogenetics Branch. He then moved to the University of Rochester School of Medicine to become Director of the Movement and Inherited Neurological Disorders Unit, Associate Professor of Neurology, and Associate Chair of Neurology for Clinical Research. Prior to Voyager, Dr. Ravina entered the biotechnology field and was Medical Director in Clinical Development at Biogen Idec. There, he worked on both small molecule drugs and biologics for the treatment of neurological disorders and was responsible for biomarker and clinical development plans in Parkinson's disease, stroke, and neuropathic pain. Dr. Ravina is the author of more than 100 scientific publications and serves on the Scientific Advisory Boards of the Michael J. Fox Foundation, Friedreich Ataxia Research Alliance, and Hereditary Disease Foundation.

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Klaus Romero, MD MS FCP

Director, Clinical Pharmacology

Dr. Romero is a clinical pharmacologist and epidemiologist by training with 15 years combined experience in academic clinical research. He is a fellow of the American College of Clinical Pharmacology and the American Society for Clinical Pharmacology and Therapeutics. He has conducted research on endemic channels for non-steroidal anti-inflammatory drug-related gastropathy, antibiotic-related dysglycemia, drug-induced QT prolongation, pharmacoepidemiology and patient education. Dr. Romero has been with C-Path since January of 2008, where he has helped lead clinical pharmacology, pharmacoepidemiology and modeling and simulation projects for the Coalition Against Major Diseases, the Polycystic Kidney Disease Outcomes Consortium and the Critical Path to TB Drug Regimens Consortium, 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. He is fluid in English, Spanish, German and Portuguese, and has published in the areas of clinical pharmacology, pharmacometrics, cardiovascular drug safety and pharmacoepidemiology.

Charles Sabine

Patient Advocate

Emmy-award winning TV journalist, Charles Sabine, worked for the us network NBC news for 26 years.

That career took him; via twelve wars, six revolutions, and four earthquakes; to most of the news events of Europe, the Middle East, Africa and Asia since the early 1980s. There, he learnt first-hand the extraordinary limits that the human spirit is capable of reaching, in the face of tragedy inflicted by both nature and mankind.

In 2008, he decided to put the lessons of those experiences to a different use, when he became a pioneering spokesman for freedom of scientific research, and sufferers of degenerative brain illnesses - in particular, Huntington's disease, which has ravaged his family.

That role has led to Sabine speaking at prestigious venues across the world, among them; the European and British parliaments; the Royal Institution in London and the World Congress on Freedom of Scientific Research.

Cristina Sampaio, MD, PhD

Chief Medical Officer, CHDI Foundation

Professor Cristina Sampaio MD, PhD joined CHDI Foundation 6 years ago. She also holds the position of Professor of Clinical Pharmacology and Therapeutics at Faculdade de Medicina de Lisboa (currently on unpaid leave). At CHDI Professor Sampaio oversees an extensive portfolio of clinical projects ranging from experimental medicine, through biomarker and rating scale development to support drug development activities, to the development and maintenance of a global clinical research platform, Enroll-HD. Professor Sampaio spent 25 years of her career in academia where her primary research interests centered on clinical research methodology, clinical trial design, and related aspects of meta-research applied to movement disorders. Together with several colleagues she founded the Cochrane Movement Disorders Group and became its coordinating editor in 1996, a position that she has shared with Professor Joao Costa since 2013. Professor Sampaio published 170 peer review papers and book chapters. From 1998 to 2011, Professor Sampaio was a member of the Committee on Human Medicinal Products and of the Scientific Advice Working Party at the European Medicines Agency. During this period, she had a very active role in

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the development of the standards of regulatory science for CNS medicinal products in the European Union. She was rapporteur, coordinator, or assessor of over 400 medicinal products files submitted to EMA for licensing or scientific advice and she coordinated the first clinical biomarker qualification in the EU. Professor Sampaio obtained her MD in 1986 and her PhD in clinical pharmacology in 1997 from the University of Lisbon. She is a board-certified clinical pharmacologist, receiving neurological training in the neurology department of Hospital St Maria in Lisbon. She was a staff member of the Movement Disorders Clinic from 1988 to 2011, President of the Portuguese Movement Disorders Society 2008–2012, and Chair of the Evidence-based Medicine Committee of the International Parkinson and Movement Disorder Society 2010–2014.

Scott Schobel, MD, MSc

Transitional Medicine Leader, F. Hoffmann-La Roche

Scott Schobel, MD, MSc, is associate group medical director and clinical science leader for the IONIS/Roche HTT Rx program at Roche neuroscience, Basel, Switzerland. He is responsible for clinical studies for the program post the IONIS-led Phase I study completion. Prior to joining Roche in 2013, Scott completed elective rotations in neurology & neurosurgery at UNC-Chapel Hill, followed by internship year in medicine & neurology, followed by psychiatric residency training, research fellowship, and early faculty at Columbia University in the city of New York from 2001–2012. While at Columbia, Scott pursued cross-platform training in basic science, brain imaging, clinical research, and biostatistics. He then applied this integrative approach to cross-species brain-imaging focused prospective studies in youths at risk for psychotic disorders, as well as in rodent models of disease, including testing of novel pharmacological interventions. His work led to recognition from the Brain and Behavior Research Foundation, extramural NIH funding, and high impact publications in *Neuron* and *JAMA Psychiatry*.

Scott then chose to join industry to help speed biomarker-based drug development in CNS disorders, with the goal of working collaboratively with industrial partners, the academy, NIH, and NGOs to bring more effective therapeutics to severe neurological and psychiatric disorders. Scott is presently focused on endpoint development in HD, including an external collaboration with investigators from UCL, Univ. of Iowa, and the Huntington Study Group, and an internal collaboration on extending clinical measures in HD to digital sensor-based platforms.

Eric Siemers, MD

Distinguished Medical Fellow of the Alzheimer's Disease Global Development Team at Eli Lilly and Company

Dr. Siemers is a Distinguished Medical Fellow of the Alzheimer's Disease Global Development Team at Eli Lilly and Company. He earned his MD with highest distinction from the Indiana University School of Medicine in 1982. After an internship in the Department of Internal Medicine at the Indiana University School of Medicine, he completed his residency in the Department of Neurology in 1986. Prior to joining Lilly, he founded and headed the Indiana University Movement Disorder Clinic; his previous research included investigations of Parkinson's disease and Huntington's disease, and he established one of the first centers for surgical PD treatments in the US. Dr. Siemers currently directs late stage clinical research efforts at Lilly concerning investigational treatments for Alzheimer's disease, and is more broadly involved with other neurological indications such as Parkinson's disease. Major research interests include the use of biomarkers in investigational drug research and the development of trial designs that broadly characterize the effects of investigational drugs on chronic diseases. Dr. Siemers is a founding member of the Alzheimer's Association Research Roundtable and is the immediate-past Chair. He is a member of

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the Steering Committee for the Alzheimer's Disease Neuroimaging Initiative (ADNI), which is funded by the National Institute on Aging and a consortium of pharmaceutical companies. He served as the chair of the Industry Scientific Advisory Board for ADNI in 2007 and previously served as a member of the Resource Allocation Request Committee. Dr. Siemers participated as a member of the NIA/Alzheimer's Association working group that proposed criteria for preclinical Alzheimer's disease in 2011 and is a member of the current working group that will evaluate the use of biomarkers in the evaluation of the Alzheimer's disease continuum. He is a past member of the Board of Directors of the American Society of Experimental Neurotherapeutics.

Glenn Stebbins, PhD

Professor, Department of Neurological Sciences, Rush University Medical Center

Glenn Stebbins, PhD, is a professor in the Department of Neurological Sciences at Rush University Medical Center. He has extensive experience with rating scale testing and development, having participated in the development of the Unified Dyskinesia Rating Scale (UDysRS) as well as serving on the Steering Committee of the Movement Disorder Society revision of the Unified Parkinson's Disease Rating Scale (MDS-UPDRS). He is actively involved in the development of validated non-English translations of rating scales including the UDysRS and MDS-UPDRS. He currently serves as the co-chairperson of the Committee on Rating Scale Development for the International Parkinson and Movement Disorder Society and as consultant and chief statistician to numerous clinical studies in Parkinson's disease and other movement disorders.

Dr. Stebbins' research interests center on the effects of normal and pathological aging on cognitive function in humans. Using advanced neuroimaging (e.g., fMRI, Diffusion Tensor Imaging, SPECT) and behavioral techniques, studies are designed to assess the relationship between structural and functional changes in the CNS and age-related behavioral changes. Specific areas of interest include the contribution of white matter microstructural integrity to cortical and subcortical gray matter function during executive and declarative memory performance.

Current research focuses on four areas: 1) Dissociation of cortical and sub-cortical contributions of executive working memory impairments in patients with movement disorders. 2) Delineation of medial temporal lobe functional activity during declarative memory performance in patients with Alzheimer's disease, mild cognitive impairment, 3). Examining the contribution of "normal appearing" white matter integrity to perceptual motor processing speed and executive working memory performance in patients with ischemic stroke. 4). Decomposing the influence of lesion pathology on cognitive function in patients with multiple sclerosis.

Diane Stephenson, PhD

Executive Director, Critical Path for Parkinson's, Critical Path Institute

Dr. Stephenson served as the Executive Director of CAMD from 2011 to 2015, and has since taken on the exciting role of Executive Director of the Critical Path for Parkinson's (CPP) consortium funded by Parkinson's UK.

Dr. Stephenson is a neuroscientist by training with 30 years combined experience in academic neuroscience and drug discovery. She is passionate about translational science and has a long-time dedication to the discovery of therapies to treat diseases of the nervous system. Dr. Stephenson received her undergraduate degree in Biochemistry at University of California, Santa Barbara and her Ph.D. in Medical Neurobiology from Indiana University. In her academic career, Dr. Stephenson focused her research on Amyotrophic Lateral Sclerosis and

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Alzheimer's disease (AD), while in industry she focused on drug discovery for Alzheimer's disease, stroke, Parkinson's disease and Autism Spectrum Disorders. From 1981-1989, she was an associate research scientist at the ALS and Neuromuscular Research Foundation in San Francisco. After joining industry, she collaborated with pioneers of the amyloid hypothesis in drug development for AD. She led animal model characterization (primate and rodent toxin and genetic models) and evaluation of drug candidates for Parkinson's disease for many years while in industry. As an ambassador for public-private partnerships, she has initiated numerous external academic collaborations including worldwide alliances. Dr. Stephenson joined Critical Path Institute as Director of the Coalition Against Major Diseases (CAMD) in August 2011. As Executive Director of CPP, she has grown the consortium membership to a world class multidisciplinary team comprised of academic experts, industry scientists, patient advocacy groups and regulatory experts collectively aimed at accelerating treatments for Parkinson's.

Julie Stout, PhD

Director of the Clinical and Cognitive Neuroscience Laboratory, Monash University

Dr. Stout is a Professor in the School of Psychological Sciences, Director of the Attention and Memory Program in the Monash Institute of Cognitive and Clinical Neuroscience, and Vice President of the Monash University Academic Board. She specialises in cognitive assessment in multisite observational studies and clinical trials, and co-developed the HD-CAB, a cognitive assessment tool for clinical trials. She led or leads the cognitive component of the Track-HD studies, Predict-HD (through 2009), and the following clinical trials: Reach2HD, Pride-HD, Legato-HD, Ionis-HD, and Signal-HD. She is director of a spin-out company that implements cognitive assessments in clinical trials, and on the Board of Directors of the Huntington's Study Group (Treasurer).

Charles S. Venuto, PharmD

Assistant Professor of Neurology, University of Rochester Medical Center

Dr. Venuto is a clinical pharmacologist specializing in modeling and simulating the exposure and response of drugs in humans. He received his Doctorate of Pharmacy from the University at Buffalo and completed a post-doctoral fellowship in Experimental Therapeutics within the University of Rochester's Department of Neurology and Center for Human Experimental Therapeutics. His research focuses on the development of mathematical models to describe the time course and progression of neurodegenerative diseases and drug effects in order to design more informative clinical trials and enhance the analysis of longitudinal clinical trial data. The modeling approaches used are based on his previous work in population pharmacokinetics/pharmacodynamics in which patient-specific demographics and therapeutic features were used to explain variability in drug exposure-response relationships. Dr. Venuto's collaborations include The Michael J. Fox Foundation, the AIDS Clinical Trials Group, and the CHDI Foundation, to apply clinical pharmacology modeling and simulation tools towards the improvement of therapeutic drug development.

Louise Vetter

President and Chief Executive Officer, Huntington's Disease Society of America (HDSA)

Louise Vetter is President & Chief Executive Officer of the Huntington's Disease Society of America (HDSA), the largest public not-profit organization devoted to the fight against Huntington's disease (HD). Since joining HDSA in 2009, she has led the expansion of the Society's reach with new initiatives to strengthen the web of support for HD

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families, advocate for better access to care for those affected by the disease, improve physician understanding of HD, and support research to bring new treatments to HD families.

A leader in patient advocacy, Ms. Vetter serves as Treasurer of the Board of Directors of the American Brain Coalition, member of the Board of the International Huntington Association, member of the Finance Committee of the National Health Council, and an editorial advisor to the Rare Disease Report.

At the heart of her experience is a firm passion for helping people face health challenges head on, always with dignity, understanding and hope.

Andrew Wood, PhD **Vice President, Clinical Neuroimaging Research, CHDI Foundation**

Dr. Wood has 20 years of drug development experience spanning discovery research, translational medicine and clinical trials. Prior to joining CHDI in 2014 Dr. Wood was Senior Director, Head of Clinical Research at Ono Pharma USA where he managed clinical development and biomarker planning for a diverse portfolio of clinical projects and led global phase II proof of concept and neuroimaging studies for CNS drug targets. Prior to joining Ono, Dr. Wood was Associate Director and Wyeth (now Pfizer) Neurosciences Discovery lead for the Stroke and MS Early Clinical Development Team and Translational Neurosciences lead for Stroke and Parkinson's disease. Before transitioning to translational sciences Dr. Wood was head of the Cellular Neurodegeneration and Neuroimaging group at Wyeth Neurosciences where he investigated regenerative signaling mechanisms for stroke and MS and developed contextual in vitro models to study protein aggregate diseases. Prior to joining industry, Dr. Wood spent several years in academic neuroscience, first as an NIH Research Fellow at the Institute of Neuroscience, Eugene Oregon studying cell fate determination in neural crest and subsequently as a Senior Research Fellow at the University of Cambridge Neurology Unit investigating mechanisms of oligodendrocyte injury and repair. He holds a PhD from Southampton University, UK in Developmental Biology and a BSc in Biological Sciences from Plymouth University, UK.

Issam Zineh, Pharm.D., M.P.H. **Director, Office of Clinical Pharmacology and Co-Director of the Biomarker Qualification Program,** **Office of Translational Sciences, CDER, FDA**

Dr. Zineh is Director, Office of Clinical Pharmacology (OCP), and Co-Director of the Biomarker Qualification Program, Office of Translational Sciences, CDER, U.S. FDA. From 2008-2012, Dr. Zineh was the Associate Director for Genomics in OCP. He is an experienced clinical pharmacist who was formerly on the faculty of the University of Florida (UF) Colleges of Pharmacy and Medicine and Associate Director of the UF Center for Pharmacogenomics. Dr. Zineh received his PharmD from Northeastern University and completed his residency at Duke University Medical Center. He did a fellowship in cardiovascular pharmacogenomics at UF where he also obtained his MPH in Health Policy and Management. He is a recognized expert in the field of clinical pharmacology, pharmacotherapy, and pharmacogenomics. As Director of OCP, Dr. Zineh is a member of the CDER Senior Management Team and leads a staff of over 200 regulatory scientists, project managers, and administrative staff in FDA's efforts to enhance drug development and promote regulatory innovation through applied clinical pharmacology.