ALS Community Therapy Development and Regulatory Pathways Workshop

Panelist and Speaker Bios

Jinsy Andrews, MD, MSc, *Columbia University*

Jinsy A. Andrews, MD, MSc is an Assistant Professor of Neurology, in the Division of Neuromuscular Medicine and currently serves as the Director of Neuromuscular Clinical Trials in the Department of Neurology. In additional to overseeing neuromuscular clinical trials, she cares for patients with neuromuscular disorders, primarily with amyotrophic lateral sclerosis (ALS). Prior to this position, she helped develop and direct a clinical trials unit for neurological diseases at Hospital for Special Care/University of Connecticut. There, she served as the Director of Clinical Trials and Co-director of the MDA/ALS multidisciplinary clinic. More recently, Dr. Andrews was Senior Director of Clinical Research and Development and Head of Neuromuscular Therapeutics at Cytokinetics where she focused on developing investigational therapies for neuromuscular diseases including Spinal Muscular Atrophy (SMA) and ALS. She has experience in conducting human clinical trials in neuromuscular disorders from Phase 1 to Phase 3 from both the academic and industry settings.

Dr. Andrews received her BS from Union College, her MSc in Biostatistics (Patient-Oriented Research) from Columbia University's Mailman School of Public Health, and her MD from Albany Medical College. Dr. Andrews completed her residency in Neurology at the University of Connecticut and completed fellowship training in both Neuromuscular Disease and Clinical Neurophysiology at Columbia. Dr. Andrews is board certified in Neurology, Neuromuscular Disease, and Electrodiagnostic Medicine.

Calaneet Balas, MS, MBA, President and CEO, *ALS Association*

Calaneet Balas became CEO and President of The ALS Association in December 2017. She joined the organization in June 2016 as Chief of Strategy, leading all three mission areas including global research, public policy and care services, which work in an integrated fashion to find a cure, advance treatments and enhance the quality of life for people living with ALS.

Prior to joining the fight against ALS, Calaneet served as Chief of Executive Officer of the Ovarian Cancer National Alliance (OCNA). In this role she had numerous accomplishments, including:

- Helping to raise the profile of ovarian cancer by testifying before Congress and speaking with major news outlets, including the New York Times, Washington Post and NBC Nightly News, about the needs of the ovarian cancer community.
- Initiating the first ever bi-partisan Congressional Ovarian Cancer Caucus and overseeing "50 States of Teal," a state report card that called out individual states on their coverage of women's health care implementation through the Affordable Care Act (ACA).

- Collaborating with a partner organization to create the largest global organization dedicated to ovarian cancer research, advocacy and patients, Ovarian Cancer Research Fund Alliance, which merged in January of 2016.

Ms. Balas also worked at the Arthritis Foundation, where she was part of an executive team that integrated and restructured four separately incorporated chapters into one region representing the Arthritis Foundation. She served as Chief Strategy Officer of the newly created Mid-Atlantic Region of the Arthritis Foundation from 2010 until September 2012. Previously, she served as President and CEO of the Arthritis Foundation's Metro DC Chapter from 2005-2009, bringing financial stability to the chapter and increasing its fundraising efforts.

Ms. Balas holds a Master of Business Administration from Herriot-Watt University, a Master of Science in Human Movement Science Education from the University of Memphis, and a Bachelor of Science in Exercise Science from Truman State University.

Benjamin Rix Brooks, MD, *Carolinas Healthcare System*

Benjamin Rix Brooks, MD, is Medical Director, Carolinas Neuromuscular / ALS – MDA Care Center in the Department of Neurology – Carolinas Medical Center – Atrium Health – Neurosciences Institute and Professor of Neurology at the University of North Carolina School of Medicine – Charlotte Campus. Professor Brooks was a founding member of the World Federation of Neurology Research Group on Motor Neuron Diseases and coordinated the workshops leading to the El Escorial and Airlie House Criteria for the Diagnosis of ALS and the Airlie House Guidelines on Therapeutic Trials in ALS. He was the principal investigator for the national ALS study group that developed the ALS Functional Rating Scale [ALSFRS] and the ALS Functional Rating Scale-Revised [ALSFRS-R], now a standard clinimetric scale used in ALS clinical trials. Professor Brooks was a leader of the North America – South America multi-center STAR clinical trial, employing at that time, a novel clinical endpoint in ALS that led to the Food and Drug Administration approval of Nuedexta for the treatment of pseudobulbar affect in ALS and other neurological conditions. The Carolinas Neuromuscular / ALS – MDA Care Center was the first ALS Clinic in the United States to achieve Joint Commission Disease-Specific Care Certification in Amyotrophic Lateral Sclerosis in 2012. Dr Brooks received the International Alliance of ALS and MND Associations Forbes Norris Award in 2012.

Lucie Bruijn, PhD, MBA, Chief Scientist, *ALS Association*

Lucie Bruijn, PhD joined The ALS Association in January 2001 and is currently the Chief Scientist. Prior to that, Dr. Bruijn led a team at Bristol Myers Squibb developing in vitro and in vivo model systems for neurodegenerative disease. Realizing the potential of stem cell therapy for neurodegenerative diseases, her team worked with experts in academia to establish stem cell studies at Bristol Myers Squibb.

Dr. Bruijn received her Bachelor's degree in Pharmacy at Rhodes University, South Africa. She received a Master's degree in Neuroscience and a Ph.D. in Biochemistry, specializing in disease mechanisms of Alzheimer's disease, at the University of London, United Kingdom. She received her MBA at Imperial College, London, United Kingdom. She joined Dr. Don Cleveland's laboratory in 1994 where she developed and characterized a mouse model of ALS (mice expressing the familial-linked SOD1 mutation). Using this model her studies focused on disease mechanisms. In addition, in collaboration with Dr. Robert Brown she looked for neurofilament mutations in familial and sporadic ALS patients.
At The ALS Association, Dr. Bruijn leads a global ALS research effort, Translational Research to Advance Therapies for ALS (TREAT ALS™) with the goal to move treatment options from "bench to bedside." She has made it a priority to collaborate with other funding agencies, in particular The National Institutes of Health, The Department of Defense (DoD) and many other not-for-profit ALS organizations, as well as other foundations focusing on neurodegenerative research. She has previously served on the NINDS advisory council, as chair of the DoD's ALS Research Program (ALSRP) integration panel and is currently a member of the ALSRP integration panel. These collaborations ensure that increased dollars are spent on ALS research. She is involved in project development, encouraging partnerships with academia and biotech, and has played a key role in forging collaborations amongst investigators. It is her strong belief that only through collaboration among a wide range of disciplines will we be successful in changing the course of ALS and finding a cure.

Through participation at scientific meetings both nationally and internationally ALSA receives wide-spread recognition amongst the scientific community. Dr. Bruijn represents The ALS Association on several scientific and research committees world-wide and acts as advisor to scientists, government officials and industry leaders seeking council in the field of ALS research. She also served as Vice Chair on the Steering Committee of the Community Developed ALS Guidance. Dr. Bruijn continues to publish in the field in peer-reviewed journals and remains actively engaged in understanding the most recent research developments.

Christopher S. Coffey, PhD, *University of Iowa*

Dr. Christopher S. Coffey joined the faculty at the University of Iowa in fall 2009 as a Professor in the Department of Biostatistics and became the Director of the Clinical Trials Statistical and Data Management Center (CTSDMC) in 2010. He received his PhD in biostatistics from the University of North Carolina at Chapel Hill in 1999 and has nearly 20 years of experience providing data management and statistical support to clinical trials. Dr. Coffey serves as the PI of the Data Coordinating Center for several large clinical studies, including the NINDS-funded NeuroNEXT Network. Dr. Coffey also serves as the head of the Statistics Core for the Parkinson's Progression Markers Initiative, and is co-PI of the NINDS-funded Clinical Trials Methodology in Neurology short course. Dr. Coffey has served as the primary statistician for multi-site trials in Huntington's disease, hypertension, multiple sclerosis, myasthenia gravis, obesity, pediatric migraine, spinal muscular atrophy, stroke, and traumatic brain injury. He is a past member of the NINDS NSD-K clinical trials study section, a Fellow of both the Society

Clinical Trials and American Statistical Association, and serves on a number of Data and Safety Monitoring Boards. Dr. Coffey has published extensively in the areas of clinical trial design, especially in the area of adaptive designs.

Merit Cudkowicz, MD, *Massachusetts General Hospital*

Dr. Merit Cudkowicz is the Chief of the Massachusetts General Hospital Neurology Service and the Julieanne Dorn Professor of Neurology at Harvard Medical School in Boston. Dr. Cudkowicz's research and clinical activities are dedicated to the study and treatment of patients with amyotrophic lateral sclerosis (ALS). She serves as director of the Massachusetts General Hospital ALS Clinic and the Massachusetts General Hospital Neurological Clinical Research Institute. She is one of the founders and co-directors of the Northeast ALS Consortium, a group of more than 100 clinical sites in the United States and Canada dedicated to performing collaborative, academic-led clinical ALS trials.

She is Principal Investigator of the Clinical Coordination Center for the National Institute of Neurological Disorders and Stroke's Neurology Network of Excellence in Clinical Trials (NeuroNEXT). She is currently leading efforts at 25 different centers that conduct high-impact Phase 2 trials in neurology, hoping to speed trials by seven months to a year. The NeuroNEXT network established an academic central internal review board led by Massachusetts General and serves as a system for efficient study start-ups.

A dedicated educator, Dr. Cudkowicz mentors many young neurologists in clinical investigation of ALS and related neurodegenerative disorders. She also serves on the medical advisory board for the Muscular Dystrophy Association and the Massachusetts Amyotrophic Lateral Sclerosis Association.

Dr. Cudkowicz completed her medical degree in the Health Science and Technology program of Harvard Medical School. She served her internship at Beth Israel Hospital in New York and her neurology residency and fellowship at Massachusetts General. She also obtained a master's degree in Clinical Epidemiology from the Harvard School of Public Health.

David L. Ennist, PhD, MBA, *Origent Data Sciences, Inc.*

Dave is a biomedical scientist with broad experience in the life science, biotechnology and pharma industries. He has published over 30 scientific articles in journals including Blood, PNAS, Amyotrophic Lateral Sclerosis & Frontotemporal Degeneration and Annals of Clinical & Translational Neurology. Dave's expertise encompasses drug development, molecular biology, immunology, gene therapy, data analytics and research management. He serves as Chief Science Officer for Origent Data Sciences, Inc., headquartered in Washington, DC.

Dave is formerly Scientific Expert and Program Team Head for Novartis and Genetic Therapy, Inc., where he transitioned a number of therapeutic candidates from the bench to phase 1 clinical testing. In addition, he also was previously Principal at DLE Consulting, where he consulted on drug development products from preclinical through phase 4 and Director of Digital Resources for the American Society for Cell Biology, where he developed the ASCB Cell Image Library now maintained by the Center for Research in Biological Systems at UCSD.

Dave holds a Bachelor's degree from the University of Rochester, an MBA from the Johns Hopkins Carey Business School and a Doctorate from The Ohio State University. He was a Staff Fellow and National Academy of Sciences Research Associate at the National Institutes of Health.

Toby Ferguson, MD, PhD, *Biogen*

Dr. Toby Ferguson is a Senior Medical Director in Research and Early Clinical Development at Biogen, a position he assumed in 2015 shortly after joining Biogen in October 2013. In this role, he serves as a clinical lead for current clinical ALS programs. He has also worked closely with neurology research and colleagues in ALS development to develop a strategy for development of ALS therapies and to improve ALS trial effectiveness. Prior to Biogen, he was the Assistant Professor of Neurology at Temple University in Philadelphia, PA, and had a clinical neuromuscular neurology practice and a lab focused on peripheral axon injury and regeneration at Shriners Research Center. Dr. Ferguson obtained an M.D. and Ph.D. (Neuroscience) from the University of Florida. He went on to complete his Neurology Residency in 2006 and his Fellowship in Neuromuscular Neurology in 2010 at the University of Pennsylvania.

Stephen Finger, Ph.D, *Person with ALS*

Stephen Finger was diagnosed with ALS in 2013. A vocal advocate, Stephen actively participated in drafting the ALS Community Developed Guidance Document submitted to the FDA. He was a Patient Fellow during the 2017 ALS/MND International Research Symposium. Stephen serves on the Board of Advisors for the Every90Minutes Foundation and is on the Board of Directors of HopeNow4ALS. He was awarded the 2014 Stephen Milne Adventurous Spirit Award by the ALS Therapy Development Institute.

Stephen grew up in Arlington, VA. After earning degrees from Princeton and Duke, Dr. Finger was an economics professor at the Moore School of Business at the University of South Carolina. He now resides in Atlanta, GA with his wife, Cara, and their two kids, Mary Adair (8) and James (6).

Timothy R. Franson, MD, *YourEncore*

Timothy Franson, M.D. is Chief Medical Officer of YourEncore, a life sciences consulting and staffing enterprise, and recently completed a five year term as President of the US Pharmacopeial Convention (USP), which sets standards for pharmaceuticals and other health products, and continues to serve on USP's Board of Directors. He also serves as Chair of the Board of Directors for the Critical Path Institute, and as Adjunct Professor of Medicine at the Indiana University School of Medicine. Dr. Franson was with Eli Lilly and Co. (Lilly Research Laboratories) from 1986 until 2008, retiring from the leadership role of Global Vice President for Regulatory Affairs and Drug Safety from 2003-2008, and was directly responsible for Lilly's regulatory submissions including over 25 major approvals, as well as regulatory compliance and policy matters. He subsequently joined FaegreBD Consulting (Washington DC) in 2009 and became a Principal and the Regulatory Practice Leader in that firm until moving to YourEncore in 2014.

Dr. Franson has extensive experience in early and late phase drug development relating to clinical, regulatory, quality and related disciplines. He has represented industry in policy matters including Congressional testimony for renewals of the Prescription Drug User Fee Act and chaired several industry committees, as well as having served on the NIH Treatment of Rare Diseases review panel; he is now on the Board of Cidara Pharmaceuticals and Paratek Pharmaceuticals. Franson was a member of the Steering Committee for the independently developed draft guidance for FDA consideration regarding development of drugs for Duchenne Muscular Dystrophy, and continues to collaborate with FaegreBD Consulting in providing rare disease drug development advice for multiple companies and advocacy groups.

Dr. Franson received his B.S. in Pharmacy from Drake University, his M.D. from the University of Illinois, internal medicine residency at the University of Iowa, and Infectious Diseases fellowship at the Medical College of Wisconsin. He is Board Certified in Internal Medicine & Infectious Diseases and authored over 50 professional publications and four book chapters.

Steven M. Hersch, MD, PhD, *Voyager Therapeutics*

Steven M. Hersch, M.D., Ph.D. is a Senior Director at Voyager Therapeutics where he leads early clinical development activities for Voyager's gene therapy pipeline programs in Huntington's disease (HD), amyotrophic lateral sclerosis (ALS), and Friedreich's Ataxia (FA). He is also a Professor of Neurology at Massachusetts General Hospital (MGH) and Harvard Medical School, former Director of the Huntington's Disease Center of Excellence at MGH, and Director of the Laboratory of Neurodegeneration and Neurotherapeutics.

Dr. Hersch is a clinical Neurologist and Neuroscientist with laboratory research experience in synaptic organization, molecular pharmacology, experimental neuropathology of neurodegenerative diseases, animal models of neurodegenerative disorders for studying pathophysiology and preclinical therapeutics development, and biomarker discovery and

validation. He was the PI of NINDS sponsored program projects focused on transcriptional alterations in HD and on biomarker development. Dr. Hersch is also an experienced HD clinician and clinical investigator who has worked on clinical outcome measure development and on many NIH and commercially sponsored observational and therapeutic trials with various roles including site investigator, steering or safety committee member or chair, and principal investigator. Dr. Hersch was the PI for the NCCIH sponsored phase II and phase III multi-center trials of creatine in HD (CREST-HD, CREST-E); was PI of the NINDS sponsored multi-center trial of phenylbutyrate for HD; led the first therapeutic trial in premanifest HD; and is currently the protocol PI for the NINDS/NeuroNext trial sponsored by Azevan Pharmaceuticals testing a novel treatment for irritability.

He co-chaired the Huntington's Study Group (HSG) for 15 years, served on the National Board of Trustees for the Huntington's Disease Society of America (HDSA) where he founded and directed the Center of Excellence Program, is a member of the Executive Committee of NeuroNext, and has served on FDA advisory panels and on the Advisory Council for NCCIH.

Madeline R. Kennedy, RN, MSN, *Person with ALS*

Madeline earned a Bachelor of Science in nursing from Georgetown University and a Master's of Science in nursing from George Mason University. She received an honorary doctorate in public service from Russell Sage College and studied at Oxford. Madeline worked as a nurse and as a nurse educator for 37 years before being stricken with ALS in 2012. Since then, she has worked relentlessly as an advocate for people with ALS and for ALS research. She has helped to raise hundreds of thousands of dollars for ALS. She has participated in numerous local and national ALS committees and advocacy activities including ALS Research Ambassadors (NEALS), Impact ALS, CrEATe, ALS Act, the Community Developed ALS Guidance Patient and Caregiver Advisory Committee and member of the Clinical Trials and Outcome Measures working group, and ALS PREFER. She maintains a website that chronicles her ALS journey at WWW.ConquerALS.com. Many of her presentations can be viewed on YouTube at madken1234.

Madeline was an avid golfer, tennis player, skier and scuba diver. She is most proud of being the mother of four children. Her ALS has progressed to where she cannot walk and has very minimal use of arms and hands. She can no longer tend to any of her self-care needs. Madeline is completely dependent upon her husband of 42 years who is her fulltime caregiver. She can speak and swallow but requires a non-invasive ventilator for much of her day. Her world may be much smaller, but her determination "to move the ball forward" in ALS research and treatment options remains undiminished.

Nicholas J. Maragakis, MD, *Johns Hopkins University*

Dr. Maragakis is Director of the ALS Center for Cell Therapy and Regeneration Research, Co-Director of the Johns Hopkins ALS Clinic and Professor of Neurology at Johns Hopkins University. The ALS Clinic at John Hopkins is a world recognized leader in providing medical care and offering the latest in clinical trials and therapies to ALS patients.

A common theme to his research has been in the study of the astrocyte biology and its role in disease pathogenesis with a particular emphasis in how astrocytes may contribute to Amyotrophic Lateral Sclerosis (ALS). His laboratory, in collaboration with others, has been interested in the development of induced pluripotent stem cells from ALS patients. Dr. Maragakis' laboratory includes a repository of over 150 ALS patient fibroblast samples and has created over 50 iPS cell lines from familial and sporadic ALS patients as well as controls. His current efforts have been to characterize iPSC-derived motor neurons and astrocytes both in vitro and in vivo with an effort towards understanding disease mechanisms; in particular modeling ALS disease heterogeneity with regard to disease progression.

Dr. Maragakis has also been heavily involved in clinical research as the principal investigator, site principal investigator, or co-investigator of numerous clinical trials in ALS, many coordinated by the Northeast ALS Consortium on which he served as an Executive Board member and currently a member of the Scientific Advisory Board. Dr. Maragakis has received several awards for his outstanding research efforts and received the Certification of Meaningful Use Stage 1 EHR from the Centers for Medicare& Medicaid Services (CMS) in 2013. He is member of the American Board of Psychiatry and Neurology, the American Academy of Neurology (AAN) and the Northeast ALS Consortium (NEALS).

Dr. Maragakis received his bachelors of science degree and medical degree from the University of Utah and he completed his residency and fellowship in neurology at John Hopkins University.

Björn Oskarsson, MD, *Mayo Clinic, Jacksonville*

Dr. Björn Oskarsson is a neurologist and the director of the ALS clinic at Mayo Clinic Jacksonville. He completed his medical training at University of Lund in Sweden in 1999 and did his post graduate medical training at University of Colorado, Denver where he stayed as faculty until 2008. In 2009 he founded the ALS clinic at University of California, Davis where he was an associate professor of Neurology and Pathology until 2016. He currently also serves as the secretary of the Western ALS study group and as a member of the ALS association medical advisory board. He has won several awards for his clinical work in ALS including a Certificate of Special Congressional Achievement. He has authored over 50 articles, chapters and scientific presentations on neuromuscular topics. He has conducted more than 20 research studies in ALS and other neuromuscular diseases. He is board certified in Neurology, Neuromuscular Medicine, Electrodiagnostic Medicine and Clinical Neuromuscular Pathology.

Jeremy M. Shefner, MD, PhD, *Barrow Neurological Institute*

Dr. Shefner is Chair of the Departments of Neurology at the Barrow Neurological Institute and the University of Arizona College of Medicine, Phoenix, AZ. Dr. Shefner's research focuses on biomarker development and the clinical therapeutics of ALS. With Merit Cudkowicz, he co-founded the Northeast ALS Clinical Trials Consortium (NEALS), the largest and most active consortium in the world dedicated to ALS. He is currently the principal investigator for two multicenter clinical trials, as well as a home based outcomes study with the goals of determining the optimum frequency of outcomes assessments and expanding the number of patients potentially eligible for clinical trials.

Dr. Shefner has published approximately 200 papers in peer-reviewed journals and has served on multiple grant review panels. He has also participated in committees organized by the Institute of Medicine to investigate the relationship between military service and ALS, as well as the health effects of Agent Orange on Vietnam War veterans. In 2014, Dr. Shefner received the Sheila Essay Award for ALS Research, presented annually by the American Academy of Neurology (AAN) and the ALS Association.

Stephen Winthrop, MA, MBA, *Person with ALS*

Retired consultant to nonprofits; diagnosed with ALS in 2013; currently serving as board chair of the ALS Association national board of trustees.

Andrew A. Wolff, MD, *Cytokinetics*

Dr. Wolff has been the Chief Medical Officer since joining Cytokinetics in September 2004. Prior to Cytokinetics, he was at CV Therapeutics from 1994 to 2004, where he was the Senior Vice President and Chief Medical Officer, after holding various positions of increasing responsibility. Before CV Therapeutics, Dr. Wolff held various drug development positions in both the U.S. and the UK for Syntex Corporation.

Dr. Wolff received a B.A. degree in Chemistry and Biology from the University of Dayton and an M.D. from Washington University Medical School in St. Louis, Missouri.