Holloway Summit 2023

Biomarkers for FTD

November 29 - December 1, 2023

Eden Roc Miami Beach Resort

4525 Collins Ave, Miami Beach, FL 33140

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A Message from Kristin Holloway

When my husband, Lee Holloway, learned he had FTD almost seven years ago, I didn't know what to do or how I was supposed to manage this devastating diagnosis. How could this young, brilliant person – the co-founder of Cloudflare, a technology company that has literally made the internet a safer place – have dementia?

Lee had, of course, been showing troubling symptoms before his diagnosis: compulsive, repetitive behaviors; erratic and inappropriate actions in public. His personality went from easygoing to aggressive to catatonic. Looking back, it was obvious that FTD had been changing his brain even while he was still working at Cloudflare. He eventually had to step down from the company in 2016, before his diagnosis, but after the disease had already drastically changed him.

Our FTD journey is the reason I am so passionate about FTD research. After Lee's diagnosis, I channeled a lot of tough emotions – grief, helplessness, sadness – into work. I joined AFTD's Board in 2020, and in 2022 I started the Holloway Summit, an event designed to bring together the world's leading FTD experts every year to work collaboratively on producing the next big scientific breakthrough – the type of moonshot that can help not just families living with FTD, but potentially those living with Alzheimer's and other types of dementia.

It took a long time for Lee to get his diagnosis – too long. Other families know the frustration of the FTD diagnostic process all too well. FTD biomarkers hold the promise of expediting diagnosis, potentially sparing future FTD families years of agony and confusion as they search for an answer to their loved one's seemingly inexplicable changes. Over the next two days, we'll learn how far we've come in the search for FTD biomarkers – and work together toward bringing that search to its triumphant conclusion.

Thank you to all who have joined us here in Miami. I truly believe that the combined knowledge, innovative spirit, and collaborative drive of those assembled here will lead to that next big breakthrough that FTD families so fervently hope for.

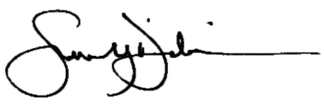
A Message from Susan Dickinson and Kimberly Pang Torres

Welcome to the 2023 Holloway Summit. On behalf of AFTD, we are grateful that you are joining us to share your insights, your creativity, and your passion for FTD research.

Some of the brightest minds in neurodegenerative disease biomarker development are on hand here in Miami to review the landscape of FTD biomarkers, and its future. As we are all aware, new biomarkers are urgently needed to help doctors recognize the presence of FTD earlier in the disease process, identify which people are affected by which underlying causes of FTD, and enable efficient clinical trials to test new treatments. Discovery and development of these biomarkers will build on a foundation of promising work in FTD and related fields of neurology, but considerable work and challenges remain.

This Summit, and the important collaborative work we will be doing over the next two days, are made possible through the generous support of AFTD Board member Kristin Holloway and the Holloway Family Fund, which she generously established in 2021. But Ms. Holloway's commitment to furthering FTD science didn't end there. The Holloway Family Fund supports the next generation of FTD research through the Holloway Scholars programs, and it has enabled AFTD to support emergent research needs. One such example of an emergent need is a collaboration between AFTD and the ALS Association to fund digital health technology at the intersection of ALS and FTD, a partnership directly inspired by the 2022 Holloway Summit on digital assessment tools.

We thank you all for joining us. Being here shows your commitment to ushering in a new future for people affected by FTD. After the family of Bruce Willis so bravely chose to share the actor's FTD diagnosis earlier this year, FTD awareness surged. But the fact remains that this disease is still too little known. Accurate diagnoses are still too difficult to come by for families struggling for answers, and effective treatments are sorely needed. That's why biomarkers are so important – and why the work you do is so vital to our community.



Susan L-J Dickinson, MSGC
AFTD Chief Executive Officer

Kimberly Pang Torres
AFTD Board Chair

Summit Co-Chairs

Danielle Graham received her PhD from Baylor University. After her doctoral training, she completed a postdoctoral fellowship at the University of Texas Southwestern Medical Center in Dallas, TX characterizing the role of BNDF and CREB in the mesolimbic DA system in neuropsychiatric illness. Dr. Graham moved to Boston MA in 2007 to join the Neurobiology department at Merck Research Labs. As a research biologist at Merck, she contributed to the early-stage drug discovery pipeline through the development of novel pharmacodynamic and efficacy models of CNS Disease. In 2009, she moved to EMD Serono, and joined the Translational Neuropharmacology group. At EMD Serono, she was responsible for leading a team of scientists in the characterization of novel therapeutics and biomarker endpoints for AD, PD, and MS. In 2014, she moved to Biogen and joined the Translational Sciences group at Biogen where she has had roles with increasing responsibility over the years. At present, Dr. Graham is Vice President, Head of Fluid Biomarkers and Bioanalytics at Biogen. In this role, Dr Graham and her team identify, develop, implement, and interpret fluid biomarkers and bioanalytical measures to support clinical programs across the Biogen pipeline. Dr Graham and her team played a critical role in the recent accelerated approval of Tofersen, a novel therapy in SOD1 ASO. Tofersen was granted accelerated approval based on a change in plasma Neurofilament light utilizing NfL as a surrogate endpoint reasonably likely to predict clinical benefit.

Stacie Weninger is the President of FBRI and a Venture Partner at F-Prime Capital Partners. Dr. Weninger received a Ph.D. in neuroscience from Harvard University, and a B.S. degree in chemistry with highest honors from the University of North Carolina, Chapel Hill. She is President of Alzforum; chairs the Collaboration for Alzheimer's Prevention; is CEO and Chairman of the Board for Rugen Therapeutics; is a member of the Board of Directors for Aratome, Atalanta, Eikonizo, Sironax, and Target ALS; is a member of the External Advisory Board for Boston Children's Hospital's Rosamund Stone Zander Translational Neuroscience Center; is a member of the Scientific Advisory Boards for the Breuer Foundation, Brown University's Carney Center for Alzheimer's Disease Research, Denali Therapeutics, the Indian Institute of Science's Centre for Brain Research, the MIT Yang-Tan Center for Molecular Therapeutics, and the UK Dementia Research Institute. She served as a founding member of the Board of Directors for Denali Therapeutics and Neumora Therapeutics.

Henrik Zetterberg is a Professor of Neurochemistry at the University of Gothenburg, Sweden, and University College London, UK, and a Clinical Chemist at Sahlgrenska University Hospital in Gothenburg, Sweden. He is Head of the Department of Psychiatry and Neurochemistry at the University of Gothenburg, leads the UK DRI Fluid Biomarker Laboratory at UCL, and is a Key Member of the Hong Kong Center for Neurodegenerative Diseases and a Visiting Professor in the UW Department of Medicine, School of Medicine and Public Health, Madison, Wisconsin. His main research focus and clinical interest are fluid biomarkers for brain diseases, neurodegenerative diseases in particular.

Summit Host

Kristin Holloway is the host and supporter of the Holloway Summit and a member of the AFTD Board. She is a communications professional with more than 10 years of experience as a strategist for high-growth technology companies. In addition to having experience in technology public relations, her expertise includes media and analyst relations, campaign management, strategic and crisis communications, project management and technical content creation. In April 2017, her husband Lee Holloway, a gifted technology pioneer who co-founded the web security and performance company Cloudflare, was diagnosed with behavioral variant FTD. To honor Lee's legacy, Kristin and the Holloway family established The Holloway Fund for Help and Hope at AFTD in 2019. Kristin is passionate about raising funds to help others as they navigate their FTD journey, as well as supporting cutting-edge research to advance treatments.

Summit Agenda

Wednesday, November 29

5:00 – 7:00 pm	**Reception**

Thursday, November 30

7:30 – 8:30 am	**Breakfast**
8:30 – 8:50 am	**Welcome** Penny Dacks, AFTD Kristin Holloway, Holloway Family

Session 1: Foundations of FTD Biomarkers
Chair: Stacie Weninger, FBRI

8:50 – 9:20 am	Current FTD biomarker landscape Adam Boxer, UCSF
9:20 – 9:35 am	Biomarkers in FTD drug development Billy Dunn
9:35 – 10:05 am	Panel Discussion/Q & A
10:05 - 10:20 am	**Coffee break**

Session 2: Differentiating FTD-TDP-43 and FTD-Tau
Chair: Henrik Zetterberg, University of Gothenburg

10:20 – 10:25 am	Session Introduction Henrik Zetterberg, University of Gothenburg

A. TDP-43 fluid biomarkers

10:25 – 10:35 am	Detection of TDP-43 protein species/FTLD-TDP-43 subtypes Leonard Petrucelli, Mayo Clinic
10:35 – 10:45 am	Cryptic peptides: an emerging biomarker Philip Wong, Johns Hopkins University
10:45 – 10:55 am	Q & A

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B. Tau fluid biomarkers	
10:55 – 11:05 am	Detecting Tau pathology in primary tauopathies **Chihiro Sato, Washington University**
11:05 - 11:10 am	Q & A
C. Combining fluid biomarkers to improve discrimination	
11:10 – 11:20 am	NfL/GFAP and combinatorial approaches **David Irwin, University of Pennsylvania**
11:20 – 11:25 am	Q & A
D. PET imaging biomarkers	
11:25 –11:35 am	Advances in PET ligands for primary tauopathies and TDP-43 **Brad Navia, Aprinoia**
11:35 – 11:40 am	Q & A
11:40 am – 12:25 pm	Session 2: Panel Discussion
12:30 – 1:30 pm	**Lunch**
Session 3: Biomarkers in FTD clinical trials **Chair: Danielle Graham, Biogen**	
1:35 – 1:40 pm	Session Introduction **Danielle Graham, Biogen**
1:40 – 2:00 pm	Fluid biomarkers in neurodegeneration trials – lessons learned for FTD **Marta del Campo, Barcelona Beta Brain Research Center**
2:00 – 2:20 pm	Novel biomarkers in use in FTD clinical trials **Giacomo Salvadore, Alector**
2:20 – 2:40 pm	Looking to the future: emerging science to inform biomarker development in clinical trials **Scott Small, Columbia University**

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2:40 – 3:20 pm	Panel Discussion
3:20 – 3:35 pm	**Coffee break**
3:35 – 3:45 pm	Research Readiness: Insights from the Community **Penny Dacks, AFTD**
3:45 – 4:00 pm	Perspectives from a caregiver: Q&A with Kristin Holloway **Moderator: Penny Dacks, AFTD**
4:00 - 4:30 pm	Panel Discussion with FTD drug developers
4:30 – 4:40 pm	Reflections on Day 1 **Olivier Piguet, University of Sydney**
6:00 – 7:00 pm	**Reception**
7:00 – 8:30 pm	**Dinner**

Friday, December 1

7:30 – 8:25 am	**Breakfast**
	Session 4: How to improve equity and access to FTD diagnosis via biomarkers? **Chair: Chiadi Onyike, Johns Hopkins University**
8:25 – 8:30 am	Session Introduction **Chiadi Onyike, Johns Hopkins University**
8:30 – 8:45 am	What's the current patient journey for diagnosis? **Chiadi Onyike, Johns Hopkins University**
8:45 – 9:00 am	Considerations of biomarkers for equity in care and diagnosis **Jalayne Arias, Georgia State University**
9:00 – 9:15 am	Volumetric imaging **Simon Ducharme, McGill University**
9:15 – 9:30 am	Imaging approaches to distinguish forms of dementia **David Jones, Mayo Clinic**

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9:30 – 9:45 am	Reflections on biomarkers as a tool to distinguish psychiatric disorders from bvFTD **Yolande Pijnenberg, Amsterdam UMC**
9:45 – 10:00 am	Diagnosis by exclusion (e.g. AD-emergent biomarkers) **Charlotte Teunissen, Amsterdam UMC**
10:00 – 10:45 am	Session 4 Panel Discussion
10:45 – 11:00 am	**Coffee break**

Session 5: Facilitating FTD biomarker discovery and development
Chair: Debra Niehoff, AFTD

11:00 – 11:05 am	Session Introduction **Debra Niehoff, AFTD**
11:05 – 11:15 am	Leveraging resources to advance biomarker development: Learnings from PPMI **Mark Frasier, Michael J. Fox Foundation**
11:15 – 11:45 am	Lightning Round: Perspectives on barriers and opportunities
11:45 am – 12:15 pm	Panel discussion: Strategies to accelerate biomarker discovery and development
12:15 – 1:00 pm	**Lunch**
1:00 – 2:00 pm	Closing discussion: Summit recommendations for the future of FTD biomarker development **Meeting Chairs**
2:00 pm	**Adjourn**

Speaker Bios

Adam L. Boxer is the Endowed Professor in Memory and Aging in the Department of Neurology at the University of California, San Francisco (UCSF). He received his MD and PhD from the New York University Medical Center Medical Scientist Training Program, completed a Neurology Residency at Stanford and a Neurobehavior Fellowship at UCSF. Dr. Boxer directs the Neurosciences Clinical Research Unit and the Alzheimer's Disease and Frontotemporal Degeneration (FTD) Clinical Trials Program at the UCSF Memory and Aging Center. Dr. Boxer has been a pioneer in developing new treatments and biomarkers for frontotemporal lobar degeneration. He is co-Principal Investigator of the ARTFL/LEFFTDS Longitudinal Frontotemporal Lobar Degeneration (ALLFTD) project, a 27 site research network focused on preparing for FTLD clinical trials, and the FTD Prevention Initiative (FPI), a global collaboration focused on preparing for prevention trials in autosomal dominant FTLD. He is PI of the Neurofilament Surveillance Project (NSP), a privately funded study that is developing blood biomarkers for use in FTLD clinical trials.

He also leads the Four Repeat Tauopathy Neuroimaging Initiative (4RTNI), a NIH-funded, multicenter, longitudinal tau PET and biomarker study focused on PSP and CBD. He has been the PI for a variety of multicenter, randomized, placebo controlled clinical trials in neurodegenerative diseases, including memantine for FTLD, davunetide for PSP, TPI-287 for primary and secondary tauopathies, a Phase 1b trial of gosuranemab for PSP, and salsalate for Alzheimer's Disease and PSP. He is the lead PI of the planned NIH Alzheimer's Clinical Trial Consortium's (ACTC) Tau Platform clinical trial. He has co-chaired the National Alzheimer's Project Act (NAPA) FTLD Research Committee for the past four years. He also co-chairs the FTLD Research Roundtable and the PSP Research Roundtable, academic-industry collaborative groups working to speed the development of new therapies for FTLD, CBD and PSP.

Adam Staffaroni is a clinical neuropsychologist and assistant professor at the UCSF Memory and Aging Center. The focus of his research is to improve early detection, prognosis, and monitoring in neurodegenerative diseases, particularly frontotemporal dementia. He is a co-investigator in the ALLFTD consortium and has led several efforts to develop remote digital data collection methods for FTD. He is also Co-PI of the FTD Prevention Initiative's data infrastructure project and has led the harmonization of data across international familial FTD consortia to enable disease progression modeling, endpoint selection, and clinical trial simulations.

Arthur Simen is the Executive Medical Director and Head of Neurodegenerative Disorders within Takeda's Clinical Science group. He is a physician and neuroscientist and leads a group of physicians and scientists dedicated to clinical development for neurodegenerative disorders. He also co-leads Takeda's Neurodegenerative Disorders Unit, a cross-functional team with membership across the organization dedicated to coordinating Takeda's drug development efforts from discovery to clinical development in neurodegenerative disorders.

Billy Dunn is the founding director of the Office of Neuroscience, CDER, at the FDA, a position he held since the founding of the office in 2019 through February 2023. He was responsible for the regulatory oversight of all research conducted to support neuroscience drug development, including the regulation and review of investigational new drug applications and marketing applications for drug and biologic products. From 2005 to 2019, he held positions of increasing seniority in the Division of Neurology Products, CDER, including his role as director of that division.

During his tenure at FDA, Dr. Dunn had a significant impact on how drugs are developed for a range of serious neurological diseases, including many that previously had only limited treatment options. His work and leadership contributed to major therapeutic advances in neuro-immunological diseases such as multiple sclerosis and myasthenia gravis, advances in rare neurological disorders, advances in migraine treatment, advances in the range of complex seizure disorders, and advances in therapies for Alzheimer's disease, among many other neurological disorders.

Dr. Dunn is a trained neurologist and vascular neurologist with experience in basic research, clinical research, and clinical care. He earned his B.A. from the University of Virginia and his M.D. from the F. Edward Hébert School of Medicine in Bethesda, Maryland.

Bradford Navia is the Chief Medical Officer, Executive Vice President at Aprinoia Therapeutics, Cambridge, MA, a clinical stage diagnostic and therapeutic neuroscience company, focused on the development of novel imaging agents for the detection of tau and alpha synuclein aggregates, and the development of novel therapeutics for neurodegenerative disorders, including selective monoclonal antibodies to tau and a protein degradation platform targeting tau and alpha synuclein aggregates. With support from ADDF, he is currently leading a phase 3 trial of APN 1607, a first in class 3R, 4R tau tracer, as a diagnostic marker for PSP and nonAD tauopathies.

Prior to joining Aprinoia, Dr Navia spent 16 years in industry in various roles with increasing responsibility and successfully led several phase 1 to 3 trials in psychiatry and neurology, and participated in multiple INDs, sNDA and NDAs, including the approval of KYNMOBI for the treatment of Parkinson's Off episodes for which he was the program lead. As an associate professor at Tufts Medical School, he received 20M in NIH (NINDS) funding for research focused on neurogenetics, the neurobiology of HIV infection. Research areas included the application of neuroimaging approaches (MRS, MRI, DTI) to investigate the chronic neurological effects of HIV infection and to clinical trials in CNS drug development. He has authored over two hundred publications, abstracts, and chapters.

He received his medical degree at Columbia College of Physicians and Surgeons, neurology training at Cornell, NY and his doctoral degree in neuroscience and genetics at Harvard University, Cambridge, MA.

Charlotte Teunissen (full professor in Neurochemistry) aims to improve care of patients with neurological diseases by developing body fluid biomarkers for diagnosis, stratification, prognosis and monitoring treatment responses. Studies of her research group span the entire spectrum of biomarker development, starting with biomarker identification, followed by assay development and validation, and extensive clinical validation to ultimately implement novel biomarkers in clinical practice.

She is responsible for the Alzheimer Center Amsterdam body fluid and leads several international biomarker networks, such as the CSF Society and the Alzheimer Association-Global Biomarker Standardization consortium, and the recently founded Coral proteomics consortium. She is the coordinator of the Marie Curie MIRIADE project, aiming to train 15 novel researchers into accelerate dementia biomarker development.

Chiadi U. Onyike, Associate Professor of Psychiatry and Behavioral Sciences at Johns Hopkins University, is a neuropsychiatrist and clinical epidemiologist and directs the clinical and research programs focused on the frontotemporal dementias, young-onset Alzheimer disease, and other neurodegenerative disorders in young and middle-aged adults.

He is a principal investigator and co-investigator of research projects sponsored by NIH, foundation, industry and philanthropy grants. He is the chair-elect of the Association for Frontotemporal Degeneration Medical Advisory Council, and he sits on the Scientific Advisory Boards of the Tau Consortium and the FTD Disorders Registry, and on the executive committee of the International Society for Frontotemporal Dementias. He has served on NIH Alzheimer's Disease and Related Dementias committees, the NIH Amyotrophic Lateral Sclerosis Strategic Planning Working Group, and the FDA Advisory Committee for Central and Peripheral Nervous System Drugs.

Dr. Onyike received his Medical Degree from the University of Nigeria, and a graduate degree in Clinical Epidemiology from the Johns Hopkins Bloomberg School of Public Health. He completed psychiatry training at the Johns Hopkins School of Medicine, and research training in Psychiatric Epidemiology at the Johns Hopkins Bloomberg School of Public Health, and Neurochemistry/Cell Signaling at the University of Maryland at Baltimore.

Chihiro Sato is an Assistant Professor of Neurology at Washington University School of Medicine. Her research goal is to understand the pathophysiology of Alzheimer disease (AD) and primary tauopathies through tau kinetics. She uses human brain tissues, cerebrospinal fluid (CSF), and cell culture models to understand how the turnover of different tau proteoforms such as phosphorylated tau, truncated tau and tau isoforms change with tauopathies. She hopes to develop fluid biomarkers for primary tauopathies that are currently missing in the field.

David T. Jones is a consultant in Behavioral Neurology and serves as Director of Artificial Intelligence in the Department of Neurology at Mayo Clinic in Rochester, Minnesota. He joined the staff of Mayo Clinic in 2014 and holds the academic rank of assistant professor of neurology and radiology, Mayo Clinic College of Medicine and Science. Dr. Jones' interests are behavioral neurology, normal pressures hydrocephalous, multimodal neuroimaging, and artificial intelligence.

David Irwin is the co-director of the Penn Frontotemporal Degeneration Center and PI of the Penn Digital Neuropathology Lab at the University of Pennsylvania Perelman School of medicine. He has dual training in cognitive neurology and neuropathology and his lab focuses on integrating human brain histopathology with image analysis methods to integrate fine-grained measures of postmortem neuropathology with antemortem biomarker data, with the overall goal of identifying therapeutic targets and markers of disease progression that can serve as endpoints in treatment trials for FTD, LBD, AD and related neurodegenerative disorders.

Debra Niehoff the Director of Research & Grants for AFTD, oversees AFTD's research portfolio, including grant programs supported in partnership with the Alzheimer's Drug Discovery Foundation and Target ALS. In addition, she coordinates networking, training, and mentorship opportunities that support the FTD research community. Dr. Niehoff holds a PhD from Johns Hopkins University and has experience in behavioral neuroscience, particularly the neurochemical and neuroanatomical basis of fear, aggression, and violence. Prior to

joining AFTD, she developed and served as the coordinator for one of the first two-year neuroscience programs in the U.S. She is the author of *The Biology of Violence: How Understanding the Brain, Behavior, and Environment Can Break the Vicious Circle of Aggression and The Language of Life: How Cells Communicate in Health and Disease*, serves on the editorial board of the journal Violence and Gender, and has conducted workshops on the neurobiology of violence for the FBI Critical Incident Response Group as well as law enforcement and educational professionals.

Fen Huang - I earned my PhD in neurobiology and did postdoctoral training in neuroscience and physiology at UCSF. Presently, I am a Director of Translational Medicine at Denali Therapeutics, where I lead the translational biomarker strategies for ALS/FTD and AD drug development. I have also been collaborating with major consortia such as ALLFTD for patient biomarker studies to inform our clinical trial design.

Giacomo Salvadore is a psychiatrist by training and is currently the Senior Vice President and Head of Clinical Development at Alector, Inc., where he oversees the development of drugs for neurodegenerative disorders such as frontotemporal dementia and Alzheimer's disease. Dr. Salvadore received his medical degree and psychiatry training at the University of Rome, Tor Vergata in Italy and completed a fellowship at the National Institute of Mental Health in the Mood and Anxiety Disorders program. Prior to joining Alector Dr. Salvadore has spent more than 10 years at Janssen as a biomarker and clinical leader in neurology and psychiatry drug development, as well as 2 years at Acadia Pharmaceuticals where he was the Head of Translational Medicine. During his career in the pharmaceutical industry, Dr. Salvadore has led the development of several experimental compounds through early and late development across multiple indications.

Jalayne Arias is an Associate Professor in the Department of Health Policy & Behavioral Sciences, in the School of Public Health at Georgia State University. Her research harnesses training in law and ethics to evaluate critical challenges associated with aging, including neurodegenerative conditions.

Jane Chan is a Senior Director at AviadoBio, where she is responsible for the clinical development of AVB-101 for FTD with GRN mutations, and the clinical strategy for early stage programmes in ALS/FTD. Dr Chan was previously at Freeline Therapeutics, where she led the clinical development of AAV gene therapies for the treatment of Gaucher and Fabry disease. Dr Chan began her Industry career over 12 years ago at UCB, initially with the Neuroscience group in Medical Affairs and late stage Clinical Development, and then subsequently with the Translational Medicine Immunology group where she worked on several early phase programmes focussed on antibody-based therapeutics for a range of inflammatory disease indications. Dr Chan received her Bachelors in Natural Sciences from the University of Cambridge, medical degree from Imperial College London, and also holds a Masters in Health Economics, Policy & Management from the London School of Economics.

Kristin Holloway is the host and supporter of the Holloway Summit and a member of the AFTD Board. She is a communications professional with more than 10 years of experience as a strategist for high-growth technology companies. In addition to having experience in technology public relations, her expertise includes media and analyst relations, campaign management, strategic and crisis communications, project management and technical content creation. In April 2017, her husband Lee Holloway, a gifted technology pioneer who co-founded the web security and performance company Cloudflare, was diagnosed with behavioral variant FTD. To honor Lee's legacy, Kristin and the Holloway family established The Holloway Fund for Help and Hope at AFTD in 2019. Kristin is passionate about raising funds to help others as they navigate their FTD journey, as well as supporting cutting-edge research to advance treatments.

Laura Mitic is the Acting President and Chief Scientific Officer at the Bluefield Project to Cure Frontotemporal Dementia (FTD), a nonprofit dedicated to enabling the development of therapies for FTD caused by mutations in progranulin (FTD-GRN). Bluefield funds 18 academic researchers to accelerate our understanding of FTD-GRN disease biology and progression. Laura oversees Bluefield's efforts to identify and execute promising ideas to support therapeutic development, including the Neurofilament Surveillance Project, a precompetitive effort to measure plasma neurofilament light in genetic FTD over 3 years via remote blood collection. She earned a PhD in cell biology from Yale University and completed postdoctoral studies at UCSF.

Leonard Petrucelli earned his Bachelor of Science degree at Barry University, Miami, and his Ph.D. degree in molecular and cellular biochemistry at Loyola University and Stritch School of Medicine, Chicago. He came to Mayo Clinic's Florida campus as a research fellow in 2000 and joined the neurosciences research staff two years later. He is currently a consultant and member of the Department of Neuroscience at Mayo Clinic in Florida, where he holds the academic rank of professor of neuroscience and has full faculty privileges in molecular neuroscience at Mayo Graduate School. He is recognized as the Ralph B. and Ruth K. Abrams Professor.

Dr. Petrucelli and his research team are constantly at the forefront of their field, researching the mechanisms that cause neurodegeneration in Alzheimer's disease, amyotrophic lateral sclerosis (ALS), and frontotemporal dementia (FTD) and harnessing their findings into the development of novel therapeutic targets and biomarkers. His lab made several vital contributions to the understanding of c9orf72-mediated ALS and FTD: they were one of the first groups to describe both RAN translation and antisense pathology in this disease, developed the first mouse model to recapitulate both behavioral and neuropathological features, and characterized important biomarkers. Dr. Petrucelli's team remains one of the leaders of this field. At the same time, they continue to extend their expertise in cell biology, disease modeling, and biomarker development to additional neurodegenerative conditions, and have over a decade's worth of experience in the study of both TDP-43- and tau-related disorders. Emerging studies focus on understanding the importance of aberrant TDP-43-mediated splicing in ALS/FTD and the mechanisms underlying the spread of tau pathology in dementias. His team's research has been published in top tier journals including Cell, Nature Science, Science Translational Medicine, Nature Medicine, Nature Neuroscience, Neuron, Journal of Clinical Investigation and Annals of Neurology.

Dr. Petrucelli is principal investigator for several grants funded by the National Institutes of Health (NIH) and is director of two funded NIH programs focused on c9orf72 and tau. In addition to his commitment to his own research goals, Dr. Petrucelli also serves on the Board of Review Editors at Science Translational Medicine and is the Chief Scientific Advisor to the Target ALS Foundation. He was also appointed as vice-chair to the Florida Alzheimer's Disease Research Grant Advisory Board. Overall, he strives to foster an environment founded on academic rigor and open collaboration both within his lab and in the field as a whole.

As Co-Chief Scientific Officer **Mark Fraiser** co-manages a team of research professionals who stay closely linked to the Parkinson's research community in order to develop an aggressive and innovative agenda for accelerating research and drug development for Parkinson's disease. This ensures that MJFF research priorities reflect and best serve the ultimate needs of patients. Mark regularly meets with academic and industry researchers around the world to identify promising proposals to support, providing troubleshooting and ongoing management of projects as they go forward. He also supports the Foundation's priority interest in developing biomarkers for Parkinson's disease that will accelerate clinical trials of new drugs.

Dr. Frasier earned an undergraduate degree in Biochemistry from the University of Dayton and a PhD in Pharmacology from Loyola University Chicago. He completed his postdoctoral work in the Neuroscience Discovery Research Group at Eli Lilly, Inc., in Indianapolis, Indiana, where he worked on drug-discovery research in Parkinson's and Alzheimer's disease. Since joining MJFF in 2006 he has led innovative strategies to support Parkinson's therapeutic development and address pre-clinical, clinical, and regulatory challenges.

Marta del Campo is a translational neurobiologist. She is a principal investigator at San Pablo CEU University and the head of the biofluid based biomarker facility at BarcelonaBeta Brain Research Institute. Her main scientific interest is to understand the biological changes that underlie the different dementia types and translate this knowledge into applicable diagnostic tests and potential therapeutic targets. The complexity of dementia has prompted her to initiate and co-lead highly collaborative studies that analyze the proteome in different biofluids from patients with different dementia types at different stages (PRIDE and bPRIDE projects); and identify, develop, and validate biomarker panels that might be useful in clinical settings and trials. By combining -omics data and using different experimental models, she aims also to identify pathways and molecules that might involve in the pathogenesis of the different dementia types.

Michael Benatar is a professor of neurology; the Walter Bradley Chair in ALS Research; Executive Director of the ALS Center; Chief of Neuromuscular Division; and Vice Chair for Clinical & Translational Research in the Department of Neurology at the University of Miami. Dr. Benatar obtained his medical degree at the University of Cape Town in South Africa, and his doctorate in neuroscience while a Rhodes Scholar at the University of Oxford. After completing residency and fellowship training at Harvard, he obtained formal training in research methodology through a Master's in the Science of Clinical Research degree while a faculty member at Emory. He runs an active clinical/translational research program focused on biomarker and therapy development for ALS. He leads the Pre-Symptomatic Familial ALS (Pre-fALS) study, which he initiated in 2007, and the CReATe Consortium, a ~35-center network focused on therapy development for ALS and related disorders. Dr. Benatar is known internationally for his pioneering work in defining the field of pre-symptomatic ALS, including discovery of the first biomarker of pre-symptomatic disease that has been critical to the design and initiation of the first pre-symptomatic ALS trial. He has also been a thought-leader in challenging existing paradigms for pre-clinical therapeutic studies; shaping how we conceptualize and use biomarkers for therapy development; and championing the use of enrichment strategies in ALS trial design.

Penny Dacks became AFTD's Senior Director of Scientific Initiatives in September 2020. Previously, she worked at the American Epilepsy Society, overseeing all mission-related programs in research, medical education, and clinical activities. Before that she spent five years at the Alzheimer's Drug Discovery Foundation, where she led development of CognitiveVitality.org and the Aging & Alzheimer's Prevention Program to source and evaluate potential therapies. She holds a Ph.D. in neuroscience from the University of Arizona. As AFTD's Senior Director of Scientific Initiatives, Dr. Dacks leads strategy for AFTD's research programs and serves as President of the FTD Disorders Registry.

Olga Uspenskaya-Cadoz is a board certified neurologist with over 10 years of experience in clinical development. Olga completed her medical education as well residency in neurology at the First Moscow State Medical University, where she continued to serve as a clinical neurologist later. In this role she was responsible for inpatient and outpatient management of patients with neurologic diseases. Subsequently, she completed a PhD program in neurosciences at First Moscow State Medical University and Ludwig-Maximilians University, Munich, Germany (focusing on CSF and blood-based biomarkers of AD); and a post-doctoral fellowship at the University Hospital Pitié-Salpêtrière, Paris, France (PIB-PET in AD vs. PCA). During her clinical and research career she has held academic positions which included research grants and lecturing on neurology topics. Prior to joining Prevail Therapeutics, Olga served as Senior Medical Director within IQVIA where she was overseeing large-scale clinical trials in AD, MS, PD, ALS, SMA. Moreover, she had global responsibility for the medical strategy of IQVIA neurology portfolio, thought leadership activities and training of junior medical directors. Before IQVIA, Dr. Uspenskaya-Cadoz had an investigator experience in neurology clinical trials and academic studies, mainly in Alzheimer's disease, FTLD, primary progressive aphasias, and age-related cognitive decline. She was medical/scientific coordinator of INSIGHT- preAD cohort sponsored by French National Institute of Health (Inserm). Dr. Uspenskaya-Cadoz has published in peer-reviewed clinical related journals, contributed to the chapters in neurology textbooks, as well as presented at neurology conferences worldwide.

Olivier Piguet is Professor of Clinical Neuropsychology and NHMRC Leadership Fellow at the University of Sydney where he is the director of FRONTIER, the frontotemporal dementia clinical research group at the Brain and Mind Centre in Sydney, Australia.

He is a registered clinical neuropsychologist with over 20 years clinical experience in the field of frontotemporal dementia and related conditions. Olivier trained in Geneva and Melbourne and completed his PhD at the University of Sydney, followed by a postdoctoral fellowship at the Massachusetts Institute of Technology.

His research program investigates early clinical markers of frontotemporal dementia, prediction of disease progression and relations to biomarkers, genetics and brain pathology. He has published over 330 peer-reviewed journal articles on these topics, attracting >22,000 citations.

Olivier is a founding member and President Elect of the International Society for the Frontotemporal Dementias. He is past President of ASSBI, the Australasian Society for the Study of Brain Impairment.

Philip C. Wong, Professor of Pathology and Neuroscience at Johns Hopkins Medicine, oversees major programs over the past three decades in the field of neurodegenerative diseases. Using a variety of molecular/biological approaches, his work focuses on pathogenic mechanisms of Alzheimer's disease (AD) and Amyotrophic Lateral Sclerosis (ALS) – Frontotemporal Dementia (FTD) and on defining molecular pathways amenable to therapeutic interventions. Wong's work on AD has been recognized by a Zenith Fellow Award from the Alzheimer's Association an Award for Medical Research from the MetLife Foundation. More recently, he is the recipient of the Daniel Nathans Scholar award from Johns Hopkins Medicine. Wong has developed novel tools, including mouse models of AD and ALS-FTD, to examine the pathophysiology of these neurodegenerative diseases with the goal of identifying therapeutic targets that would ultimately lead to effective therapies for these devastating diseases of the elderly. Wong has mentored numerous graduate students, postdoctoral fellows, and junior faculty, many of whom are women or minorities. His recent efforts are designed to clarify the biology and pathobiology of TDP-43, particularly the loss of TDP-43 splicing repression in ALS-FTD, Inclusion Body Myositis, and in AD-related dementia, as well as development of functional diagnostic biomarkers and an AAV gene therapeutic strategy for human disease exhibiting TDP-43 pathology.

Scott A. Small is the Director of the Alzheimer's Disease Research Center at Columbia University. Dr. Small's lab focuses on disorders that effect the hippocampus, a brain circuit targeted by Alzheimer's disease, schizophrenia, and the normal wear & tear of the aging process ('cognitive aging'). The lab first developed novel MRI tools applicable to patients and animal models that were used to pinpoint the parts of the hippocampal circuit differentially affected by each disorder (Nature Reviews Neuroscience, 2011). This anatomical information was then used as a guide to uncover pathogenic drivers (Neuron, 2014): Retromer-dependent endosomal recycling in Alzheimer's disease, dietary flavanols in cognitive aging, and the glutamate metabolic cycle in schizophrenia. Most recently, his lab has been developing interventions and biomarkers for each disorder, and the lab's discoveries were the cornerstone for the formation of a new biotechnology company, Retromer Therapeutics (https://retromertherapeutics.com/).

Simon Ducharme is a Neuropsychiatrist and a clinician-scientist at the Douglas Mental Health University Institute and Montreal Neurological Institute (Montreal, Canada). He is specialized in frontotemporal dementia, and the interface between psychiatric disorders and dementia. He conducts research on novel dementia biomarkers and clinical trials. He is the author of over 130 scientific articles and 8 book chapters. Dr. Ducharme is the director of the McGill Division of Geriatric Psychiatry & Neuropsychiatry and the Associate Chair of Research for the Department of Psychiatry at McGill University. He is the co-director of the Neuropsychiatric International Consortium for FTD.

Sue Browne is VP of Biology, Head of Preclinical Biology and Head of Biomarkers and Precision Medicine at Passage Bio, based in Philadelphia PA. Sue received her BSc in Pharmacology from the University of Aberdeen and a PhD in Neuropharmacology from the University of Glasgow in Scotland. Since her post-doctoral research with Flint Beal in the Neurology Department at Massachusetts General Hospital her career has centered on efforts to understand the etiologies of neurodegenerative disorders, with a focus on preclinical & translational CNS drug development for symptomatic and disease modifying approaches. After academic research within the Neuroscience Program Faculty at Cornell University's Weill Medical College, Sue moved to industry to put CNS drug development into practice, first at Merck Research Labs. Currently she leads the scientific support activities, and exploratory and clinical biomarker initiatives, for gene therapy programs targeting FTD-GRN and other rare diseases at Passage Bio.

The research conducted by the Translational Neurobiology of Amyotrophic Lateral Sclerosis and Frontotemporal Dementia Laboratory led by **Tania Gendron** focuses on deciphering the molecular causes of, and identifying biomarkers for, amyotrophic lateral sclerosis (ALS), frontotemporal dementia (FTD) and other neurological disorders with the ultimate goal of expediting the discovery of effective treatments for these diseases. Using patient biofluids and brain tissues along with preclinical models, Dr. Gendron seeks to uncover protein markers that will facilitate the earlier diagnosis of ALS and FTD, better estimate disease prognosis, aid in the screening of therapeutic candidates, and confirm target engagement of potential therapies in clinical trials.

Yolande Pijnenburg is a behavioral neurologist working at the Alzheimer Center Amsterdam at the Amsterdam University Medical Center. Her main focus of research are the clinical manifestations of early-onset Alzheimer's disease and biomarkers for frontotemporal dementia. For 10 years she has run a neuropsychiatric clinic together with a psychiatrist where they conduct a longitudinal study called the Social Brain Project in order to investigate tools to differentiate between bvFTD and primary psychiatric disorders. She is the supervisor of 17 PhD students and 4 postdoctoral researchers. She is the chair of the Dutch FTD experts group, and together with Dr. Simon Ducharme she leads the Neuropsychiatric International Consortium for Frontotemporal Dementia.

Yossi Dagon is a biomarker discovery scientist at QurAlis, leading the development of biofluid biomarkers and technologies to stratify ALS patients based on molecular signatures and markers of treatment response, enabling a precision medicine approach to clinical development

Dr. Dagon is an author on more than 35 publications, a co-inventor on more than 15 issued U.S. patents and serves as a reviewer for several scientific journals.

Dr. Dagon received a Master's degree in Cellular and Molecular Biology from Bar-Ilan University, Israel, a Ph.D. in Pharmacology from the Hebrew University of Jerusalem and completed his postdoctoral training at Harvard Medical school in Professor Barbara Kahn's laboratory.

Attendee List

Gail Andersen	AFTD Board
Jalayne Arias, **JD, MA**	School of Public Health - Georgia State
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Sue Browne, **PhD**	Passage Bio
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Josefina Cruzat, **Postdoctoral Researcher**	BrainLat, Universidad Adolfo Ibañez
Penny Dacks, **PhD**	AFTD
Yossi Dagon, MS, PhD	QurAlis
Susan Dickinson, **MSGC**	AFTD
Simon Ducharme, **MD**	McGill University
Billy Dunn, **MD**	Invited Speaker
Mark Frasier, **PhD**	Michael J. Fox Foundation
Samantha Garner	AFTD
Tania Gendron, **PhD**	Mayo Clinic
Amanda Gleixner, **PhD**	AFTD
Bridget Graham	AFTD
Danielle Graham, **PhD**	Biogen
Dale Greenberg	ConferenceDirect
Percy Griffin	Alzheimer's Association
Jonas Hannestad, **MD, PhD**	Proximity Therapeutics
Emma Heming-Willis	Make Time Wellness Inc.
Alexandra Holloway, **BS, MS, PhD**	Holloway Family
Kristin Holloway	Holloway Family
Fen Huang, **PhD**	Denali Therapeutics
Serena Hung, **MD**	Arkuda Therapeutics
David Irwin, **MD**	University of Pennsylvania

Attendee List

David Jones, **MD**	**Mayo Clinic**
PJ Lepp	**AFTD**
Terri Maher	**Holloway Family**
Qinwen Mao	**University of Utah Pathology Department**
Laura Mitic, **PhD**	**Bluefield Project to Cure FTD**
Bradford Navia, **MD, PhD**	**Aprinoia**
Debra Niehoff, **PhD**	**AFTD**
LeighAnne Olsen, **PhD**	**Robertson Foundation**
Chiadi Onyike, **MD, MHS**	**Johns Hopkins University**
Meriel Owen, **PhD**	**ADDF**
Leonard Petrucelli, **PhD**	**Mayo Clinic**
Olivier Piguet, **PhD**	**The University of Sydney**
Yolande Pijnenburg, **MD, PhD**	**AmsterdamUMC**
Madolyn Rogers, **PhD**	**Alzforum**
Giacomo Salvadore, **MD**	**Alector, Inc.**
Chihiro Sato, **PhD**	**Washington University School of Medicine**
Kristin Schneeman	**AFTD Board**
Arthur Simen, **MD, PhD**	**Takeda Development Center Americas, Inc.**
Scott Small, **MD**	**Columbia University Irving Medical Center - Alzheimer's Disease Research Center - ADRC**
Adam Staffaroni, **PhD**	**UCSF**
Aggie Stephens, **MSc**	**Milken Institute**
Tao Sun, **PhD**	**NINDS, NIH**
Jorge Torres, **MD**	**Alexion AstraZeneca Rare Disease**
Kimberly Torres	**AFTD Board**
Olga Uspenskaya, **MD, PhD**	**Eli Lilly/Prevail Therapeutics**
Stacie Weninger	**FBRI**
Philip Wong, **PhD**	**Johns Hopkins University**
Henrik Zetterberg, **MD, PhD**	**University Of Gothenburg**

The Association for Frontotemporal Degeneration

AFTD's mission is to improve the quality of life of people affected by FTD and drive research to a cure. We work every day to advance:

Research. We promote and fund research toward diagnosis, treatment and a cure.

Awareness. We stimulate greater public awareness and understanding.

Support. We provide information and support to those directly impacted.

Education. We promote and provide education for healthcare professionals.

Advocacy. We advocate for research and appropriate, affordable services.

AFTD BOARD OF DIRECTORS

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