SESSION LECTURE

No.21

Onset/progression of Neurological Disorders & Intervention Strategies - From Bench to Bedside
Room: Dong Yu Grand Ballroom 1

Co-Chairs:
Zhiying Wu

Aaron D. Gitler

Day 2 October 20th (Sunday) 14:00 – 17:30

Time	Speaker	Title
14:00-14:30	**Don W. Cleveland** University of California,San Diego, USA	Designer DNA drug therapy for neurodegenerative disease
14:30-15:00	**Yichang Jia** Tsinghua University, China	From a rational disease model to ALS therapy
15:00-15:30	**Zhiying Wu** Zhejiang University,China	Precise Diagnosis and Treatment of Wilson's Disease
15:30-16:00	**Tea Break**	
16:00-16:30	**Edward B. Lee** University of Pennsylvania,USA	Untangling Protein Aggregates: VCP Activators
16:30-17:00	**Aaron D. Gitler** Stanford University, USA	New TDP-43 targets in ALS
17:00-17:30	**Boxun Lu** Fudan University,China	New Insights into Huntington's Disease Mechanisms: beyond polyQ proteins

Zhiying Wu

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Dr. Zhi-Ying Wu is a Qiushi distinguished professor of Zhejiang University, a Chief physician and Director in Department of Medical Genetics and Center for Rare Diseases at Second Affiliated Hospital of Zhejiang University School of Medicine. Dr. Wu has long been committed to the screening of causative genes and the study of precision medicine in hereditary rare diseases. She has led 10 national natural science foundation projects and has been selected as "Winner of the National Outstanding Youth Science Fund" in 2011. She has been selected as "National Candidates for the New Century Millions of Talents Project", "Young and Middle-aged Experts with Outstanding Contributions to National Health and Family Planning" and "The State Council Special Allowance Experts". Serving as corresponding author, Dr. Wu has published over 150 SCI papers in esteemed international journals, such as Nature Genetics, The Innovation, Molecular Neurodegeneration, Brain, Neurology, Movement Disorders, Cell Reports, Advanced Science, Cell Discovery and others. She has been invited to give keynote or special reports at international academic conferences over 20 times.

Boxun Lu

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Dr. Boxun Lu is currently a full professor at Fudan University, China. He obtained his Ph.D. degree at University of Pennsylvania, and then became a Presidential Postdoctoral Fellow at Novartis. In 2012, Dr. Lu started his own lab at Fudan University, studying Huntington's disease and other neurodegenerative disorders with a focus on degrading the pathogenic proteins for potential therapeutic treatment for these diseases. He proposed the original concept of ATTEC (Autophagy-tethering compounds) and worked with key collaborators to lead the studies of ATTECs tackling various targets such as polyQ proteins and organelles such as lipid droplets and mitochondria. He also identified novel genetic modifiers of different disease proteins. His most recent work revealed potential pathological mechanisms mediated by RNA and protein condensates in the cells.

Aaron D. Gitler

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Aaron Gitler, Ph.D., is the Stanford Medicine Basic Science Professor in the Department of Genetics at Stanford University. He received his B.S. degree from Penn State University and did his PhD studies on cardiovascular development in the laboratory of Dr. Jonathan Epstein at the University of Pennsylvania. Then he performed his postdoctoral training with Dr. Susan Lindquist at the Whitehead Institute for Biomedical Research and MIT. In 2007, he established his laboratory at the University of Pennsylvania and moved to Stanford in 2012. His laboratory has been using a combination of yeast and human genetics approaches to investigate pathogenic mechanisms of ALS. His laboratory has made several fundamental discoveries into neurodegenerative disease mechanisms. These discoveries include discovery of modifiers of aggregation and cytotoxicity of the FTD/ALS disease protein TDP-43, a mechanism to explain how FTD/ALS-linked TDP-43 mutations affect the protein and contribute to disease, and the discovery of novel genetic contributors to human FTD and ALS, including mutations in the ataxin-2 gene as one of the most common genetic risk factors for ALS and a role of cryptic splicing of UNC13A and other synaptic protein encoding genes as a mechanism in ALS and FTD. Gitler's work has helped to uncover unexpected and novel therapeutic targets for ALS, including preclinical studies of ataxin-2 as a therapeutic target for sporadic ALS, demonstrating that reduction of ataxin-2 levels markedly extends lifespan in TDP-43 transgenic mice.

Don W.Cleveland

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Don Cleveland is Professor and Chair of Cellular and Molecular Medicine at UC San Diego. He has been elected to the U.S. National Academy of Sciences and National Academy of Medicine. He is an elected fellow of the American Association for Cancer Research. He has identified principles of genome instability in cancer, demonstrating that single chromosome missegregation can trigger repeated chromosome shattering (chromothripsis) that initiates and drives genome evolution in cancer. For this work, in 2019 he became the 15th recipient of India's Genome Valley Excellence Award and the 2022 E.B. Wilson Medal from the American Society for Cell Biology. In neurosciences, he purified and characterized the first microtubule associated protein – tau – which misassembles in affected neurons in Alzheimer's disease and chronic brain injury. He uncovered mechanisms underlying the major genetic forms of Amyotrophic Lateral Sclerosis (ALS) and developed "designer DNA drugs" for silencing disease-causing genes responsible for the major diseases of the nervous system, with clinical trials now ongoing in ALS, Huntington's, Parkinson's, and Alzheimer's diseases. For his efforts in neurosciences, he received the 2018 Breakthrough Prize in Life Sciences and the 2023 Rainwater Prize.

Edward B.Lee

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Edward B. Lee graduated Phi Beta Kappa and with honors from Stanford, and obtained his MD and PhD degrees from the University of Pennsylvania under the mentorship of Virginia M.-Y. Lee, PhD. After clinical training in Neuropathology with John Q. Trojanowski, MD, PhD, Edward was appointed to the faculty within the Department of Pathology and Laboratory Medicine where he is now the Director of the Center for Neurodegenerative Disease Research Brain Bank, Co-Director of the Institute on Aging, Associate Director of the Penn ADRC, and principal investigator of the Translational Neuropathology Research Laboratory, supporting studies on the molecular neuropathology of AD, FTD, ALS, and trauma related neurodegeneration. With over 250 publications, he has contributed to our fundamental understanding of numerous aging-related neurodegenerative diseases including the discovery of a novel form of dementia called vacuolar tauopathy linked to mutations in VCP and the implementation of cryo-electron microscopy of human brain tissue. Edward is also dedicated to educating the next-generation of neuroscientists and physician-scientists as the course director for a national career development workshop for neuropathologists. Edward's vision is to promote multidisciplinary interactions across basic, translational, clinical and educational domains to better our fundamental understanding of aging as the basis for discovering novel therapeutics and diagnostics for aging-related diseases.

Yichang Jia

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Dr. Yichang Jia, Ph. D., is a Tenured Professor in School of Basic Medicine, Tsinghua University. He is a Principal Investigator at the Peking-Tsinghua Joint Center for Life Sciences, the IDG/McGovern Institute for Brain Research at Tsinghua University, and the Tsinghua Laboratory of Brain and Intelligence (THBI). Dr. Jia received his Ph.D. in neuroscience, Institute of Neuroscience (ION), Chinese Academy of Sciences, Shanghai (2006). Subsequently, he joined Dr. Susan Ackerman's laboratory at The Jackson Laboratory/ Howard Hugh Medical Institute for his postdoctoral training in mouse genetics and neurodegeneration..In 2012, Dr. Jia established his own laboratory at Tsinghua and focuses on: 1) the disease mechanisms underlying neurodegenerative/neurodevelopmental disorders caused by RNA metabolism abnormalities and misregulation of ER homeostasis (Brain, 2020; Protein & Cell, 2023; Cell Research, 2023); 2) the molecular mechanisms underlying mammal vocalization and their dysfunctions in neurodevelopmental disorders (Molecular Psychiatry, 2023); 3) Generation of new cell and animal models for neurological/neurodegenerative diseases and exploration of gene therapies for these diseases (Brain, 2020; Nat. Commun., 2023). In addition, Dr. Jia is an entrepreneur and founder of Sineugene, a biotech company focusing on gene therapy for brain disorders. The first pipeline product of Sineugene,, SNUG01, has been conducted IIT (Investigator-Initiated clinical Trial) in ALS (Amyotrophic Lateral Sclerosis) patients. The progress has been reported by well-known domestic and international media, including People's Daily, South China Morning Post, and BNN (Breaking News Network).