The CurePSP International Research Symposium
San Francisco

October 26-27, 2017

Mission Bay Conference Center at UCSF
1675 Owens Street, San Francisco, CA
The CurePSP International Research Symposium
San Francisco

CurePSP’s annual International Research Symposium is the event that most effectively renews my energy in PSP research and provides new ideas and collaborations. This year’s topics range from autophagy, a hot, new idea being examined by many labs, to more tentative discoveries with huge potential such as arginase 1 overexpression. We will hear about using new technologies to sequence the exome in PSP and to re-examine lytico-bodig as a key to the tauopathies. We will learn of discoveries with potential for near-term application in diagnosis of tauopathy (skin biopsy) and its prevention (chronic traumatic encephalopathy). We will hear the latest on perhaps the most important story in PSP in the last few years, the increasing involvement of the pharmaceutical industry. Also on the agenda are immediate, practical advances such as a protocol to assess functional ability in PSP, updates on PSP and FTD registry cohorts, and an announcement launching the CurePSP Centers of Care.

I hope this year’s Symposium will be as energizing for your thinking and your work as I expect it to be for mine.

Lawrence I. Golbe, MD
Chair, Scientific Advisory Board

SYMPOSIUM SCHEDULE

THURSDAY, OCTOBER 26TH

Welcome Reception: 5:00–7:00pm
Thirsty Bear Brewing Company
661 Howard Street
San Francisco, CA 94105

FRIDAY, OCTOBER 27TH

Registration and Breakfast: 7:30–8:15am
Symposium: 8:15am – 5:15pm
Poster Presentation and Cocktail Reception: 5:15 – 7:00pm
Mission Bay Conference Center at UCSF
Fisher Banquet Room
1675 Owens Street, #251
San Francisco, CA 94158
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Our mission is clear.

We are pioneers in neuroscience.

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**2017 International Research Symposium Agenda**

**THURSDAY, OCTOBER 26TH**
**Thirsty Bear Brewing Company**
661 Howard Street, San Francisco, CA 94105
5:00–7:00pm **Welcome Reception**

**FRIDAY, OCTOBER 27TH**
**Mission Bay Conference Center at UCSF**
1675 Owens Street, San Francisco, CA 94105
Fisher Banquet Room

7:30–8:15am **Registration**

8:15–8:30am **Welcome and Introductions**
Alex Klein
Vice President - Scientific Affairs, CurePSP
Lawrence I. Golbe
Chair of the CurePSP Scientific Advisory Board, Board of Directors, CurePSP

8:30–9:00am **Latest Findings in PSP and other Tauopathies**
Bruce Miller
University of California, San Francisco, CA (USA)

9:00–9:20am **Assessment of Guam PDC/ALS in 2013-2014**
CurePSP Grantee: Michael Geschwind
University of California, San Francisco, CA (USA)

9:20–9:40am **Small Molecule Activators of Phospholipase D and Tau Clearance via Autophagic Flux**
CurePSP Grantee: W. Haung (Ho) Yu
Columbia University, New York, NY (USA)

9:40–10:00am **Tau in Peripheral Tissues of PSP and CBD**
CurePSP Grantee: Brittany Dugger
University of California, San Francisco, CA (USA)

10:00–10:20am **Coffee Break and Poster Session**

10:20–11:00am **Repetitive Head Impacts and Tauopathies: The Need for Collaborative Research to Move Science Forward**
Robert Stern
Boston University, Boston, MA (USA)

11:00–11:20am **Brain Bank Update**
Dennis Dickson
Mayo Clinic, Jacksonville, FL (USA)

11:20–11:40am **Investigating Functional Ability in PSP**
CurePSP Grantee: Adam Gerstenecker
University of Alabama, Birmingham, AL (USA)

11:40am–12:00pm **CurePSP Center of Care Initiative**
Lawrence I. Golbe

12:00–1:00pm **Lunch and Poster Session**

1:00–2:10pm **Clinical Research**
Chair: Lawrence I. Golbe
Clinical Trials Overview
Adam Boxer
University of California, San Francisco, CA (USA)

Tau-Targeted Antisense Oligonucleotides Findings in Mice and its Implications for Patient Therapies
Kathleen M. Schoch
Washington University, St. Louis, MO (USA)

**Pharma Updates:**
Beatrice Rendenbach-Mueller
AbbVie Deutschland, Ludwigshafen (Germany)
Roger Lane
Ionis Pharmaceuticals, Carlsbad, CA (USA)
Tien Dam
Biogen, Cambridge, MA (USA)

2:10–2:30pm **ARTFL/LEFFTDS Update**
Adam Boxer
University of California, San Francisco, CA (USA)

2:30–2:45pm **FTD Disorders Registry Update**
Dianna Wheaton
FTD Disorder Registry San Francisco, CA (USA)

2:45–3:10pm **Coffee Break and Poster Session**

3:10–4:20pm **PSP Genetics Consortium – Update**
Chairs: Patrick Brannelly
Program Director - Tau Consortium
Jeff Friedman
Director - PSP Genetics Consortium
Gerard Schellenberg
University of Pennsylvania, Philadelphia, PA (USA)
Giovanni Coppola
University of California, Los Angeles, CA (USA)
Brian Kraemer
University of Washington, Seattle, WA (USA)
Vivek Swarup
University of California, Los Angeles, CA (USA)

4:20–4:40pm Impact of Arginase1 Over-Expression and SAT1 Deficiency during Tauopathies
CurePSP Grantee: Daniel C. Lee
University of South Florida, Tampa, FL (USA)

4:40–5:00pm **The Establishment of a Preclinical Model for Progressive Supranuclear Palsy**
CurePSP Grantee: Stewart Clark
University of Buffalo, Buffalo, NY (USA)

4:45–5:00pm **Closing Remarks**
David Kemp, President of CurePSP

5:05–5:15pm **Closed Session and Cocktail Reception**

* Agenda is subject to change
Symposium Speakers

Bruce Miller
Dr. Miller holds the A.W. and Mary Margaret Clausen Distinguished Professorship in Neurology at the University of California, San Francisco (UCSF), and directs the UCSF Memory and Aging Center. He is a behavioral neurologist whose work in frontotemporal dementia and other neurodegenerative conditions emphasizes brain-behavior relationships and the genetic and molecular underpinnings of disease.

Michael Geschwind
Dr. Geschwind received his M.D. and Ph.D. in neuroscience at the Albert Einstein College of Medicine in New York. He completed his internship in internal medicine at UCLA Medical Center, his neurology residency at the Johns Hopkins University in Baltimore, and his fellowship in behavioral neurology at the UCSF Memory and Aging Center (MAC). He is now on faculty in the UCSF Department of Neurology where he is Professor of Neurology at the MAC.

W. Haung (Ho) Yu
Dr. W. Haung (Ho) Yu is an Assistant Professor at the Taub Institute for Alzheimer's Disease Research and in the Department of Pathology and Cell Biology at Columbia University. His research focuses on clearance mechanisms of proteinopathies in PSP, CBD, FTD, Alzheimer's and Parkinson's disease. This research includes understanding why proteins like tau build up in neurons in neurodegenerative disorders and development of small molecules for improving its clearance via the autophagic-lysosomal pathway and removal of proteotoxic substrates.

Brittany Dugger
Dr. Brittany Dugger completed her undergraduate studies at Michigan State University, received her Ph.D. from the Mayo Clinic Graduate School, completed a postdoctoral fellowship and was an associate scientist at the Banner Sun Health Research Institute. She has been a faculty member at the University of California, San Francisco, for the past two years leading a neuropathology core to support drug and biomarker discovery programs and recently accepted a faculty position at the University of California Davis.

Robert Stern
Dr. Robert Stern is Professor of Neurology, Neurosurgery, and Anatomy and Neurobiology at Boston University (BU) School of Medicine, where he is also Director of the Clinical Core of the BU Alzheimer's Disease Center (one of only 30 centers funded by NIH). He is also Director of Clinical Research for the BU Chronic Traumatic Encephalopathy (CTE) Center. A major focus of Dr. Stern's research involves the long-term effects of repetitive brain trauma in athletes, including the neurodegenerative disease, CTE.

Dennis W. Dickson
Dr. Dickson is the Neuropathology Core Leader for the Mayo Clinic Alzheimer Disease Research Center (NIA) and Director of the Mayo Clinic Udall Center for Excellence in Parkinson's Disease Research (NINDS). His professional career started at Albert Einstein College of Medicine, where studies led to the recognition that tau, not amyloid, was the most important correlate of dementia. He received his B.S. and M.D. degrees from the University of Iowa College of Medicine, his awards include the Metropolitan Life Award (2001) and Potamkin Prize (2011).

Patrick Brannelly
Patrick Brannelly holds a BA in Psychology from Harvard College and an MBA with Distinction from Harvard Business School and is currently the Program Director of the Tau Consortium. Its mission is to accelerate the development of new treatments for Alzheimer’s, PSP, and other neurodegenerative diseases. He currently serves on the Board of the FTD Disorders Registry and is a former Board Member of the Alzheimer's Association, National Capital Chapter.
Adam Gerstenecker
Adam Gerstenecker, Ph.D., is an Assistant Professor of Neurology at the University of Alabama at Birmingham (UAB). He is a neuropsychologist with particular interest in PSP. His prior work has detailed the cognitive and behavioral aspects of PSP and their clinical correlates. More recently, his work has focused on instrumental activities of daily living (IADLs) in PSP, with a focus on IADL scale development.

Lawrence I. Golbe
Lawrence I. Golbe, MD, is Professor of Neurology at Rutgers Robert Wood Johnson Medical School in New Brunswick, NJ. He led the clinical portion of the project that in 1997 identified the first known Mendelian mutation causing Parkinson’s disease in the gene for alpha-synuclein. He devised the PSP Rating Scale, which since its publication in 2007 has become the standard clinical measure and treatment outcome marker for PSP world-wide. He has worked closely with CurePSP since 1992, presently as Director of Clinical Affairs, chair of its Medical Advisory Board, and a member of its Board of Directors.

Adam Boxer
Adam L. Boxer, MD, Ph.D., is a Professor of Neurology and the Vera and John Graziadio Scholar in Alzheimer’s Disease Research at the University of California, San Francisco (UCSF), where he directs the Neurosciences Clinical Research Unit. He also directs the Alzheimer’s Disease and Frontotemporal Lobar Degeneration (FTLD) Clinical Trials Program at the UCSF Memory and Aging Center, where he participates in the evaluation and management of patients and attends on the Moffitt Inpatient Neurology Service.

Kathleen M. Schoch
Kathleen Schoch, Ph.D., is a postdoctoral researcher in the laboratory of Timothy Miller, M.D., Ph.D. at Washington University in St. Louis, Missouri. Her research is focused on employing antisense oligonucleotide strategies to reduce or modify toxic proteins in mouse models of Alzheimer’s disease and tauopathies. She strives to understand the molecular mechanisms of neurodegeneration to direct the development of novel, clinically relevant treatment strategies.

Beatrice Rendenbach-Mueller
Beatrice Rendenbach-Mueller, Ph.D., serves as a Senior Project Leader in the Department of Neuroscience Development at AbbVie. Over her career, she held various positions in Clinical Research, Project Management and Discovery at AbbVie, Abbott, and BASF. Beatrice graduated as a chemist at the University of Bonn, Germany, in 1984, and earned a doctoral degree in natural sciences with a thesis in organic chemistry from the RWTH Aachen University, Germany, in 1987.

Roger Lane

Tien Dam
Tien Dam, MD, was trained in the U.S. and is board certified In Internal Medicine and Geriatrics. She has 14 years’ experience in academia and 2+ years in the pharmaceutical industry. At Biogen, she is the Medical Director for the anti-tau antibody currently being investigated as a potential treatment in Progressive Supranuclear Palsy.
Dianna K.H. Wheaton
Dr. Wheaton joined the field of FTD research as Director of the FTD Disorders Registry in January 2016. She has more than 20 years of clinical science research experience and genetic counseling for patients and at-risk family members. Dianna directs Registry operations working directly with persons diagnosed with FTD and their families, and leading outreach efforts to the lay and health professional communities.

Gerard Schellenberg
Dr. Schellenberg received his Ph.D. at the University of California, Riverside, in 1978. Subsequently, he was a Research Professor at the University of Washington. In 2008, he became a Professor in the Department of Pathology and Laboratory Medicine in the Perelman School of Medicine, University of Pennsylvania. His career has focused on applying advanced genome technology to the problem of finding the underlying causes of human diseases.

Giovanni Coppola
Giovanni Coppola, MD, is a Professor of Psychiatry and Neurology at the University of California, Los Angeles. The long-term goal of his work is to advance the understanding of the genetic architecture of neurodegenerative disorders by adopting a combination of genetic (sequencing, genotyping), genomic (gene expression, epigenetics), and bioinformatic (integrative network analysis) approaches.

Brian Kraemer
Dr. Kraemer completed his postdoctoral studies with Gerard Schellenberg, where he focused on developing animal models of neurodegenerative tauopathies. Dr. Kraemer has developed animal models of the tau pathology seen in progressive supranuclear palsy and Alzheimer’s Disease using both transgenic mice and transgenic C. elegans. Dr. Kraemer’s current work focuses on dissecting the genetic requirements for tau mediated neurodegeneration in these models.

Vivek Swarup
Vivek Swarup is a Larry L. Hillblom postdoctoral scholar at the University of California, Los Angeles, in Dr. Daniel Geschwind's lab. He completed his Ph.D. in 2012 from University Laval, Quebec, Canada, studying mouse models of Amyotrophic Lateral Sclerosis. He is currently using systems biology and genomic approaches to understand disease mechanisms in neurodegeneration and neuropsychiatry. He is also using transcriptomics and proteomics approaches to unravel novel drug targets in tauopathies including PSP, FTD, and AD.

Daniel C. Lee
Dr. Daniel C. Lee attended Lincoln University from 1995-1999, and graduated with a BS degree in chemistry. He later pursued his graduate work at Florida A&M University College of Pharmacy & Pharmaceutical Science in 1999, to graduate with his Ph.D. in Pharmaceutical Sciences in the area of lysosomal protease alterations in Parkinson’s disease.

Stewart Clark
Dr. Clark received his Ph.D. from the University of California, Irvine, followed by a post-doctoral fellowship at the Centre for Addiction and Mental Health in Toronto, Canada. He is currently an Assistant Professor of Pharmacology at the State University of New York at Buffalo. His research centers on the characterization of a novel neuropeptide system; urotensin II.
IN RECOGNITION OF

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for his generous support in memory of Carol J. Major

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