2016 International Research Symposium

October 27-28, 2016

Hyatt Regency on the Hudson
Jersey City, New Jersey
Dear Colleagues:

After 20 years of organizing this symposium each year as CurePSP’s Director of Research and Clinical Affairs, I have eased out of that task in favor of Alex Klein, for whom we created the new, full-time post of Vice President for Scientific Affairs. He’s terrific and I’m proud to have shown him the ropes. I’m continuing as Director of Clinical Affairs and as Chairman of the Scientific Advisory Board.

CurePSP is most grateful for the hard work and spirited discussions by our SAB. Its current members are Ed Burton, Ted Dawson, Dennis Dickson, Jason Gestwicki, Michael Gold, Günter Höglinger, Virginia Lee, Irene Litvan, Huw Morris, Christiane Richter-Landsberg and Jerry Schellenberg. We have been busy: In the past 12 months, we have evaluated 23 grant applications and have recommended funding for five. Many others were deserving, but funds are finite. We consider not only the scientific quality of the proposal and the skills of the investigators, but also whether the project contributes to a balance and diversity in CurePSP’s grant portfolio. We also seek balance between high-risk, high-reward projects and those with a comfortable likelihood of advancing the field modestly.

This year’s CurePSP International Research Symposium looks like a real winner. It highlights the use of stem cells as laboratory models for tauopathy. Falling into that category are most of our talks before the lunch break, highlighted by the keynote talk from Sally Temple. We will have updates on our Brain Bank, on the landscape of clinical trials, and on new clinical diagnostic criteria for PSP. Of course, the tau spread hypothesis, for better or worse known as the “prion hypothesis,” will be updated by keynoter Karen Duff with special reference to its therapeutic implications. Finally, as dusk falls over the Manhattan skyline, we will learn the unpublished results of the whole-exome sequencing project by the PSP Genetics Consortium, led by Jerry Schellenberg.

A major attraction of our symposium this year, as always, is the platform presentations of new results by our most recent grantees. These are often junior researchers with tremendous talent and promise who seek new collaborations, networking opportunities and ideas. The same is true of our poster presenters, but more so, as they haven’t yet received a grant from CurePSP and comprise our next wave of research innovators.

So, everyone, please learn, network, think -- and enjoy.

Sincerely,

Lawrence I. Golbe, MD
Chair, CurePSP Scientific Advisory Board
SCHEDULE

Thursday, October 27
Reception: 7:00 - 9:00 pm
Liberty Prime Steakhouse
111 Montgomery Street
Jersey City, NJ 07302

Friday, October 28
Registration and Breakfast: 7:30 am - 8:15 am
Symposium: 8:15 am - 5:10 pm
Poster Presentation and Cocktail reception: 5:30 pm - 8:00 pm
Hyatt Regency on the Hudson
2 Exchange Place
Jersey City, NJ 07302

HOTEL/VENUE INFORMATION

Hyatt Regency on the Hudson
2 Exchange Place
Jersey City, NJ 07302
Phone: (201) 469-1234

FREE WI-FI
Network: Hyatt meeting
Username: cure PSP
password: 102816

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IN SPECIAL RECOGNITION OF
Thursday, October 27, 2016
7:00 – 9:00 pm  Welcome Reception
Liberty Prime Steakhouse, 111 Montgomery Street
Jersey City, NJ

Friday, October 28, 2016
Hyatt Regency on the Hudson
2 Exchange Place, Jersey City, NJ 07302
Manhattan Room
7:30 – 8:15 am  Registration
8:15 – 8:30 am  Welcome & Introductions
William R. McFarland
Chair, Board of Directors, CurePSP
Alex Klein
Vice President – Scientific Affairs, CurePSP
Jeffrey S. Friedman
Head of Research Committee, CurePSP
8:30 – 9:10 am  Keynote
Investigating the Role of Microglia in Neurodegenerative Diseases Using Stem Cell Technology
Justin Ichida
University of Southern California, Los Angeles, CA (USA)
9:10 – 9:35 am  CurePSP Grantee
Characterization of Tau and Its Pathology in Oligodendrocytes Derived from Induced Pluripotent Stem Cells from Patients with a PSP-like Phenotype
R. Thóra Káradóttir
University of Cambridge, Cambridge (UK)
9:35 – 10:00 am  CurePSP Grantee
Specific Targeting of PSP Brain-Derived Tau Oligomers
Rakez Kayed
University of Texas at Galveston, Galveston, TX (USA)
10:00 – 10:20 am  Coffee Break
10:20 – 11:00 am  Keynote
Human Stem Cell Studies of Tauopathy
Sally Temple
Neural Stem Cell Institute, Rensselaer, NY (USA)
11:00 – 11:25 am  CurePSP Grantee
Elucidating PSP Genotype-Phenotype Relationships Using Human Isogenic iPSCs
John Steele
Humboldt State University, Eureka, CA (USA)
11:25am – 11:50 pm  FTD Disorders Registry Update
Dianna Wheaton
FTD Disorders Registry & The Association for Frontotemporal Degeneration (USA)

11:50 – 1:00 pm  Lunch & Poster Session
1:00 – 1:30 pm  Clinical Trials Update
Trish Caruana
President and CEO of Patient Engagement Program, New York, NY (USA)
Irfan Qureshi
Medical Director at Bristol-Myers Squibb (USA)
1:30 – 1:55 pm  New Clinical Diagnostic Criteria for PSP
Lawrence I. Golbe
Rutgers University, New Brunswick, NJ (USA)
1:55 – 2:35 pm  Keynote
Propagation and Clearance: Consequences and Therapeutic Opportunities
Karen Duff
Columbia University, New York, NY (USA)
2:35 – 3:00 pm  Coffee Break
3:00 – 3:25 pm  Brain Bank Update
Dennis W. Dickson
Mayo Clinic, Jacksonville, FL (USA)
3:25 – 4:10 pm  PSP Genetics Consortium – Update
Jeffrey S. Friedman
Managing Director of the PSP Genetics Consortium and CurePSP Board Member (USA)
Representatives of the Consortium
4:10 – 4:35 pm  CurePSP Grantee
Understanding the Relative Contributions of Genetic Risk Factors in PSP
Aimee Kao
University of California San Francisco, San Francisco, CA (USA)
4:35 – 5:00 pm  CurePSP Grantee
MOBP, STX6 & EIF2AK3 Expression and Distribution in PSP Brains
Rohan de Silva
University College London, London (UK)
5:00 – 5:10 pm  Closing Remarks & Poster Prize
David Kemp
President of CurePSP
5:30 – 8:00 pm  Poster Session & Cocktail Reception
Hyatt Regency on the Hudson
2 Exchange Place, Jersey City, NJ 07302
Harborside Room
William R. McFarland
Bill McFarland, Chair, CurePSP Board of Directors, has extensive experience in operations management, information technology, project management, process improvement, profitability enhancement, organizational development, and mergers and acquisitions. The effective use of technology as a strategic asset is his forte. Bill's hands-on experience as an operations executive, coupled with the knowledge he gained as the director and chief technology officer of a large data processing organization, give him the ability to address compelling issues and provide practical solutions in today’s fast-moving banking environment.

Alex Klein
Dr. Alex Klein was appointed Vice President of Scientific Affairs at CurePSP in July 2015. He earned his Ph.D. in neuroscience from the University of Freiburg, Germany, and is the author of several original research papers, reviews, and book chapters in the field of restoration of motor function using stem cell in the context of in Parkinson's and Huntington's disease. Dr. Klein also holds a Master's degree in biology from the University of Tuebingen, Germany.

Jeff Friedman
After receiving a BS in biology from MIT, Jeff earned a MD and PhD in Cancer Biology at Stanford University. Dr. Friedman went on to train in Pediatrics at Boston Children’s Hospital and was a fellow in Pediatric Hematology/Oncology at the Dana Farber Cancer Institute. From 2003-2012 Dr. Friedman was a Principal Investigator and Assistant Professor at the Scripps Research Institute (La Jolla, CA) studying red cell and bone marrow disorders. He is Chair of the Research Committee and a board member of CurePSP, and also the managing director of the recently formed PSP Genetics Consortium.

Justin Ichida
Justin Ichida completed his Ph.D. at Harvard Medical School in the laboratory of Dr. Jack Szostak. From 2007-2012, he was a postdoctoral fellow in the laboratory of Dr. Kevin Eggan in the Department of Stem Cell and Regenerative Biology at Harvard University. Since 2013, he has been an Assistant Professor of Stem Cell Biology and Regenerative Medicine at the University of Southern California, where he has received awards from the Baxter Foundation, the New York Stem Cell Foundation, and the Department of Defense. His current research is focused on using patient-specific disease models to define the mechanisms that lead to neuronal loss in ALS and frontotemporal dementia.

Ragnhildur Thóra Káradóttir
Ragnhildur Thóra Káradóttir did her undergraduate degree in Biochemistry at the University of Iceland, and entered the Wellcome Trust 4 year PhD Programme in Neuroscience, at UCL. Dr. Káradóttir is currently running her own lab at the University of Cambridge, at the Wellcome Trust – MRC Stem Cell Institute. Her current research focus is to understand how neuronal activity can regulate oligodendrocyte precursor cells’ (OPCs) differentiation and (re)myelination. Since starting her own lab she has demonstrated that NMDARs play a role in activity dependent myelination and the axon-OPC synaptic inputs are essential for remyelination after demyelinated injury.

Rakez Kayed
Rakez Kayed is world expert on amyloid and tau oligomers and made enormous contributions to the amyloid and tau fields. As a post-doctoral fellow, he reverse-engineered amyloid-beta oligomers and was able to successfully reproduce these oligomers in vitro. The Kayed laboratory has since made important discoveries for the role of tau oligomers in synaptic dysfunction, cell death and disease progression in AD, PSP and other tauopathies and validated them as potential drug targets. Through the application of innovative research tools and approaches, his laboratory also pioneered the first tau oligomer immunotherapy studies.
Sally Temple

Sally Temple, PhD, is the Co-Founder and Scientific Director of the Neural Stem Cell Institute located in Rensselaer NY, USA. Dr. Temple trained at Cambridge University and University College London with Dr. Martin Raff FRS. In 1989, Dr. Temple discovered that the embryonic mammalian brain contained a rare, multipotent stem cell that could be grown in tissue culture, producing both neurons and glia. Dr. Temple is currently the president-elect of the International Society for Stem Cell Research.

John W. Steele

After receiving a B.A. in psychology from Kenyon College, John earned a Ph.D. in neuroscience with Sam Gandy at the Icahn School of Medicine at Mount Sinai in New York City. He then went on to train in molecular neurobiology with Paul Greengard at Rockefeller University and in stem cell biology and genetics with Larry Goldstein at the Sanford Consortium for Regenerative Medicine at UC San Diego. His goal is to build on our understanding of human disease, to develop new platforms for drug discovery in human neurons, and to bring new therapies to the clinic for patients with rare neurological diseases.

Dianna K.H. Wheaton

Dr. Wheaton joined the field of FTD research as Director of the FTD Disorders Registry in January, 2016. She has authored numerous papers describing genotype/phenotype characterization and genetic modifiers of disease, participated as co-investigator for interventional clinical trials, and acted as investigator for genetic epidemiology studies. As Director of the FTD Disorders Registry, Dianna will manage the daily operations working directly with persons diagnosed with FTD and their families. She will assist clinicians, other researchers and organizations interested in using the registry to answer important research questions and to support clinical trials.

Trish Caruana

Trish Caruana received her Master’s in Social Work at the University of Maryland and worked as a senior clinical manager at the Johns Hopkins Hospital in the Department of Psychiatry and Neurosciences. She has extensive experience in the field of mental health and the effects of chronic illness on patients and their families. At CurePSP, Trish has spearheaded new programs and broadened the reach of education and support services to patients, care partners and healthcare professionals. In her role as President, Patient Engagement Program (PEP), Trish collaborates with pharmaceutical companies to improve recruitment and retention of study participants thereby accelerating the development of treatments and discovery of cures.

Irfan Qureshi

Irfan Qureshi, MD, is a neurologist serving as Medical Director in the Exploratory Clinical & Translational Research group at Bristol-Myers Squibb. He focuses on clinical development of targeted therapies for rare and genetically defined diseases, including progressive supranuclear palsy. Previously, Dr. Qureshi was at the Albert Einstein College of Medicine, where he held appointments of Assistant Professor in the Departments of Neurology and Medicine and Investigator at the Institute for Brain Disorders and Neural Regeneration. At Einstein, he conducted translational research with an emphasis on epigenetics, non-coding RNAs, and stem cells and regenerative medicine. Dr. Qureshi earned his MD from Einstein (Philip Hunt Scholar), and holds a BS in Biomedical Engineering from Johns Hopkins University.

Lawrence I. Golbe

Lawrence I. Golbe, MD is Professor of Neurology at Rutgers Robert Wood Johnson Medical School in New Brunswick, NJ. He graduated from Brown University and NYU School of Medicine and did residency training at NYU/Bellevue before assuming his current position in 1983. 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.
Karen Duff
Dr. Duff is professor of pathology and cell biology at Columbia University. After receiving her PhD from Sydney Brenner’s department at the University of Cambridge in 1991, she undertook postdoc positions in London with Alison Goate (1991-92) and John Hardy at the University of South Florida (1992-94). Her current interests are in exploring the role of the risk factor ApoE4 in AD pathogenesis, exploring the mechanisms and circuitry involved in spread of disease within the brain, and identifying the role and therapeutic potential of autophagy and proteasome-mediated clearance to remove pathological proteins. Dr. Duff has published more than 120 peer-reviewed research articles and received a number of prizes, including the Potemkin Prize in 2005.

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). He is past president of the American Association of Neuropathologists (2002) and recognized for Meritorious Service to Neuropathology in 2016.

Aimee W. Kao
Aimee W. Kao is a graduate of Brown University and the University of Iowa School of Medicine where she received her MD and a PhD in Physiology and Biophysics studying the molecular mechanisms of insulin signaling. Dr. Kao completed a clinical fellowship in Behavioral Neurology, then joined the laboratory of Cynthia Kenyon as a post-doctoral fellow to study the role of aging in development of neurodegenerative diseases such as Alzheimer Disease, Parkinson Disease and frontotemporal dementia. Since starting her own lab in 2011, Dr. Kao’s interests have been in understanding the basic pathophysiological mechanisms underlying genetically encoded forms of neurodegeration.

Rohan de Silva
Born in Sri Lanka, Rohan completed studied Biochemistry and Molecular Biology at the University of Bern followed by a Doctorate at the University of Oxford. He spent his first postdoctoral fellowship at Duke University Medical Centre with Professor Allen Roses and contributed to the first reports on alpha-synuclein and presenilin 1 in neurodegeneration. Rohan subsequently returned to the UK and first studied the role of cytokines in APP gene expression and since 1999, he has been at University College London, Institute of Neurology at Queen Square, with primary focus on the tau gene regulation and the role of genetic risk in PSP and CBD.

David Kemp
Dave has served as President of CurePSP, the foundation for prime of life neurodegeneration, since January of 2015. Previously, he owned his own brand strategy and design firm, Jager Di Paola Kemp Design, for 25 years with more than 100 employees, three offices and clients including Burton Snowboards, Nike, Virgin, Microsoft Xbox, Black Diamond, Levi’s, Merrell and others. Earlier, his corporate design firm, Harmon Kemp, Inc., based in New York City, worked for leading financial brands and manufacturers. Dave started his career in journalism as a community newspaper and trade-magazine editor, reporter and columnist for The Boston Globe and Manager of Public Relations for Dow Jones & Co. He is a graduate of Dartmouth College and received his MBA from Dartmouth’s Tuck School of Business.