From the President

As we emerge from a tough winter in many parts of the country, we look forward to a new season of growth. Likewise, we at CurePSP are growing through the expansion of existing programs and the cultivation of new ones during this year and beyond.

Research

Thanks to your generosity, we hope to award at least six Venture Grants this year. These provide $100,000 in seed funding to early career investigators seeking proof of concept for their innovative ideas, which may lead to larger grants. This is a unique niche for CurePSP because there are few other sources for seed funding of basic science in PSP and related diseases. We also plan to award four student research grants.

We are currently organizing our next PSP Research Roundtable in collaboration with the Rainwater Charitable Foundation’s Tau Consortium. The Roundtable brings together six pharmaceutical companies that are committed to finding treatment for PSP and other neurodegenerative diseases. In addition, we are also working with the Rainwater Foundation to plan a major research symposium in early 2020 that will bring together scientists from all over the world. Our research symposium held in London with PSPA, our UK counterpart, was a tremendous success, attracting some 220 investigators.

Our brain donation program with the Mayo Clinic also continues, with 44 brain donations funded last year.

Education and Events

With the help of Diane Breslow, LCSW, a long-time advisor to the foundation, we are expanding the library’s content offering, covering topics like advanced directives, grieving and loss, levels of care, and palliative and hospice care. Much of our literature has already been translated into Spanish, and we will add other languages as time and resources allow.

Under the direction of Dr. Lawrence Golbe, we will be publishing a second edition of our reference Guidebook with both print and interactive online editions.

We are looking forward to our Patient & Caregiver Education Day, Healthcare Professionals Education Day, Caregiver Retreat, CurePSP/UNC Family Conference, and our panel on chronic traumatic encephalopathy (CTE) led by Boston University’s eminent authority on the disease, Dr. Robert Stern. Please see the calendar on www.curepsp.org for details.

Office Operations

As you may be aware, we have consolidated our operations in New York City for greater efficiency and management oversight. Our much-appreciated VP-Scientific Affairs, Dr. Alex Klein, recently returned to his native Germany, and we are honored to have Dr. Lawrence Golbe, the leading PSP clinician and neurology professor at Rutgers University, succeed him in the role.

Most of all, I want to thank our donors, without whom none of this would be possible. You can be confident that your generous contributions are being put to good use. As always, I welcome phone calls and emails with suggestions, ideas, personal experiences, and even complaints!

For the cure,

Dave

David Kemp | 802-734-1185 | kemp@curepsp.org
Centers of Care + Volunteer Update

CurePSP has an extensive network of support that continues to grow and expand around the world. We are proud to announce that our community now has over 100 peer supporters, 60 in-person support groups in the US and Canada, and 10 online support groups. These groups are a safe space for patients, caregivers, and loved ones to connect, recharge, and voice their fears, hopes, and questions about the future.

In 2018, we set out to establish CurePSP Centers of Care, medical centers where patients can find qualified professionals to diagnose and care for patients with PSP, CBD, MSA, and related diseases. To date, we have been able to designate 16 locations across the country, and we continue to seek new opportunities.

We are so grateful for our dedicated volunteers and the consistent effort they make to help guide others on this journey.

Rush University Study

A research team led by Dr. Jori Fleisher, MD, MSCE, at Rush University Medical Center is currently conducting several studies involving patients with PSP and related disorders. With support from CurePSP, Dr. Fleisher’s team has begun an innovative home visit program for homebound patients who receive visits from an interdisciplinary care team including a nurse, a social worker, and a movement disorders specialist.

Dr. Fleisher has also begun a caregiver peer mentor program called Share the Care, and several individuals from the CurePSP network of support group leaders across the US were selected to participate. Support from CurePSP enabled these individuals to travel to Chicago to attend a mentor training session led by Dr. Fleisher and her research team at Rush.

Over the next few months, the mentors will be paired with current caregivers of patients with advanced Parkinson’s disease. Dr. Fleisher and her team are so grateful to have these amazing members of the PSP community as a part of this project.

If you would like to get involved, Dr. Fleisher’s research team is currently conducting an online survey study for patients with PSP and related disorders, including patients diagnosed with multiple system atrophy and corticobasal syndrome (CBS), and their caregivers. The study aims to understand how and why people with PSP and related disorders use various health services. Subjects will be contacted 12 months following the initial survey to complete a follow-up survey. If you are interested in participating, please contact Dr. Fleisher’s Research Coordinator, Ellen Klostermann Wallace, at 312-563-0674 or eckw@rush.edu.
The Power of Social Media

On November 27, 2018, CurePSP celebrated #GivingTuesday by coming together with our community through the power of social media. Over 24 hours, we shared 24 family stories on our Facebook and Instagram pages—stories of love, sorrow, and hope. Members of our community saw their own experiences reflected in these stories and shared them to their social media pages. These stories inspired members of our community to donate and create their own fundraisers. Together we raised over $50,000! Through social media and the dedication of our community, we were able to spread the message of hope to thousands of people around the world.

Our use of social media, specifically Facebook, has been incredible. We have a community of over 10,000 people on this social platform, which has created over 250 fundraisers since August 2018—and more are being added almost every day! From birthdays to memorials to “just because,” our community members are using their social media platforms to tell their stories and support our search for a cure.

Emily Ulrich created a #GivingTuesday Fundraiser for CurePSP and raised $1,185 in honor of her father, Lute Ulrich, who has CBD.

Kimberly Gorman Muto created a Facebook fundraiser for CurePSP and raised $2,890 in memory of her father-in-law, Salvatore Muto, who passed away from PSP.
Targeting a Cure for PSP: The PSP Genetics

Anyone who has experienced or witnessed the devastating effects of progressive supranuclear palsy (PSP) wants to know one thing: Are therapies waiting in the wings to cure this monster disease? Unfortunately, the answer is not yet. Currently, physiotherapy is the only method to improve PSP symptoms for patients—but this, too, is a short-lived option.

But great hope is on the horizon because of advancements in genetic research by the investigators who make up the PSP Genetics Consortium, a collaborative effort supported by the Tau Consortium and CurePSP. This global effort brings together a team of internationally recognized geneticists, neuroscientists, and neurologists who are fighting neurodegenerative diseases with large-scale genetic sequencing and analysis. The effort is coordinated by Dr. Jeffrey Friedman, a CurePSP volunteer and board member who lost his father to PSP in 2012. Dr. Friedman explains their mission: “We are in the process of identifying new genes that are risk factors for PSP and may be involved in other neurodegenerative diseases like Alzheimer’s, amyotrophic lateral sclerosis (ALS), and Parkinson’s. The goal is to identify genetic variants that lead us to specific pathways in the brain that malfunction in PSP, and to target therapies to repair these pathways or prevent them from being damaged in the first place.”

Relentlessly pursuing a PSP cure at the molecular level, CurePSP has been at the forefront of genetic exploration and discovery for more than 15 years. CurePSP has collaborated with researchers at the National Institutes of Health (NIH), with the Rainwater Charitable Foundation (RCF) through its Tau Consortium, and more recently, through a new Prime of Life Brain Initiative with RCF, which will combine the resources of the two organizations to support tauopathy research.

PSP and Tau

Currently, genetic studies point to the protein tau as the major driver in the development of PSP. Tau is a structural protein found in the neurons of the brain and central nervous system—cells that transmit information through electrical or chemical stimulation. In PSP, the tau protein misfolds, malfunctions, and accumulates inside and around neurons in specific brain locations. Over time, this causes progressive complications that lead to life-limiting deficits in mobility, vision, swallowing, speech, physical strength, and executive function.

To complicate matters, a PSP diagnosis can only be made by analyzing a person’s complex of symptoms; current imaging studies or tests are not yet validated. Only through brain tissue verification upon autopsy—examining where tau aggregates in the brain—can a neuropathologist unequivocally declare a diagnosis of PSP.
Hunting the Genome for PSP Clues
In 2010–2011, an important PSP genetics study shed new light by identifying specific genes that may increase an individual’s risk—although very minimally—for developing PSP (Hoeglinger et al., 2011). The study, entitled “Identification of common variants influencing risk of the tauopathy progressive supranuclear palsy,” was a large, two-part investigation that used a technique called genome-wide association, and examined genetic samples from thousands of patients: those with PSP autopsy-confirmed diagnoses compared with healthy controls. Dr. Friedman says, “The statistical power of the study’s well-curated genetic data uncovered five variants in the genome associated with PSP.” It was a critical step forward in mining the genome for PSP and provided important insights and leads for drug development.

Next Step: Examining the Exome
As sequencing technology advanced, investigators wanted a closer look at the exome section of the genome—the portion that includes all of the protein-encoding regions of genes. The exome study, coordinated by Dr. Gerald (Jerry) Schellenberg at University of Pennsylvania, examined samples from an additional 750 autopsy-verified PSP cases and found additional genes associated with the development of PSP, amyotrophic lateral sclerosis (ALS), and Parkinson’s. It was an important breakthrough, showing that different neurodegenerative disorders can share common risk genes.

Currently: The Most Ambitious PSP Genetics Study Ever
“The idea behind the whole genome sequencing study now in progress is to collect a complete and definitive data set using the latest technology,” says study director Dr. Friedman. An international roster of researchers from the PSP Genetics Consortium and the Tau Consortium have joined forces, funded with significant resources from the Tau Consortium and CurePSP, as well as a major grant from the National Institutes of Health championed by Dr. Margaret Sutherland, a program director at the National Institute of Neurodegenerative Diseases and Stroke.
The study goal is to examine more than 2,000 autopsy-confirmed PSP cases to create the world’s largest PSP genetics database; to examine the frequency of risk-related alleles across the neurodegenerative disease spectrum; and to identify novel risk alleles that are associated with the development of PSP. One and a half years into the study (Summer 2018), the genomes of 1,400 confirmed PSP cases have been sequenced, with an additional 650 samples in the process of being sequenced as of early 2019. The PSP Genetics Consortium and the whole genome sequencing it supports brings together some of the best and brightest scientists from around the world, and is committed to data sharing to generate novel ideas in pursuit of a cure for PSP.

Prominent study members include Dr. Schellenberg and Giovanni Coppola, MD, Professor of Neurology and Psychiatry and Biobehavioral Sciences at the University of California, Los Angeles, who explains, “This is an unprecedented effort to study the largest number of patients with PSP ever collected, and investigators from multiple institutions worldwide will share the data and participate in the analysis.”

Is PSP Inherited?

“There is clearly a heritable component to PSP, but it presents an extremely low risk,” says Dr. Friedman. In the general population, PSP is estimated to affect three to six people per 100,000 individuals. Carrying a PSP-risk allele may increase an individual’s chance of developing PSP to one in 5,000 to 10,000 people, but that is still a very rare occurrence.” Alex Klein, PhD, former VP-Scientific Affairs at CurePSP, says, “What we now see is a pattern of changes in the genome that perhaps plays a role in making a person susceptible to developing PSP.”

A Drug Trial Targets PSP

Currently, there are two anti-tau antibody drugs in Phase II trials in progress that target tau in PSP patients. Only time will tell if these drugs are effective. But what happens if targeted therapies against tau fail and do not control or cure PSP? What then?

Dr. Friedman explains, “As in cancer therapy, we need to identify many possible genetic targets so that we can facilitate the development of a range of therapies to prevent or cure this disease and others like it. It may be that a combination of drugs, rather than a single silver bullet, is what will ultimately be required.” Dr. Klein adds, “We need to study any type of changes in the genome of PSP patients to learn which pathways are involved in disease processes that could offer new drug targets.”

“We will not stop hunting down effective therapies until PSP is stopped. That is a promise,” says Dr. Friedman.
Staff Update

Lawrence I. Golbe, MD, one of the world’s leading clinical experts in PSP and related neurodegenerative conditions, has joined CurePSP as Director – Scientific Affairs. He succeeds Alexander Klein, PhD, who served for almost four years as head of scientific affairs. Dr. Golbe has long been associated with CurePSP as a board member, Director of Clinical Affairs, and head of its scientific advisory board (SAB). He also serves as emeritus professor of neurology at Rutgers Robert Wood Johnson Medical School, where he teaches, performs research, and has a referral practice confined to PSP and corticobasal degeneration (CBD). Dr. Golbe brings a wealth of knowledge and experience to the job as well as his long affiliation with CurePSP and the tremendous respect of our patients, families, donors, and professionals. We are so excited to have Dr. Golbe’s role here expanded to be Director – Scientific Affairs and look forward to the great work we will be able to accomplish through his leadership.

Alisa Hansen joined CurePSP in December of 2018 as the Database Administrator. Alisa oversees timely gift processing, donor acknowledgment, and accuracy and integrity of the database. Alisa has worked in the nonprofit sector for fifteen years. Thirteen of those years have been in database management. She has a B.S. degree in business management and economics from SUNY Empire State College.

Denise Forero joined CurePSP in October 2018 as the PR and Social Media Assistant. Denise works directly with the Marketing Manager, Sabrina Da Rocha, in assisting with various campaigns, promoting news and events, creating digital content, and managing all social media channels. Denise graduated from Stony Brook University in 2016 with a bachelor’s degree in a multidisciplinary study focusing on health science, psychology, and sociology.
Upcoming Events

OPPORTUNITIES FOR LEARNING AND SUPPORT

CurePSP and Texas Health Presbyterian
Caregiver Retreat
May 4, 2019
Texas Presbyterian Hospital
8200 Walnut Hill Lane, Dallas, TX 75231

The Impact of CTE: After the Heroics are Over, The Damage of Sport and War to the Brain
May 7, 2019
Royalton Park Avenue
420 Park Avenue South, New York, NY 10016

CurePSP and UNC Family Conference
June 7, 2019
Embassy Suites Raleigh Durham Airport Brier Creek
8001 Arco Corporate Drive, Raleigh, NC 27617

Sundaes to Stop PSP
June 23, 2019
The Abington of Glenview
3901 Glenview Road, Glenview, IL 60025