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CurePSPConnection

2015 INTERNATIONAL RESEARCH SYMPOSIUM

ALEX KLEIN
CUREPSP VICE PRESIDENT-SCIENTIFIC AFFAIRS



Finding a cure for progressive supranuclear palsy (PSP) and related prime of life diseases such as corticobasal degeneration (CBD), multiple system atrophy (MSA), frontotemporal dementia (FTD), amyotrophic lateral sclerosis (ALS) and chronic traumatic encephalopathy (CTE) is one of our main goals at CurePSP. As the new Vice President-Scientific Affairs, I feel very passionate about making the quest for cure a success story and propelling neurodegeneration research to the next level in therapy development. Interestingly, prime of life diseases share some of the disease-causing pathways (the misfolding and accumulation of proteins), so tackling those shared characteristics across the diseases may allow us to find cures not only for PSP, but also for the other related brain diseases.

I joined CurePSP this past July because I truly believe we can make a step change in how these diseases are treated. By saying 'we', I mean all of us together: patients, care partners, friends and families, our generous donors and supporters, researchers, and CurePSP staff, including members of our Board of Directors who know from personal experiences what

PSP does to loved ones and families. I feel very honored to be part of this team.

International Research Symposium

The 2015 International Research Symposium, held in La Jolla, California, November 5-7, included world-class researchers, awarded scientists and CurePSP grantees at this annual scientific event. Hosted at the Scripps Seaside Forum, the program was packed with fascinating talks about the current status of drug development and understanding the pathological (disease-making) processes in PSP and related brain diseases. The symposium was presided over by Dr. Lawrence Golbe, Chair of the CurePSP Scientific Advisory Board and Dr. Jeff Friedman, Chair of the CurePSP Research Committee.

The morning talks started with Dr. Marc Diamond, University of Texas Southwestern Medical Center, and Dr. Kenneth Kosik, University of California, Santa Barbara, giving updates about the disease-causing characteristics of tau, which is the protein that misfolds in prime of life diseases and causes brain cells to die. Then, Dr. Dennis Dickson from the Mayo Clinic in Florida spoke about his research at the Eloise H. Troxel Memorial

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Brain Bank. CurePSP has a long history in supporting brain donations to the Mayo Clinic. This brain donation program has been extremely valuable as it allows researchers to conduct experiments on human tissue, helping to understand the mechanisms of tau disease.

Next on the podium was Dr. Jeffrey W. Kelly, The Scripps Research Institute, who reported on his experience with a different field of protein misfolding — a disease called trans-thyretin amyloidosis. This disease affects not only the peripheral nervous system, but also the body's organs and tissues. Dr. Kelly successfully developed a drug that slows down the disease progression, and he is now interested in sharing his experience with scientists in PSP research.

Perhaps of most interest was the update on clinical trials and the subsequent panel discussion. The panel was chaired by Dr. Adam Boxer, University of California, San Francisco (UCSF), who also oversees the Alzheimer's Disease and Frontotemporal Lobar Degeneration (FTLD) Clinical Trials Program at the UCSF Memory and Aging Center. Dr. Boxer reported on his activities in clinical research and the progress in developing biomarkers for better and earlier diagnosis of PSP and other prime of life diseases. The panel discussion comprised speakers from pharmaceutical companies (Dr. Joel Braunstein, C2N Diagnostics; Dr. Roger Lane, Isis Pharmaceuticals; Dr. Irfan Qureshi, Bristol-Myers Squibb; and Dr. Darryle Schoepp, Merck Research Laboratories), academia (Dr. Boxer, UCSF, and Dr. Günter Höeglinger, German Center for Neurodegenerative Diseases, DZNE, Munich, Germany), and CurePSP (Trish Caruana on behalf of the Patient Engagement Program-PEP). All panelists noted great progress in developing new treatments, with more clinical trials planned and some already recruiting patients.

It became clear, however, that joint efforts are necessary to overcome obstacles on the path to finding a cure. As many new experimental drugs are in the pipeline, new clinical trials need to be carefully planned and patients made aware of new studies. PEP wants to fill this gap and increase the visibility of clinical trials and facilitate the connections between the different study centers and patients.

Heading into the late-afternoon sessions, two CurePSP grant awardees presented their research results. Dr. Christoph Wiessner from Asceneuron, Switzerland, reported on the development of a potential new therapeutic target that was investigated with the help of this grant. Dr. Natura Myeku, Columbia University, New York, showed her exciting results reusing an existing and approved drug for clearing misfolded tau from the brain.

The closing session of the symposium was on the genetics of PSP and related diseases. The panel was chaired by Dr. Friedman and the three panelists (Dr. Giovanni Coppola, University of California, Los Angeles; Dr. Hoeglinger, DZNE, Germany; and Dr. Gerald Schellenberg, University of Pennsylvania) discussed their discoveries and future plans in finding cures for these diseases. They emphasized the importance of working jointly and cross-continentially to speed up the drug discovery for PSP.

An important part of the symposium was the poster session, which occurred during lunch. Ten posters were introduced and presented by the respective researcher. The award for best poster was given to Dr. Dominique Caparros-Lefebvre from Centre Hospitalier de Wattrelos, France. Her study investigated a geographical cluster of PSP in Northern France and the potential environmental causes of such increased numbers of PSP patients.

After a long but scientifically outstanding day, Drs. Golbe and Friedman adjourned the meeting, making the 2015 International Research Symposium the most successful in CurePSP's history. Prominent scientists in PSP research gave stimulating talks, and vivid discussions disclosed great progress in drug development that had been made over the past years. We are excited to hear about more new developments at the 2016 International Research Symposium in New York City.

Patient Engagement Program (PEP)



Our cooperation with the research community also includes pharmaceutical companies with whom we have established close relationships through the newly formed Patient Engagement Program (PEP). Trish Caruana, CurePSP Executive Vice President and President/Executive Officer for PEP, has observed over the years that both academia and pharmaceutical companies were in need of assistance with conducting clinical trials for new drug development. Many studies fail because of insufficient patient recruitment or high dropout rates. PEP was established to help address these issues.

The primary goal of PEP is to connect patients with clinical trials and to offer support services for the pharmaceutical industry. Because CurePSP has established a strong relationship with patients and care partners through support groups and many other outreach activities, we can serve as the missing link between clinical trials and patients. You can read more about the exciting work of PEP in this newsletter.

None of our work would be possible without the support of our generous and loyal donors. Thanks to your donations, PSP research has made great progress leading to many clinical trials and potentially new therapies. I encourage you to contact me with your thoughts and ideas at klein@curepsp.org or 917-499-9310.

Best regards,
Alex

12th Annual CurePSP Awareness & Memorial Walk



SAVE THE DATE!

2016 marks the twelfth year of the CurePSP Awareness & Memorial Walk around the lake at Mackle Park on Marco Island, Florida. The walk is scheduled for Saturday, March 12, 2016, from 10:30 am - 1:30 pm. Registration is \$25 for adults, \$10 for students. Participants receive a T-shirt and lunch, plus can partake in a silent auction and giveaways.

Proceeds from the walk help support the efforts of CurePSP to raise awareness about devastating prime of life neurodegenerative diseases. If you live in the Marco Island area, mark your calendars now for this special event. Don't miss a day of fun, prizes and more!

For more information, call 239-353-3960. Or, contact Kathleen Matarazzo Speca at specac@curepsp.org.

Join TeamCurePSP

Whether through a sporting event, silent auction, wine tasting, family yard sale, or another type of fundraiser, your support of CurePSP programs and services makes a difference in increasing awareness of neurodegenerative diseases. The funds generated from your event will benefit patient, family and care partner services, education for healthcare providers and worldwide research efforts.

When you are a member of Team CurePSP, you have assistance from our Development team to make your event a success. If you need help with ideas or advice on how to organize your event, CurePSP can supply you with valuable information and support to raise awareness and reach your fundraising goal.

You don't need to be a fundraiser to raise funds! There are many creative ways to coordinate events to show your support of CurePSP.

If you are interested in hosting a fundraiser, please email Kathleen Matarazzo Speca at specac@curepsp.org or call 800-457-4777, ext. 5672.



10 Questions for Trish Caruana about PEP



Trish Caruana
PEP, President & Chief Executive Officer
CurePSP, Executive Vice President

Q. What exactly is the Patient Engagement Program (PEP)?

A. As part of CurePSP's mission to find treatment and cure for PSP and other prime of life neurodegenerative diseases, we are actively focused on ensuring that clinical trials of promising compounds are able to proceed by having enough eligible participants. Through PEP, we can connect patients more quickly with clinical trials sponsored by pharmaceutical companies, and assist patients throughout their participation in the studies. These trials are a critical step in bringing drug treatments to millions of people suffering from prime of life diseases.

The efforts of PEP fall into three main areas:

1. Educating clinical trial coordinators about PSP.
2. Recruiting patients for clinical trials and helping with retention.
3. Conducting outreach to physicians about clinical trials.

Q. Is PEP a separate entity from CurePSP?

A. PEP is a subsidiary of CurePSP.

Q. How does the work accomplished by PEP differ from the research efforts CurePSP already has underway?

A. The partnerships made through PEP are our first direct collaborations with pharmaceutical companies. It's important to know that the trials conducted by the pharmaceutical companies are medication trials, while other research efforts are focused on basic science and animal models.

Q. Can you explain how the PEP initiative will be funded?

A. PEP is a fee-for-service, profitmaking business that is paid for by pharmaceutical companies. Any funds generated by PEP flow back to CurePSP and will be used to fund our work in the care, consciousness and cure of prime of life diseases.

Q. If PEP is a fee-for-service business, will CurePSP promote it more than other studies it is not profiting from?

A. No, CurePSP lists all of our research studies and clinical trials prominently on our website, where visitors can read more information about each and how they are different. It's important to note that the trials being conducted through PEP are medication trials.

All our research studies and clinical trials demonstrate that we are fiercely dedicated to our mission to eradicate prime of life diseases, and that we are doing everything we can to ensure accelerated research into and development of an effective treatment and cure for these devastating disorders.

Q. How will you ensure patients' "end-to-end" participation in clinical trials? Does this mean that patients can't drop out of the trials?

A. We will help patients in their "end-to-end" participation by providing case management services to each patient in the trials. CurePSP has always been by the side of patients, caregivers and families affected by PSP and other prime of life neurodegenerative disorders, guiding them through their journeys. They are our number one priority, and these trials are a critical research step to bringing drug treatments to them. With PEP, we are looking forward to working with patients and their families to take this monumental next step toward achieving our goal – safe and effective treatments – together.

Q. Why is PSP the focus of PEP? Why did you select it over one of the other disorders? Will PEP ever expand the program to focus on the other diseases?

A. PEP will initially target PSP, which, as our name suggests, is our organization's primary focus. PSP is the subject of intensive research and several clinical trials related to this disease are in development. Because mechanisms in PSP are involved in multiple prime of life disorders – and in other common neurodegenerative diseases, including Alzheimer's disease and Parkinson's disease – many researchers are hopeful that finding an effective treatment for PSP will open new pathways for treatment of other neurodegenerative disorders.

Q. Can you tell us more about the drugs that are being tested in these trials? How promising are they?

A. AbbVie recently initiated a Phase 1 clinical trial for PSP, and trial enrollment is ongoing. AbbVie's asset received orphan drug designation for PSP by the U.S. Food and Drug Administration.

Bristol-Myers Squibb also recently initiated a Phase 1 clinical trial for PSP, and trial enrollment is ongoing. Bristol-Myers Squibb's asset received orphan drug designation for PSP from the U.S. Food and Drug Administration, as well.

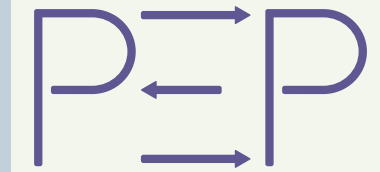
Q. Is there a timeline for when patients can expect a treatment? One year? Five years? Ten years?

A. We cannot speculate about a timeline. But we are extremely excited about PEP and taking this next step of connecting patients more quickly with clinical trials sponsored by pharmaceutical companies, which is critical in bringing drug treatments to millions of sufferers.

Q. Your title is now President & Chief Executive Officer of PEP. Are you still CurePSP Executive Vice President?

A. Yes, I am extremely happy to have dual roles within CurePSP and PEP and managing both these important work streams.

This interview was conducted by Cecelia Huffman, CurePSP Marketing and Communications Manager and CurePSP Connection Editor-in-Chief



PATIENT ENGAGEMENT PROGRAM

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Current Clinical Trials

Multiple Ascending Dose Study of BMS-986168 in PSP Patients. This study will evaluate the safety and tolerability of multiple ascending intravenous infusions of BMS-98616, and assess the pharmacodynamics of BMS-986168 on cerebrospinal fluid (CSF) extracellular tau (eTau) concentrations in patients with PSP. For more information, please visit PSPclinicalstudyinfo.com or call 855-907-3286..

Safety, Tolerability, and Pharmacokinetics of C2N-8E12 in Subjects With Progressive Supranuclear Palsy. This study will evaluate the safety and tolerability (maximum tolerated dose (MTD) within the specified dosing range) of single intravenous (IV) infusion of C2N-8E12 in patients with progressive supranuclear palsy (PSP). For more information, please call 702-487-0133.



2015 Care Partners Retreat

a special day full of relaxation techniques, everyday meditation methods, sensible eating strategies, chair massages and coping mechanisms.

Highlights of the day included a compassionate and thoughtful presentation by Shirali Nigam, illustrating how a granddaughter's love transcended the PSP diagnosis of her grandfather; ways to create meals that are simple, nutritious and delicious; and a live flute concert featuring beautiful imagery and easy meditation techniques. During the Community Support Building session, attendees had the opportunity to interact with each other using dance and mirroring performances.

The day also featured a discussion concerning the issue of neglecting a care partner's needs, including sexual needs, and a session exploring issues of guilt and self-care with a 3-Step Transformational Process: Awareness... Acceptance... Allowance. During the day, the attendees had opportunities to connect with one another and share personal stories of their journeys with these prime of life diseases. All the care partners truly enjoyed a day built especially for them and the chance to unwind.

CurePSP is looking forward to hosting another Care Partners Retreat in 2016. If you are interested in being a speaker or would like to suggest a location, please contact:

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443-578-5669 or 800-457-4777, ext. 5669

JACLYN ZENDRIAN, MA
CUREPSP CONFERENCE MANAGER

CECELIA HUFFMAN
CUREPSP MARKETING AND COMMUNICATIONS MANAGER

CurePSP held its first Care Partners Retreat on Saturday, September 12, 2015, at the Turf Valley Resort in Ellicott City, Maryland. This event was developed to give care partners of patients with PSP, CBD and other neurodegenerative diseases

Help Others through Their Journey

If you share our passion for helping families dealing with PSP, CBD and other prime of life neurodegenerative diseases, consider becoming a peer supporter or group leader.

There are still many areas in the United States and Canada where support is not readily available or convenient for those dealing with these devastating diseases. CurePSP wants to expand the number of peer supporters and support groups to give patients, care partners and their families emotional and practical support and reduce feelings of isolation.

As a peer supporter, you accept phone calls and/or email from family members; share personal experiences; and follow up with at least three calls and/or emails within the first three months of the initial contact.

As a support group leader, you arrange regular meetings; decide if the meeting will be for patients, care partners or both; stay in contact with group members to increase a sense of connection; and have a basic knowledge of PSP, CBD and other prime of life neurodegenerative diseases, with a willingness to learn more.

And, CurePSP is always available to answer questions and assist peer supporters and support group leaders, so that your volunteering experience is rewarding for both you and those you support. If you have any questions or would like to volunteer, please visit www.curepsp.org or email info@curepsp.org. In Canada, contact MJ Overby at overby@curespscanada.ca or 1-844-CUREPSP.



Spotlight on Staff

CECELIA HUFFMAN
CUREPSP CONNECTION, EDITOR-IN-CHIEF

This new series will introduce CurePSP staff to the community we serve. The articles will highlight the backgrounds of our staff and the roles they play in awareness, education, care and cure for devastating prime of life neurodegenerative diseases.



Alex Klein, PhD,
Vice President-Scientific Affairs

Meet Dr. Alexander Klein, the new Vice President-Scientific Affairs at Cure PSP. With a PhD in neuroscience, Alex has an impressive list of published research relating to Parkinson's disease and movement disorders. His studies have included looking at how environmental factors, such as stress or pesticides, play a part in Parkinson's disease and developing a rating scale for Huntington's disease. He also investigated the effects of brain repair on motor symptoms using stem cells in Parkinson's disease models. In addition to his background in neuroscience, Alex's experience in science management and coordination offers a unique skillset to the CurePSP team.

A native of Germany, Alex did his post-doctoral work in Alberta, Canada and Wales, UK, and has also lived and studied in France and England. Before coming to the United States, he was a senior research associate at Hanover Medical School in Germany, and worked as a scientific officer at the Project Management Agency, which is part of the German Aerospace Center in Bonn, Germany. In this role, Alex managed the European initiative, ERA-NET NEURON, which is a large neuroscience funding measure.

At CurePSP, Alex is the primary contact person for all matters regarding the science around the prime of life diseases, which includes reaching out to individuals who are not scientists and translating the science into lay terms. A typical day involves screening what research has been published in the last 24 hours, developing research strategies, managing the grants program, and meeting with scientists to encourage their involvement in the prime of life research community. In addition, Alex helps to organize the CurePSP International Research Symposium, which is held every year.

"This is a very exciting time for prime of life neurodegenerative diseases because there are new therapies in the pipeline," says Alex. As Vice President-Scientific Affairs, his goals are to position CurePSP as a major player in the scientific world, promote new therapies, and set up a system to increase funding for the grants program.

Alex is based in the CurePSP New York office and loves living there. "In New York, every day is different and there is so much to do and see," according to Alex. In his spare time, he enjoys going to jazz concerts and running in Central Park. Alex is very excited to be a part of the CurePSP team and looks forward to the day when there is a cure for devastating diseases such as PSP.



Unlocking the Secrets of Brain Disease™

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OUR MISSION:

Awareness, education, care
and cure for devastating prime of life
neurodegenerative diseases.

Upcoming Events

www.curepsp.org

OPPORTUNITIES FOR LEARNING AND SUPPORT

SPECIAL EVENTS

12th Annual CurePSP Awareness and Memorial Walk

Saturday, March 12, 2016
Marco Island, Florida

CONFERENCES

2016 Western Family Conference

Friday, February 19 - Saturday, February 20, 2016
San Mateo, California

2016 Eastern Family Conference

Friday, March 11 - Saturday, March 12, 2016
New York, NY

2016 Canadian Family Conference

Friday, May 13 - Saturday, May 14, 2016
Hamilton, Ontario, Canada

