Research Funding News

Every year, CurePSP awards students with an interest in PSP research with summer student fellowships. This program, called the Urso Summer Student Program in PSP Research, recognizes the generous support of Paul and Ruth Urso and Ruth Urso’s struggle with PSP. CurePSP is particularly proud of this program’s goal of educating and nurturing the next generation of researchers who are trying to find a cure for PSP and related diseases.

We present the three latest grant awardees below.

Awardee: **Mr. Jacob B. Kantorowitz**, University of Pittsburgh School of Medicine
Title: Role of Oligodendroglial & Astrocytic Tau Pathology in a PSP Model
Mentor: Dr. Edward Burton

In addition to nerve cells, the human brain contains supporting cells called glia that are essential to keeping nerve cells healthy and functional. In this study, Mr. Kantorowitz will investigate how the accumulation of human tau protein in the glial cells of genetically modified zebrafish affects the health of nerve cells. His study will show whether loss of supporting cell function caused by tau accumulation is important in PSP, and whether this could be targeted for new therapies.

Awardee: **Mr. Benjamin D. Boros**, University of Alabama at Birmingham
Title: Rho Kinases for PSP and CBD Therapeutics
Mentor: Dr. Jeremy Herskowitz

Dr. Herskowitz’ research group has recently identified that a certain protein called Rho-associated protein (ROCKs) is elevated in PSP and CBD brains, and that treating cultured neurons with ROCK inhibitors both reduces tau production, and improves neuronal communication. Mr. Boros will continue to test this hypothesis. The researchers hope that some existing drugs can be repurposed to combat the over-production of harmful tau and reverse tau-induced changes in the brain, and that these results will pave the way for future therapies to tackle PSP and CBD.

Awardee: **Mr. Benjamin M. Woodruff**, Humboldt State University in Arcata, CA
Title: iCRISPRa into Neurons to Regulate Autophagic Clearance of Tau
Mentor: Dr. John W. Steele

The focus of this study will be on neurons and glial cells, which show the greatest degree of accumulation of harmful tau in PSP. Using stem technology, in conjunction with a modern gene editing technique called inducible-CRISPR-activation (iCRISPRa), Mr. Woodruff will investigate the garbage disposal system in cells (autophagy). The results of this work will allow scientists to probe the autophagy pathway of diseased neurons and glia in a way not previously possible.
Funny is Good

In the midst of winter,
I finally learned
there was in me
an invincible summer.
- Albert Camus

We were saddened to learn of the untimely death on September 10 of John T. Royer at the age of 71. John became the voice of the PSP patient with his honest, perceptive, inspirational, erudite, educational – and yes, funny – dispatches from the front lines of the disease. This issue of our newsletter is dedicated to John.

As the tributes piled up on our Facebook page, there was a common theme: John helped many, many others to get through the day-to-day struggles with the disease, whether patient or family member. Underlying his intelligence and wisdom was the firm conviction that as the body is ravaged, the spirit soars; it survives our temporal existence and remains to inspire others who will face the “monster” (as he called PSP) until we are able to find a cure.

John’s ability to find humor in suffering is found in the best of us, but few can express it as brilliantly as has he. His favorite saying – “funny is good” – became a mantra that took the pathos out of raw descriptions of his suffering, as if he was saying, “I got through another day and had a few good laughs” over human foibles and the absurdity of life. Being alive is reward enough.

Some years ago, John’s mother suffered from Alzheimer’s disease and was in a nursing home. Her days were monotonous and depressing. John writes:

“It occurred to me one day that possibly her sense of humor was still intact. Like sadness and anger, it is a basic emotion.

“I tried an experiment.

“We were sitting at the end of a hallway by a window in the nursing home and an idea occurred to me. I was going to become outrageous. A smile formed on my face and she stared at me in bewilderment. ‘Mom, do you know how I got here? I flew in on my 747 jet and parked outside the window. The door was too far off the ground so I had to open it and crawl through the window.’

“She looked at me and started laughing. I hadn’t seen her smile about anything for years. She always had this blank, serious look on her face.

“This was a good idea, I thought to myself.

“Serious doesn’t work. Funny does.”

Austrian psychiatrist Victor Frankl, in his book Man’s Search for Meaning, recounted his horrific experience as a prisoner in Auschwitz. Frankl believed that when all else has been taken away, man still has his last freedom — the freedom to “choose one’s attitude in a given set of circumstances.” John echoed Frankl when he wrote, “PSP destroys you only if you let it. The human spirit is separate from the body and eventually leaves it when the body dies.

“I told my wife that if I precede her in crossing over to the other side, I will build her a grand house, which she will never have to clean.

“That was an appealing thought to her. No housecleaning.”

“Funny is good.”

RIP John T. Royer.
Care, consciousness, and cure are central to the mission of CurePSP, but how we advocate for people with prime of life brain diseases is also important. The lack of understanding in the medical community and the public about PSP and other prime of life brain diseases is a source of pain and frustration to all those affected. The good news is that we can each do something about it.

*Advocacy is critical* to raising awareness about these diseases and advancing policies related to services and benefits for those who are suffering. To become an advocate is to stand up for the rights of those who are suffering and their loved ones who suffer with them. But the idea of advocacy remains challenging for many people, especially when related to rare diseases, and many are unclear about what they can do. They fear, perhaps, that it is too time consuming, or too difficult, or that they don’t know enough to confidently put their knowledge or opinions forward.

In truth, advocacy is a way to channel your passion and frustration into action, and there are so many ways to get involved. You can work alone from your computer keeping up to date and sharing local, state, and national policies that affect the rare disease community, or join with others to meet with your political representatives. We all have talents and ideas and our voices matter.

There is another benefit to becoming an advocate: You will become part of a community with all of those who suffer with rare diseases and experience the power of creating a united voice. You have an important place at the table and the momentum of the broader rare disease community will inspire you.

Below are some of the primary goals of one advocacy organization, the Rare Action Network (*rarediseases.org*):

- Build a unified state and local network of rare disease advocates who can take action on issues impacting patients.
- Increase awareness among local healthcare policy and decision makers of the challenges faced by the rare disease community.
- Address specific legislation related to access and coverage for essential medical therapies.

If you’re interested in becoming an advocacy volunteer with CurePSP, or joining a small committee tasked with defining objectives and an action plan, please contact our office manager, Joanna Teters at *teters@curepsp.org* or 347-294-2871.

Below are some informative websites and contact information.

**National Organization for Rare Disorders**  
National Headquarters  
Phone: 203-744-0100  
[https://rarediseases.org](https://rarediseases.org)

**Global Genes**  
[https://globalgenes.org](https://globalgenes.org)

**Legislative Advocates:**  
Every Life Foundation for Rare Diseases  
77 Digital Drive, Suite 210  
Novato, CA 94949  
Office: (415) 884-0223  
[http://rareadvocates.org/about-us/](http://rareadvocates.org/about-us/)
Understanding the Drug Research and Development Process

Developing new drugs and therapies is a very slow and complex process. This can be deeply frustrating for patients, caregivers, and their loved ones who are desperate for treatments, or a cure.

There are, however, good reasons to test any new drug: unwanted side effects, efficacy, and safety need to be evaluated before a drug reaches the market. To this end, every drug in the U.S. is evaluated in a thorough and vigorous procedure by the U.S. Food and Drug Administration (FDA), an independent federal agency that has set the standards for drug approval worldwide.

The true cost of bringing a new drug through the development process is the subject of considerable discussion. One estimate, by the Tufts University Center for the Study of Drug Development (CSDD) places it at a staggering $2.6 billion, largely funded by private industry. This amount was confirmed in a peer review journal later (source: [www.ncbi.nlm.nih.gov/pubmed/26928437](http://www.ncbi.nlm.nih.gov/pubmed/26928437)).

Other sources say that the cost of new drug development are significant lower, and are partially covered by philanthropy and governments (see: Doctors without Borders, R&D Cost Estimates: MSF Response to Tufts CSDD Study on Cost to Develop a New Drug; [http://bit.ly/2mMyTrX](http://bit.ly/2mMyTrX). and NPR, R&D Costs For Cancer Drugs Are Likely Much Less Than Industry Claims, Study Finds; [http://n.pr/2jDa7xO](http://n.pr/2jDa7xO)).

CurePSP, which is supported entirely by donors, is proud to be actively involved in sponsoring and promoting research projects into treatment and cure for PSP, CBD, and related diseases. For more information please visit our website at [www.psp.org](http://www.psp.org).

---

**THE BIOPHARMACEUTICAL RESEARCH AND DEVELOPMENT PROCESS**

<table>
<thead>
<tr>
<th>BASIC RESEARCH</th>
<th>DRUG DISCOVERY</th>
<th>PRE-CLINICAL</th>
<th>CLINICAL TRIALS</th>
<th>FDA REVIEW</th>
<th>POST-APPROVAL RESEARCH &amp; MONITORING</th>
</tr>
</thead>
<tbody>
<tr>
<td>IND SUBMITTED</td>
<td></td>
<td></td>
<td>PHASE I</td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>PHASE II</td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>PHASE III</td>
<td></td>
<td>PHASE IV</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>1 FDA-APPROVED MEDICINE</td>
</tr>
<tr>
<td>NUMBER OF VOLUNTEERS</td>
<td>TENS</td>
<td>HUNDREDS</td>
<td>THOUSANDS</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

The following table explains each step in the drug development process, and may shed some light on why we are still waiting for treatments and a cure.

Please do not hesitate to contact our Vice President - Scientific Affairs, Dr. Alex Klein, if you have any further questions, at klein@curepsp.org.

This table was taken and modified from the PhRMA brochure “Biopharmaceutical Research & Development” (http://phrma-docs.phrma.org/sites/default/files/pdf/rd_brochure_022307.pdf).

<table>
<thead>
<tr>
<th>DISCOVERY</th>
<th>BASIC RESEARCH</th>
<th>Scientists in biopharmaceutical research companies, government, academia, and for-profit research institutions contribute to understanding of the disease and potential drug targets.</th>
</tr>
</thead>
<tbody>
<tr>
<td>DRUG DISCOVERY</td>
<td>Researchers create a new molecule or select an existing molecule as the starting point, then perform tests on that molecule and optimize it to maximize its effect. They then move forward with one or more drug candidates.</td>
<td></td>
</tr>
<tr>
<td>PRECLINICAL</td>
<td>Researchers test extensively to determine if the drug is safe enough for studies in humans using lab and animal models.</td>
<td></td>
</tr>
<tr>
<td>CLINICAL DEVELOPMENT</td>
<td>IND SUBMITTED</td>
<td>The company provides FDA with an investigational new drug application (IND), which contains all preclinical testing results and plans for clinical testing, so the FDA can determine if the drug is safe enough to move to human trials.</td>
</tr>
<tr>
<td></td>
<td>CLINICAL TRIALS</td>
<td>The candidate drug is assessed for safety and efficacy in three phases of clinical trials, usually beginning with tests in a small group of healthy volunteers, and then moving into larger groups of patients.</td>
</tr>
<tr>
<td></td>
<td>NDA/BLA SUBMITTED</td>
<td>The sponsor submits a new drug application (NDA) or biologics license application (BLA) to the FDA requesting approval to market the drug. These applications contain the results and data analysis from the entire clinical development program and earlier preclinical testing, as well as the proposed labeling and manufacturing plans of the new medicine.</td>
</tr>
<tr>
<td></td>
<td>FDA REVIEW</td>
<td>The FDA reviews the NDA or BLA submission to determine if the drug can be approved for patients to use. They may solicit the opinion of an independent advisory committee.</td>
</tr>
<tr>
<td></td>
<td>FDA APPROVAL</td>
<td>Following comprehensive reviews of the medicine’s safety and efficacy, the FDA will either approve the medicine or request additional studies. If the medicine is approved, formulation, scale-up, and manufacturing of the medicine will get underway.</td>
</tr>
<tr>
<td>ONGOING STUDY OF THE MEDICINE</td>
<td>POST-APPROVAL RESEARCH AND MONITORING</td>
<td>The company monitors the drug as it is used in the larger population to capture any unexpected serious side effects and to accumulate additional data, both through formal clinical studies and through the collection of real-world evidence, which may reveal greater therapeutic potential in other indications, formulations, combinations, etc.</td>
</tr>
<tr>
<td>TOTAL</td>
<td>HOW MUCH:</td>
<td>$2.6 billion, on average</td>
</tr>
<tr>
<td></td>
<td>HOW LONG:</td>
<td>10 years, on average</td>
</tr>
</tbody>
</table>
The 12th annual Jennings Kroemer Putt for PSP was another stellar event, celebrating the memories of Ken Jennings and Cheryl Kroemer.

The event, held in Syracuse, IN, began in 2005, after Ken Jennings, a well-known local businessman and respected member of the Syracuse community, was diagnosed with PSP. At that time, Ken was an active man who enjoyed playing golf in his free time. Alarmed by his rapid rate of decline, his children Jack and Janelle were compelled to do something to raise awareness and support CurePSP, so they created the Ken Jennings Putt for PSP Golf Tournament.

The event evolved in 2014 when the Jennings family met Cheryl Kroemer, another Syracuse local, who was approaching the final stages of PSP. Cheryl’s husband, Jim Kroemer, a former publisher of the local Goshen News, reached out to the Jennings family in order to gain more insight into the disease. Sadly, Cheryl passed away later that year; the event was renamed in 2015 to include and honor her memory.

This year’s event took place on Saturday, July 29, under a bright Indiana summer sky, and with 22 golf teams in attendance they raised over $15,000 for CurePSP. Some of the day’s highlights included: a visit and interview with Andrew Dobda, who made the one day trip from Illinois, (Andrew’s wife Beverly is currently battling PSP), homemade desserts by Rosie Hill and Carol Jennings, Golf Ball Drop raffle assisted by Chief Mickey Scott and the Syracuse Fire Department (once again the Golf Ball Drop was won by Drew Parr and once again he donated his winnings back to CurePSP!), and the ever-popular two-hour cruise around Lake Wawasee on the SS Lillypad II dinnerboat.

“We created this event in 2005, and even though he couldn’t play because of the quick progression of the disease, Ken very much enjoyed attending each year in order to visit with friends and family. He is no longer with us but, through our continued efforts, we have been blessed to get to know and aid other families who have been affected by this disease,” said Jack Jennings.

Although neither family benefit directly from the funds being raised for PSP, it allows them to celebrate their loved ones’ memories, and they hope the funds and awareness will help other families avoid a similar unbearable loss.
A Poem by Bailey

We received an email from Merci Leffler, whose daughter wrote a poem about PSP for a school research project when she was 11 years of age, in honor of her grandfather, Kenneth Metzger.

Her dedication on the report read: “This is for my Grandpa that has PSP and for my Grandma. My Grandma is having a hard time and so is my Mom, which this is for them too. It is also for everyone else that has PSP.”

I AM PSP
by Bailey Nix, age 11

I am .... PSP
(Progressive Supranuclear Palsy)

I wonder.... if they will ever find a cure for me
I hear .... crying
I see... sad people
I want.... to be a cure
I am... PSP

I pretend... like I’m a cure
I feel....pain and sadness
I touch....to kill
I worry... about the people that I hurt
I cry.... sadness
I am...PSP

I understand...that I kill people
I say... I’m sorry
I dream... about people living
I hope... they will cure me
I wish... people did not die from me
I am... PSP

Kenneth Metzger, center, with grandchildren Benjamin, Bailey, Ashley, and Hannah, at Kenneth’s home, 2008

Bailey, right, with Grandad Kenneth in his home, 2009
Upcoming Events

OPPORTUNITIES FOR LEARNING AND SUPPORT

CurePSP International Research Symposium
Thursday, October 26 - Friday, October 27, 2017
San Francisco, California

PSP/CBD Research Update and Family Conference
Saturday, October 28, 2017
San Francisco, California

Southwest Patient & Medical Professionals Education Day
Wednesday, November 15, 2017
Phoenix, Arizona

CurePSP Awareness and Memorial Walk
Saturday, March 10, 2018
Mackle Park, Florida