President’s Message

We hope you are enjoying a beautiful start to summer! We’re happy to share with you an update on some of our many activities.

Prion2015 Conference: At Colorado State University this May, Lori Nusbaum and I had the opportunity to represent our families at this international gathering of scientists in the prion disease field. This year’s meeting highlighted the outstanding and innovative work occurring not only in the prion disease field but also in related neurodegenerative diseases, and how progress in each area might support the other. (You can download the abstracts here: https://prion2015.files.wordpress.com/2015/05/prion2015abstracts.pdf).

Along with CJDF board member Deana Simpson and her CJD International Support Alliance co-chair Suzanne Solvyns, I shared stories from our families at this gathering of 500 scientists. We spoke during the opening ceremony, which many researchers said helped to set a tone for the days of scientific presentations that followed.

Thanks to the Memorial Research Grants donated by our families, I was able to announce to the researchers at the conference our invitation to apply for 2016 CJD Foundation Research Grants (see our website under “info” for that announcement). I was also happy to speak at the conference with each of the recipients of our 2015 Research Grants: Emiliano Biasini, Jean-Philippe Deslys, Julie Moreno, Christina Sigurdson, and Wenquan Zou. Each one expressed their gratitude for receiving the grant, and their enthusiasm about coming to the CJD Foundation Family Conference in July to share an update on their work. Wholehearted thanks to all who have contributed to our research grant program!

2015 Family Conference: We look forward to seeing many of you at the 2015 CJD Foundation Family Conference, July 10-12 in Washington, DC. Details are available on our website and on page 12. For those not able to attend, the presentations will be posted on our website after the conference.

Advocacy: In this challenging budget year, we hope you’ll consider reaching out to your legislators, either through a meeting or a letter, to share your story and voice your support. This is especially imperative if your legislator is a member of the Appropriations Committee. If you attend our conference in Washington, consider participating in Advocacy Day on Monday, July 13. Let’s reinforce to each legislator that prion diseases have impacted their constituents at home. If you’d like to help even more with the Advocacy program, contact Lori@cjdfoundation.org.

Continued on page 2
President’s Message (continued)

Our co-Medical Directors, Dr. Safar and Dr. Appleby, and I met in early May with the staff of six Appropriations Committee members, and found interest and support for the activities of the CJD Foundation and NPDPSC in some of those meetings. We also met with the USDA to urge more vigilant animal surveillance. We will visit Washington, DC again later this year to help ensure that our cause stays on the legislators’ radar screens as the budget is reviewed and finalized.

Thank you to all who are supporting our programs, including Grand Rounds, Family Workshops, Support Groups and Teleconferences, and Fundraising. You can learn more about all of these programs in the pages that follow.

Best wishes,

Debbie Yobs

Dr. Jiri Safar, Dr. Brian Appleby and Debbie Yobs on Capitol Hill
CJD Foundation Family Workshops

**Family Workshops:** Thanks to sponsorships by Janine Kock and family, in memory of Davey Kock, and the Centers for Disease Control and Prevention, this year we have hosted Family Workshops in Atlanta and Miami. Watch for details about Family Workshops in these locations: Salt Lake City, Utah - Mid October; Indianapolis, Indiana - Mid September; Phoenix, Arizona - Possibly late summer.

Pictured in this photo are Dr. Jim Lah, Emory University, Department of Neurology; Dr. Larry Schonberger, Centers for Disease Control and Prevention; Debbie Yobs, President, CJD Foundation; and the dedicated, wonderful Atlanta families! Also pictured in this photo is family member Maria Thacker, who helped coordinate this workshop. Thank you to all!
CJD Foundation Family Fundraisers

Many families have been very busy holding fundraisers to benefit the CJD Foundation - two of them are shown below. We thank you and appreciate it very much! Please send us photos of your family fundraisers! We’d like to display them at the conference, post them on our website, and use them in future newsletters. Thank you!!!

Joe Lozito ran the NY half-marathon and raised funds in honor of his sister Lorraine Meys

Megan McGill held a comedy night fundraiser in honor of her father, Raymond McGill, who passed away from CJD, and her brother, Timothy McGill, a Staff Sergeant in the Special Forces, who was killed in Afghanistan. The proceeds from this fundraiser were split between the CJD Foundation and the Green Beret Foundation.

You Can Fundraise Just by Shopping!

If you shop at any of the places below, the CJD Foundation receives a portion of the proceeds:

- givebackamerica.com - http://www.givebackamerica.com
- Amazon Smile - http://smile.amazon.com
  on facebook https://www.facebook.com/amazonsmile?fref=ts
- Facebook - Follow us The Creutzfeldt-Jakob Disease Foundation
On October 31, the CJD Foundation will host Strides for CJD, a 5K run/walk to benefit the CJD Foundation general fund. To sponsor, donate a raffle item, or register to walk/run, visit our website. To help with fundraisers in any of our planned locations, contact Melany for more information. To start a Strides for CJD “satellite” event in your area on or around October 31, contact Melany at melany@cjdfoundation.org or 1-800-659-1991.

Listed below are the local captains.

Morris County, NJ: Melany Pritchard - melany@cjdfoundation.org
Atlanta: Maria Thacker Goethe - Contact Melany for details
Chicago: Molly Nocerino - strides4cjdchicago@gmail.com
Los Angeles (Orange County): Trevor Baierl - Contact Melany for details
West Virginia: Nikki Bland - Contact Melany for details
CJD Foundation Support Groups

Teleconferences and Support Groups: Held twice per month by phone, sporadic and familial teleconferences allow families to share their stories and get answers to their questions. Many thanks to Dr. Brian Appleby, our co-Medical Director, for sharing his time and knowledge through these programs. In addition, we continue to host monthly support groups in Cleveland and New York.

Sporadic Teleconference Support Group - Second Tuesday of every month**
Familial Teleconference Support Group - Third Tuesday of every month**
Cleveland Area Support Group - First Monday of every month
New York Area Support Group - Please check the website or call the CJDF office for information about the next scheduled meeting 1-800-659-1991.

**note: After the June call, these calls are on hiatus for July/August and will resume in the fall.

NOTE: The June Teleconference Call has been changed. Here is the information for that call:

CJD Foundation Teleconference on Testing for Familial Prion Disease

Our June meeting will be held on the fourth Tuesday of the month instead of the third. We will meet on June 23 at 7:00 p.m. Eastern. Here’s information about that meeting:

**Topic:** Genetic Testing for Prion Diseases

**Speaker:** Jill Goldman, MS, MPhil, CGC
Genetic Counselor
Columbia University

Jill will answer families’ questions about Genetic Testing for Prion Diseases, including:

- How does genetic counseling work?
- What are the risks and benefits of testing?
- What impact can genetic testing results have on families?
- How and when should I communicate my results to my family?
Meet the Researchers

In our March newsletter we announced that we had awarded five significant research grants to outstanding researchers in the prion disease field. All of these grant recipients plan to join us at our conference this July to share mid-year status reports on their exciting research projects. In the meantime, here's a preview from four of the researchers:

WENQUAN ZOU, MD, PhD
Developing Therapeutics for CJD Using Patient-Specific iPSC-Derived Neurons
National Prion Disease Pathology Surveillance Center, Case Western Reserve University School of Medicine, Cleveland, Ohio

What is the objective of your project?
The objective of our project is to employ the newly-generated human brain cells to investigate the cellular mechanism of anti-prion activity of a compound that exhibited therapeutic effects in animal models.

What will your findings mean for the prion disease community?
Our research will facilitate development of therapeutic drugs for prion diseases.

Are there any interim findings you would like to share?
We are purifying prions from infected human brains and labeling prions. The labeled prions will be used to infect the cultured stem cell-derived brain cells and then we will monitor how the compound prevents the infection and spreading of prions in the brain cells.

How has the CJD Foundation grant impacted your work?
This study is mostly supported by the CJD Foundation grant which provides funding for us to purchase experimental supplies and to hire a technician to conduct experiments.

Is there anything else you’d like to share?
We wanted to thank the CJD Foundation and CJD patients and their family members for their support.

Pictured above:
Jue Yuan, a Research Assistant from Dr. Wenquan Zou’s laboratory, is examining the patient-derived stem cells. Photographed by Aaron Foutz
EMILIANO BIASINI, PhD
Pharmacological Chaperones for the Cellular Prion Protein
Center for Integrative Biology (CIBIO), University of Trento
Trento, Italy

What is the objective of your project?
The main objectives of my laboratory are centered on the idea that the cellular prion protein (PrP<sup>C</sup>) plays an active role in prion diseases. PrP<sup>C</sup> has classically been considered a benign form of the protein, acting as a passive substrate for infectious prions, but a great deal of recent data indicates that it may mediate the neurotoxic effects of prions, as well as of aberrant proteins linked to other neurodegenerative diseases. Our efforts are currently focused on understanding the normal function of PrP<sup>C</sup> and how it could be modified to generate toxicity. In parallel, we are trying to develop new series of pharmacological agents that target PrP<sup>C</sup> directly or indirectly, with the objective of testing the therapeutic properties of these molecules in experimental models of prion diseases.

What will your findings mean for the prion disease community?
Our long-term goals are to shed new light on the basic mechanisms of prion pathogenesis and to provide novel therapeutic tools to fight prion diseases.

Are there any interim findings you would like to share?
We’ve recently identified small compounds that show encouraging results in experiments performed in test tubes and cell models of prion disease. We are now trying to further characterize these molecules and improve their biological properties. I look forward to presenting a detailed update of our results during the CJD Foundation Family Conference in Washington DC next July.

How has the CJD Foundation grant impacted your work?
I have had the honor of receiving the grant twice, in 2011 and 2015. I’ve found that receiving a grant from the CJD Foundation, where resources come directly from the fundraising efforts of patients and relatives, has a profound psychological impact on scientists, perhaps even stronger than the impact of receiving larger grants from public institutions. These grants not only support our ongoing research economically, they also give us a daily reminder that our efforts are directed toward a precise cause, which is to find a cure for those who suffer from these terrible diseases.

Is there anything else you’d like to share?
I would like to conclude with a message of optimism. The scientific community is gaining ground in the long battle against prion diseases. Multiple laboratories around the world are now devoting more attention to the basic pathogenic mechanisms of the disease. As a result, new potential pharmacological targets are emerging. I believe that important discoveries, possibly leading to new therapeutic strategies for prion diseases, are on the horizon.

Pictured Above:
Claudia Stincardini (Student)
Tania Massignan (Senior Scientist)
Emiliano Biasini (PI)
Saioa Elezgarai (Postdoctoral Fellow)
Meet the Researchers (continued)

JULIE MORENO, PhD
The Role of Cellular Chaperones and the Unfolded Protein Response in Gerstmann-Straussler-Scheinker Syndrome
Prion Research Center, Pathology and Immunology
Fort Collins, Colorado

What is the objective of your project?
The objective of the project is to identify certain genes and pathways important to the progression of Gerstmann-Straussler-Scheinker (GSS) syndrome. We will use our knowledge from previous studies, specifically the unfolded protein response (UPR) and recent studies where we have identified genes involved in various prion diseases to determine if these genes and pathways are important in the disease course of GSS. We will use both cell models and mouse models of GSS to address this.

What will your findings mean for the prion disease community?
Our findings will help us determine if there are certain genetic changes during the disease that we could inhibit or activate to halt the progression of GSS syndrome.

Are there any interim findings you would like to share?
We have identified a protein found in the cell that is important in dealing with misfolded proteins, like the misfolded prion protein. This protein, HSPB8, is increased in brains from mice expressing a GSS mutation. Interestingly this protein may also act on the unfolded protein response (UPR), which we have shown to be activated in prion disease.

How has the CJD Foundation grant impacted your work?
By receiving the CJD Foundation grant we are able to study and hopefully identify important cellular factors that are changing during GSS.

Is there anything else you’d like to share?
I would really like to thank the families who donated to the CJD Foundation and the organizers who made it possible for me to receive this grant.

Pictured Above:
Julie Moreno hard at work in the lab
CHRISTINA SIGURDSON, DVM, PhD

Mechanisms of Selective Cell Vulnerability in Human Prion Disease

University of California, Diego, California

What is the objective of your project?

Human prion diseases show remarkable variability in terms of the cells in the brain that are affected. Our objective is to define the molecules in the brain that bind different forms of prions and that lead to prion replication. We are targeting a set of molecules abundant on cell surfaces and in the extracellular matrix (between cells), known as heparan sulfate proteoglycans. These molecules are proteins that contain polysaccharides and that vary in different cells in the brain. Our group is fortunate to be collaborating with a world-renowned expert on heparan sulfate proteoglycans, Dr. Jeff Esko.

What will your findings mean for the prion disease community?

By identifying the specific heparan sulfate proteoglycans that bind prion aggregates, we will be able to better design agents that prevent the prion from binding to the neurons in the brain. Our hope is to develop a drug that prevents prion conversion and halts CJD progression in patients. We have excellent cell and mouse models to test new compounds.

Are there any interim findings you would like to share?

Sure. Dr. Patricia Aguilar, a talented postdoctoral scientist in the lab, has developed an assay for prion conversion and has found that heparin, a form of heparan sulfate that is currently used clinically for other purposes, promotes prion conversion, as we had suspected. With this assay, she will next test a variety of polysaccharides to find the ones that show the strongest interaction – these are likely to be the best inhibitors of prion conversion. We also have a genetic way to study the problem in mice to see how heparan sulfate will impact CJD disease progression.

How has the CJD Foundation grant impacted your work?

The CJD Foundation grant has greatly impacted our work by enabling our laboratory, having expertise in prion disease, to pursue these important studies together with experts in heparan sulfate proteoglycans. This interdisciplinary research project is being pursued using new tools available, developed in part by the Esko lab, and would not be possible without the assistance of the Foundation. We are very grateful for the support of the Foundation members.

Is there anything else you’d like to share?

The community of prion disease researchers is communicating with other neurodegenerative disease researchers now more than ever. It is our hope that by sharing our findings and recognizing common mechanisms among these diseases, treatments for these devastating conditions will be developed more rapidly.
Meet the Board of Directors

In the March newsletter we introduced you to some of our board members. Here are a few more!

Mark Goldfarb, Chairman

Why I participate in the CJD Foundation:

I participate in the CJD Foundation because I saw this fatal disease take the life of a close friend and mentor of mine. The very least I could do out of respect to him and his family was to try to help other families cope with the devastating effects of this disease and to try to use the skills I have to be part of an organization dedicated to finding a cure.

Ruthie George, Treasurer

Why I participate in the CJD Foundation:

I began participating in memory of a dear friend, but continued through the years in support of all families who sought out the CJD Foundation for information and guidance. I am proud to have been a part of the formation and growth of the Foundation, creation of our programs and establishment of relations with researchers as they work to unlock the mysteries of CJD.

Chuck Cash, Secretary

Why I participate in the CJD Foundation:

As one who has lost a loved one to CJD, serving on the Board of Directors gives me the opportunity to think each day about a future when we have eradicated this terrible disease.
2015 CJD FOUNDATION FAMILY CONFERENCE

The CJD Foundation Family Conference is less than five weeks away! On Monday, June 8, the registration fee increases to $200 per person. Use this link to get to the registration form, sponsorship information, and a tentative conference schedule. http://www.cjdfoundation.org/2015-registration-form

The Washington Court Hotel reservation cutoff date is TODAY, June 8, 2015. At midnight tonight, they will be releasing our room block and the special hotel rates will also be gone.

Please, if you haven’t already done so, and you plan to attend the CJD Foundation Family Conference, reserve your hotel room today by midnight by calling the hotel directly at 202-628-2100.

Call or email us at 1-800-659-1991 help@cjdfoundation.org with any questions or concerns.

Newsletter Subscription

If you would like to be on our email list please email Lori at lori@cjdfoundation.org. Please also include your mailing address for other CJD Foundation updates, including conference information and invitation, and the name of your loved one who has been affected by CJD. Your information is confidential and never shared with any 3rd parties.

Comments/Suggestions

If you have any comments or suggestions, please email us at help@cjdfoundation.org.