



WU Wolfram  
Research Clinic

# Washington University School of Medicine

## Wolfram Research Clinic Newsletter

August, 2018 Vol.12

### RESEARCH UPDATE

Dear Wolfram Community:

I am very pleased to announce that we have received notification from the NIH that our Wolfram natural history study has been **OFFICIALLY FUNDED!** This means that we can start having our Wolfram Research Clinics again, probably starting next summer and continuing for 5 more years! Samantha, Dr. Marshall and I will begin planning for the research clinic and will keep you all informed as things move along.

We are excited to continue working with Dr. Bess Marshall as our caring and dedicated Medical Director! We will also continue to collaborate with Dr. Fumi Urano by collecting samples for his lab and coordinating with the Dantrolene trial. In addition, Dr. Tim Barrett in Birmingham, UK will share MRIs from his clinical trial, so that we can learn about individual differences in neurological progression. Finally, Dr. Gordon Xu, at Mt Sinai in New York, is helping us measure the optic nerve with MRI. We are excited to work with all of the incredible scientists and clinicians on our team.

Thank you for your support and patience during this time of being in limbo. Please know that we never stopped caring and we never stopped working on understanding Wolfram syndrome and its effect on the brain and its functions. We have high hopes that the information we gain from this study will have a positive and lasting impact on all people affected by Wolfram syndrome! *Please call me or Samantha with any questions about the research clinic.*

Sincerely,  
Tamara Hershey, PhD  
Professor  
Scientific Director and Principal Investigator  
WU Wolfram Research Clinic  
[tammy@wustl.edu](mailto:tammy@wustl.edu); 314-362-5593

### CLINICAL CARE UPDATE

Dear Wolfram families,

Hello to all of the Wolfram family. We are all celebrating Dr. Hershey's fantastic grantsmanship today! We are so fortunate to have her leading the TRACK study! While we were disappointed that the clinic could not go forward this year, we did have a Wolfram researcher meeting in Paris. This year we heard of several new projects in animals with Wolfram that are getting started that should produce some helpful information soon. Dr. Barrett's trial of valproate is not quite ready to produce data, but it should not be much longer - waiting is difficult, of course! Overall, it was a productive meeting with the added perk of great food!

All the best to everyone,

**Bess Marshall, MD** ([Marshall@kids.wustl.edu](mailto:Marshall@kids.wustl.edu))

Pediatric Endocrinologist

Medical Director, WU Wolfram Syndrome Research Clinic



*Wolfram scientists and family organization leaders from around the world gathered at the Wolfram Annual Meeting in Paris.*



### **Neuropsychiatric features of Wolfram Syndrome and other genetic disorders**

As discussed in a previous newsletter, we have learned that anxiety and depression symptoms are fairly common in people with Wolfram Syndrome. These symptoms are also very common in people without Wolfram Syndrome, and may be influenced by genes, life stress, and other factors.

In preliminary examination of our data, I have also noticed some trends indicating that psychiatric symptoms and certain types of neurological signs/symptoms might be related. I plan to further analyze the data to confirm whether this is true. I would also like to take a closer look at the brain imaging findings to investigate whether specific difference in brain structure or function may be related to specific psychiatric and/or neurological symptoms.

I also hope to do some investigation comparing the types of symptoms seen in Wolfram Syndrome to those reported in other genetic disorders that affect the functioning of ryanodine receptor calcium channels (RYRs), which release calcium from the endoplasmic reticulum (ER) within cells. In Wolfram Syndrome, too much calcium is released through these channels. Studying disorders with similar disease mechanisms could help us understand what produces the symptoms of both Wolfram Syndrome and other related other disorders. It is possible that diseases with similar mechanisms may respond to similar types of treatment.

*By Angela M. Reiersen, MD, MPE*



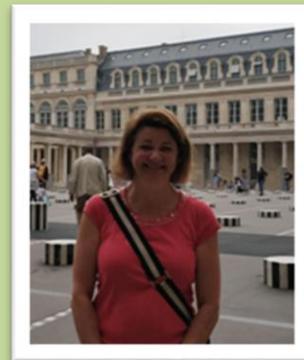
Need Help? For questions or requests regarding the Wolfram Syndrome Research Clinic please contact the WFS Research Clinic Coordinator, Samantha Ranck, MSW at 314.362.6514 or [rancks@npg.wustl.edu](mailto:rancks@npg.wustl.edu)

### **Of Note...**

The article "Longitudinal hearing loss in Wolfram syndrome" has been published in Orphanet Journal of Rare Diseases. The article is open access, so it can be freely accessed by anyone. To access the full-text use the following link: <https://rdcu.be/1Z1d>



Dr. Hershey, Dr. Marshall, Dr. Urano and Ms. Olga Neyman (graduate student in Dr. Hershey's lab) attended the 7<sup>th</sup> international workshop on Wolfram Syndrome in Paris on June 11-12<sup>th</sup> 2018, hosted by the Association Syndrome de Wolfram.



*Dr. Bess Marshall (top and bottom, left) is a pediatric endocrinologist at St. Louis Children's Hospital in St. Louis and Medical Director of the WU Wolfram Research Clinic. Ms. Olga Neyman (bottom, right) is an M.D. / Ph.D. student in Dr. Hershey's lab studying brain development.*

