



WU Wolfram
Research Clinic

Washington University School of Medicine

Wolfram Research Clinic Newsletter

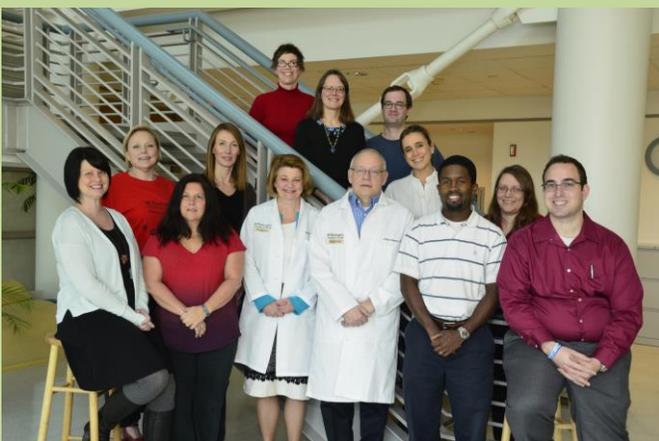
February, 2017 Vol.6

WU 2017 Wolfram Research Clinic

At this point we've attempted to contact all of the research clinic patients regarding the changes in the 2017 WFS Research Clinic. For those that we were not able to be contacted by telephone (after several attempts), a letter was mailed to their homes. It is important that you know the parameters of the 2017 clinic and how your role may or may not have changed. As a reminder, there will be a total of 9 families attending the clinic in 2017 – 6 returning and 3 new families. If you are a WU WFS Research Clinic patient and you have not heard from us or if you are unsure if you will be needed at the clinic in 2017, please contact Samantha Ranck at your earliest convenience.

Rare Disease Day, 2/28/2017

Wolfram Syndrome meets the definition of a rare disease in the USA as it affects fewer than 200,000 Americans at any given time. Rare Disease Day is celebrated on the last day of February each year. While we celebrate on a specific day, involvement is ongoing throughout the year and anyone can get involved. "The main objective of Rare Disease Day is to raise awareness amongst the general public and decision-makers about rare diseases and their impact on patients' lives." You can learn more about how to get involved at rarediseaseday.us.



Dr. Hershey and Dr. Marshall with members of the Wolfram Research Clinic Team gathering in support of Rare Disease Day.

RESEARCH UPDATE

Dear Research Clinic Families:

I would like to give you an update on the **Washington University Wolfram Research Clinic study**, also known as our 'natural history' study. This work began in 2010, initially supported by the Snow Foundation. For the past 5 years, it has been supported by a National Institutes of Health (NIH) grant in Dr. Hershey's lab (*Tracking Neurodegeneration in Wolfram Syndrome; Hershey, Principal Investigator*). The focus of this grant is on understanding the neurological changes that occur over time in Wolfram Syndrome, including the function (e.g. vision, balance) and the structure of the brain (e.g. the volume or integrity of different regions of the brain).

I wanted to thank all of you for the time, effort and support that you have provided to Wolfram research.

The trips to St. Louis, answering questions, doing tests and behind-the-scenes support have all had a huge impact. The data we collected has allowed us to determine what measurements can be used in clinical trials to detect whether a treatment is working or not. Before this study, we had no way to tell if someone's progress was getting worse or better. This information has helped in the design of Dr. Urano's safety study in St. Louis and Dr. Barrett's intervention study in Europe. In addition, we now know that there are individual differences in the severity and rate of change of brain-related effects of Wolfram Syndrome. One of our next goals is to understand why some people have mild brain-related symptoms that change slowly and some have more significant symptoms that occur earlier and change more rapidly. Such information could help families and doctors, and would be important information for clinical trials to take into account.

I also wanted to remind you that the grant supporting the WU Wolfram Research Clinic is in its last year. We are submitting a revised proposal to NIH to continue the project and will know if it will be funded by the fall of 2017. In the meantime, this summer will be a very small clinic just to finish up a third year of data collection on a small number of families.

RESEARCH UPDATE cont.

It is my hope that we can return to larger clinics in subsequent years. As always, we will keep you up to date with our WU Wolfram Research Clinic newsletters, the Snow Foundation newsletters, our website (<http://hersheylab.wustl.edu>) and phone calls, letters or emails.

Please contact me at any time if I can provide more information or assistance, I would love to hear from you!

Best,

Tamara Hershey, PhD (tammy@wustl.edu)
Professor & Principal Investigator,
WU Wolfram Research Clinic



CLINICAL CARE UPDATE

Dear Wolfram Families,

We appreciate the understanding you have all shown with regards to the smaller research clinic this year. Some of you may find it a welcome break! But, I will certainly miss seeing you all. We do hope to see you again next year if the grant funding comes through. The safety study with Dantrolene headed by Dr. Urano is underway. As the study has only recently gotten started, it is much too early to say whether Dantrolene will be safe for use in Wolfram. This study will not determine if Dantrolene affects the progression of Wolfram. A study that can give that answer will have to wait until the safety study tells whether or not Dantrolene is safe.

I have just submitted an abstract reporting on your diabetes mellitus testing – everyone’s favorite Boost test. The meeting is the 10th International Meeting of Pediatric Endocrinology and will be in Washington, D.C., this fall. Fingers crossed that they like the abstract!

I know that you all keep a close watch for new developments in care of Wolfram. So, you may have heard about the “Hybrid Closed Loop” insulin pump system from Minimed/Medtronic that has been approved by the FDA. This system is not a true “artificial pancreas” as it uses only insulin and not glucagon. But, it does have

Clinical Care Update cont.

a continuous glucose monitor that “talks” to the pump, telling the pump to give less insulin if the glucose gets too low and to give more if the glucose gets too high. If it works as the FDA studies showed, it may be something valuable for people with Wolfram diabetes. The system is not yet on the market, but you will likely hear a lot about it. The company has said they expect it to be available later this year. Unfortunately, there is usually a lag time before insurance begins to cover new developments such as this.

I have written a few letters for appeals to insurance, disability, and school accommodations. If anyone needs this sort of thing, please feel free to ask. College bound people please be aware there are some scholarships for people with various health problems, including diabetes.

If we will not be seeing you this year in the research clinic, please update Samantha if you have any health changes. We are interested in changes in your medications, any new or changed diagnoses, admissions to the hospital, illnesses, or operations, or changes in symptoms of existing problems. As always, please feel free to call or email if you have any questions, concerns, or requests.

All the best,

Bess Marshall, MD (Marshall@kids.wustl.edu)
Pediatric Endocrinologist & Research Clinic Medical Director

RESOURCES

For more information about Wolfram Syndrome and the WU Wolfram Research Clinic go to: hersheylab.wustl.edu

* Children Living with Inherited Metabolic Diseases (CLIMB) – <http://climb.org.uk>

*National Organization for Rare Disorders (NORD) – <http://www.rare diseases.org>

*American Foundation for the Blind (AFB) – www.AFB.org

*Additional information about the WU Wolfram Research Clinic study can be found at <http://ClinicalTrials.gov>; *WU IRB approval #: 201301004