



WU Wolfram
Research Clinic

Washington University School of Medicine Wolfram Research Clinic Newsletter February 2022 Vol.21

RESEARCH CLINIC UPDATE

From Dr. Hershey & Dr. Marshall

Dear WU (Washington University) Wolfram Research Clinic community:

We hope that you are doing as well as can be expected during this prolonged period of uncertainty caused by the pandemic.

Before talking about the details of the 2022 Research Clinic, we would like to take some time to reflect on its *purpose, contributions to the understanding of Wolfram Syndrome, and its role in clinical trial design and outcomes.*

First, some history:

Our WU Wolfram Research Clinic started in 2010, with the support of WU, the ADA, and the Snow Foundation. In 2012, we were awarded a large National Institute of Health grant, called an R01, to focus on the brain-related aspects of the disease. In 2018, that grant was renewed to improve our brain imaging and other measures and to add new participants, including siblings without Wolfram syndrome. In 2019, we held the largest research clinic and family conference ever! As we were gearing up for an even better 2020 research clinic, the pandemic hit, and we have been struggling to find ways to continue this project. Now that omicron is subsiding and vaccinations are widespread, we believe it is time to get started again, but with fewer people on site at any one time. Our current grant support goes until 2024, so we only have a few years left to perform this work!

Second, our purpose:

Since the research clinic is not testing or offering treatments, it can sometimes be difficult to understand what just measuring symptoms and brain structure can do. Our goal is to discover how the brain changes and what measures of neurodegeneration are the most sensitive and reliable. This information allows us to recommend meaningful ways to determine if new drugs slow or stop the neurodegeneration underlying loss of vision, balance, and other brain functions. **Without this information, clinical trials would not know whether their drugs work for brain-related aspects of Wolfram syndrome.**

Third, the contributions:

The data we have collected with your help over the past 10+ years has already been used to help design clinical trials in the UK (Tim Barrett), and the US (Fumi Urano, Neil White), inspired ongoing animal and cellular studies, and led to over 22 peer-reviewed research papers and several additional grants. Without your willingness to lie in the MRI (Magnetic Resonance Imaging) machine, drink that nasty Boost, give blood samples, and look at pictures with Dr. Hoekel, we would not know that MRI, vision, and insulin production measures can be used to tell if a medication is helping or not. We also would not know how big a problem bladder and digestion issues are for individuals with Wolfram Syndrome. In the future, the Research Clinic activities may be merged with clinical trials, helping both efforts. Thus, clinical trials and the Research Clinic can work together to achieve the goals that we all want more quickly: **effective treatments to slow or stop neurodegeneration in Wolfram Syndrome!**

Finally, thank you!

All of this depends on **YOU!** Research to find a treatment for a disease always takes a lot longer than anyone wishes it would. We thank all of you who have contributed to this long first step in finding that treatment. We appreciate the participation of as many Wolfram families as possible to get us to these important shared goals. Thank you to all who have joined us on this journey so far, and we look forward to seeing all of you very soon.

Your friends,

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2022 WOLFRAM RESEARCH CLINIC - An All-New Look!

“Hello” to all our Wolfram Research Clinic friends and families! We are so glad to be back! Over the past two years, we have all had to adjust to the many twists and turns COVID has thrown at us - and the Wolfram Research Clinic was no exception. To accommodate this new world, the clinic will look a little different in 2022.

Changes in the annual research clinic in 2022 include a hybrid approach to testing:

- **virtual testing** for assessments that can be done virtually, on-line REDCap surveys, and
- **in person mini clinics** in St. Louis for small groups of patients, siblings, and proxies throughout the year.

The 2022 clinic format offers some benefits too:

- increased flexibility when scheduling clinic visits
- a shorter visit to St. Louis
- reduced risk by limiting the time participants spend in the hospital setting

Provided the Omicron numbers continue to decrease, and another surge is not in effect, **we will begin virtual testing and scheduling the mini clinics in March 2022.**

Soon, families who have participated in a previous clinic will receive a call from Beth or Jaridd, asking if they would like to participate in the 2022 clinic as well as some additional questions. Once this information is gathered, virtual and in-person clinic visits will be scheduled, and information packets will be sent. Next, new families will be contacted and will go through the same process.

We expect challenges to pop up with the new format, yet we are confident that the 2022 clinics will be a success! We will follow updated COVID-19 guidelines throughout your visit. Currently, masks are required in hospital and associated buildings. All WU and hospital employees are required to be vaccinated.



New Team Members

The clinic’s newest team members are Alana McMichael, MA, MRSO and Jaridd Albert, MS. You may have had the pleasure of meeting Alana during the 2020 virtual clinic. Alana trained to administer the cognitive tests and took the lead in this effort by becoming the clinic’s primary cognitive tester. Jaridd is brand new to the Wolfram Clinic and will be learning the ropes. We are blessed as both are quick studies! As excited as we are for you to meet them, we are even more excited for them to meet you! Jaridd and Alana are joining the rest of the team to navigate this new path to a very different and exciting 2022 Wolfram Research Clinic!



Something Just Got Easier!

That something, is contacting clinic staff. Due to the increase in the number of people participating in the clinic, we now have a specific e-mail address dedicated to the Wolfram Research Clinic.

wolframresearchclinic@wustl.edu



Please use this e-mail address to submit any questions that you may have regarding clinic details, on-line surveys, virtual visits, clinic visits, medical records, and requests for information, or anything else we may be able to help with. Each inquiry will be directed to the best person to answer your question. Please do not hesitate to contact the clinic team. Chances are that if you have a question, someone else is wondering the same thing. Responses to frequently asked questions will be shared anonymously with the group. This account will be monitored daily.