



WU Wolfram
Research Clinic

Washington University School of Medicine

Wolfram Research Clinic Newsletter

February, 2019 Vol.14

WU 2019 WOLFRAM RESEARCH CLINIC

We're doing things a little differently this year in lots of ways and getting your input is no exception. Along with other changes, this year we will be sending out a 2019 Pre-Clinic Survey. This survey is intended to inform clinic planners (WFS Clinic and the Snow Foundation) on topics such as attendance, choice of clinic, your interest level in the possible weekend activities, and to provide the team with updates on the patients' health status. There will be an opportunity to upload updated medical records as well as provide a description of the changes. Depending on your answers, the questionnaire should take approximately five to 25 minutes to complete. The questionnaire is comprised of six brief sections. A couple sections only have two or three questions each.



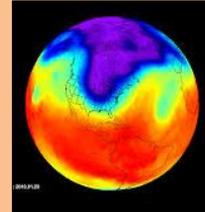
The **first section** consists of the consent form for this questionnaire only. This is not the clinic consent form. By clicking "Yes" at the end of the consent form you will be giving consent to continue with the survey. If you choose "No", simply "Submit" the survey and the survey will end.

The **second section** simply asks if you are planning on attending the clinic and if so, which clinic you prefer. For data collection purposes, even if you've already confirmed your clinic choice, go ahead and indicate it again on the survey.

The **third section** is asking who will be coming with you as well as a chance to provide contact information. This will then be compared to our records and updated with any new information.

RESEARCH UPDATE

Dear Wolfram Community,



Greetings from chilly St. Louis! We hope you are all surviving the Polar Vortex that is affecting most of the US right now and are looking forward to complaining about the heat and humidity during our research clinic in July! In between snowstorms and preparations for the clinic, we have working to analyze data we collected over previous years. One new effort takes advantage of the world-class expertise that Washington University has in developing biomarkers of neurodegeneration.

We have started a collaboration with Dr. Anne Fagan, an expert on Alzheimer's disease and other neurodegenerative disorders, who perfects blood tests for tracking brain degeneration. We have arranged to have the blood samples collected over the past 8 years in our research clinic to be analyzed by Dr. Fagan's lab. She will measure a new marker for brain cell degeneration that has showed great sensitivity to other common neurodegenerative conditions. We hope to determine if patients with Wolfram Syndrome have elevated levels overall and whether these levels increase as symptoms and brain imaging measures get worse. Depending on the results of these analyses, we may want to expand our blood samples during the clinic to include family members as a comparison group. If we had such a marker, it would be very helpful in future clinical trials and in understanding the rate and mechanisms of change in symptoms.

Sincerely,

Tamara Hershey, PhD

Professor

Scientific Director and Principal Investigator

WU Wolfram Research Clinic

tammy@wustl.edu; 314-362-5593

CLINICAL CARE UPDATE

Dear Wolfram families,

We are so excited to see that Dr. Barrett's clinical trial has finally started! In his recent announcement he describes the rigorous process that the trial has to follow in order to provide reliable and trustworthy results. We applaud his team's efforts and look forward to hearing about their progress. As a side note, Dr. Barrett is sharing the neuroimaging data from his trial with us so that we can understand more about the individual variability in brain changes in Wolfram Syndrome.

See Dr. Barrett's January 27th post on the "Wolfram Syndrome UK (DIDMOAD)" Facebook page and this website (<https://clinicaltrials.gov/ct2/show/NCT03717909>) for the details of the trial.

All the best to everyone,

Bess Marshall, MD (Marshall@kids.wustl.edu)

Pediatric Endocrinologist

Medical Director, WU Wolfram Syndrome Research Clinic



If you know of anyone who would like to be added to the Wolfram Research Clinic Newsletter please ask them to contact Samantha Ranck, Study Coordinator. In addition to adding people to the newsletter, we can also share previous volumes in case you missed something.

NEED HELP?

For questions or requests regarding the Pre-Clinic Planning survey or the Wolfram Syndrome Research Clinic please contact the **WFS Research Clinic Coordinator, Samantha Ranck, MSW** at 314.362.6514 or rancks@npg.wustl.edu

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cont...

The **fourth section** asks questions about what sort of things you may be interested in doing on the weekend that falls between the two clinics. This would be Sat., 7/13 and Sun., 7/14. There are some different activities being considered and assessing participants' interest in these activities will help to guide clinic planners in the right direction. Please note that these activities are tentative and are partially dependent on the interest of the families. By surveying the families now, we will have more time to adjust any of the events based on the responses we receive.

The **fifth section** provides you with an opportunity to upload any updated medical records. There are also a few questions that ask about changes in the patients' health in six different areas. We are asking about changes since the last time they attended the clinic. Please provide as much detail as possible. This information will then be communicated with the appropriate physicians and testers at the clinic so that they may review the information and better prepare for the patients' visits.

The **sixth and final section** asks about any other Wolfram Syndrome studies, clinics or trials in which the patient may have participated. Please provide as much detail as possible in this section as well.

Since planning is in full swing and we will soon need to make some decisions, **it is important to complete your survey and return it no later than Saturday, 2/16/19.**

If you agree to complete the survey but will not be attending the clinic, you'll be able to note this in question 3 and the survey will end. All you need to do at this point is "Submit" the survey and that provides us with much needed information as well.

