

TREATWOLFRAM CLINICAL TRIAL UPDATE FOR WOLFRAM SYNDROME UK

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Dear friends and colleagues,

The last year has obviously been very difficult for everyone, but I am pleased to say that the TreatWolfram clinical trial is continuing to recruit and follow up participants. The study is now open in 6 sites (Birmingham Women's and Children's (Dr Renuka Dias), University Hospitals Birmingham (Dr Ben Wright), Almeria (Dr Gema Esteban Bueno), Paris (Prof Christophe Orssaud), Montpellier (Prof Agathe Roubertie), and Lodz (Prof Wojciech Mlynarski). Recruitment has now reached 28 children and adults; and the first recruits have now been taking part for 2 years. We are incredibly grateful to all participants for their time and commitment to the study, and helping us answer the question: does this medicine slow down progression of the condition?

As you know, neither the participants nor the doctors delivering the study, know who is taking placebo or who is taking the treatment. This is essential, so that we can tell if the treatment works, without any bias. However, the study is set up so that two thirds of participants are taking the treatment and only one third the placebo. This is because we want as many people as possible in the study to benefit from the treatment, if it works. When we get to the end of the study, we are allowed to find out who has been taking the treatment and who the placebo. If the treatment is shown to have slowed progression of Wolfram, then we will apply for a licence to prescribe it to anyone with Wolfram who would like the treatment.

The patient support group Wolfram syndrome UK has generously donated funds to support travel and accommodation to each participant and carer visiting Birmingham to take part in the study. This has been really helpful for families travelling from some distance. Each of our hospitals has taken stringent measures to keep participants protected from any risk of Covid infection- these include only seeing study participants in covid-free areas; weekly testing of staff; and minimising participant travel around the hospital site. Our Clinical Trials Unit has also helped by changing some face to face visits to remote, video study visits.

We have a meeting of our Data Monitoring Committee coming up. This is a group of independent experts who meet every 6 months. They are allowed to see all the data and know who is taking the treatment and who the placebo. This is for safety reasons, to ensure that no-one is coming to any harm through participation in the trial. I am pleased to say that the Committee have had no safety concerns so far.

I, and the Data Monitoring Committee, are keen to find out how many more people with Wolfram in the UK would like to take part. As you know, the key requirement is to have enough vision so that we can measure it over the course of the study. If you know what size font you can read, this gives a rough indication. For instance, if you can read font size 48 or smaller, your vision is most likely good enough to take part in the study. Another is if you can read a car number plate from a distance of 1.25 metres (about 4 foot). If you can read either of those, and would like to take part in the study, please do let myself or Tracy know.

Recruitment to the TREATWOLFRAM trial will end in June 2021; so if you would like to take part, please do let Tracy know as soon as possible.

I hope to write again with a further update around Easter time. Meanwhile, thank you from all of the study team for your interest, and we are all really hoping for a positive outcome from this clinical trial!

With best wishes