

Tim Barrett update for TreatWolfram newsletter

Dear friends and colleagues,

I hope everyone is keeping OK and managed to cope with the 2 heatwaves this Summer. Our hospital research teams have kept busy seeing people taking part in the TREATWOLFRAM clinical trial, and we have had meetings of the Trial Steering Committee, and the Trial Management Group. The Trial Steering Committee, chaired by Professor Marc Peschanski, commended all the study teams and participants for keeping the trial on track despite the pressures the National Health Service and international health services are under. They were really pleased that most participants are having study investigations on time, and information being sent to the Clinical Trials Unit. They are pleased with how the trial is being delivered, and have no safety concerns. In July, the Trial Management Group brought together our international colleagues from Spain, Poland and France, together with our UK sites in Birmingham. We were able to share our experiences of delivering the clinical trial.

Firstly, all the researchers are incredibly grateful to those of you who are giving up your time to take part in this study. We know this trial is onerous, and some people have had to miss time off school or work and travel some distance to take part. The information you are helping us collect is really valuable and will allow us to have a definite answer to the question: does sodium valproate slow down the progression in Wolfram syndrome?

Secondly, I am really pleased to say that we are well over half way through this trial. This started back in 2016 when our team was awarded funds from the UK Medical Research Council to conduct the study. The first participant was recruited in 2019, and the last visit for the last participant will be in October 2024. Some participants have already finished taking part. This is a long study, and thank you for your patience while we complete all the regulatory requirements. We should have a result fairly soon after the trial ends, and if we have shown that sodium valproate works, it will be made available for all people who would like it.

Finally, I am pleased to say that the story does not end there. We are using the knowledge we have gained about running an international trial, to apply for a European research grant to conduct new studies of treatments for Wolfram. The knowledge from the TREATWOLFRAM trial is highly influential in design of the next study. We have a grant being submitted in September, which if funded, will allow us to start a trial of other medicines, from Autumn 2024. The help you have given us is invaluable to getting future studies off the ground.

All our study team owes a big thank you to Wolfram syndrome UK for their generous support towards travel and accommodation costs for UK participants. I am really looking forward to meeting some of you at the upcoming Wolfram syndrome UK conferences

Sincerely