

Tim Barrett update for TreatWolfram newsletter

Dear friends and colleagues and participants in the TREATWOLFRAM clinical trial,

I hope everyone is keeping OK. As some of you may know, we had to change supplier of the medicine and placebo, and this caused an interruption in supply for some participants. I am relieved to say that participating centres were resupplied with medicines in December, and everyone who is still being followed up in the trial should have restarted their medicine.

I was so pleased to see some young people face to face in clinic and for study visits in January, and hear about peoples' experiences. While we don't know who is on which treatment, I am gratified to know that people are willing to continue, and some feel they are benefitting from taking part. All of us as researchers and health care professionals are incredibly grateful to those of you who are giving up your time to take part in this study. 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?

Our Data Monitoring Committee (DMC) met in February. This is an independent committee, chaired by Professor Karen Morrison. The committee is independent of the clinical trial team, and includes experts in child health, adult medicine, and clinical trials. They look carefully at all the data so far. The DMC are allowed to know who is taking sodium valproate (study medicine) and who is taking the placebo (dummy medicine). They also know the data on all the things being measured in the study, including the vision of participants. Finally, they look carefully at all the information on any safety concerns. The DMC looked at all the results so far, and are happy for the study to continue. This means that there is not enough information yet to know if the medicine is working; or if the medicine is not working. This decision is in line with what we expect for this stage of a clinical trial. In addition, the committee did not have any safety concerns.

This clinical trial is planned to complete in Autumn 2024 and we will have a definite answer for you after that.

Finally, as researchers, we are looking forward to the international conference in April, organised by WSUK. This will be an opportunity to discuss the clinical trial progress so far with our international colleagues, and to hear about other research into Wolfram going on around the globe.

All our study team owes a big thank you to Wolfram syndrome UK as always for their generous support.

Sincerely