

Tim Barrett update for September TreatWolfram newsletter

Dear friends and colleagues,

I hope everyone is keeping OK and managed to enjoy at least some good weather this Summer.

We are getting to the end of the TREATWOLFRAM trial, and the last participants will be having their last visits this October. We then hope to collect all outstanding data from study sites by the end of the year. The Clinical Trials Unit will then 'lock' the database so that our statistician can do the analysis. We expect to be told whether the treatment is effective or not, by the end of March or April 2025. As soon as I am told the results, I will write a report to our funder, the UK Medical Research Council; and I will of course share results through Wolfram Syndrome UK. Our trials unit team, Amy Lamb, Lewis James, Victoria Homer and Darren Barton, are working hard to make sure all the information that participants have kindly provided, will be ready to be analysed when the trial ends in October 2024.

Going forwards to the end of the clinical trial: if the trial is successful and sodium valproate is shown to be effective in slowing progression in Wolfram syndrome, then we will make it available to everyone with Wolfram who wants the medicine. We will, however, strictly follow the national patient safety alert advice so that it is not given to females while they are trying to conceive or while they are pregnant.

All our study team owes a big thank you as always to Wolfram syndrome UK for their generous support, and for bringing the Wolfram community of families, researchers and health care professionals together.

Sincerely