

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

I hope everyone is keeping OK. I don't have much to update this newsletter, except to say that the trial is still in follow-up phase, and I am really grateful to all people who are taking part, and their families. We are in the final 18 months of the trial, so please keep going with study visits and completing your study medicine diaries!

Our Trial Steering Committee (TSC) met in earlier this week. This is an independent committee, chaired by Professor Marc Peschanski. The committee experts in child health, adult medicine, and clinical trials. They were happy with how the trial is progressing, and will meet again in the Autumn, after the independent Data Monitoring Committee (DMC) has met. It is hoped the DMC will be able to do an interim analysis of the results. They will not make these results public unless the data shows beyond doubt that the treatment either works or does not work. 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 very grateful to Tracy Lynch and Stephanie Snow Gebel for organising a fantastic international Wolfram researcher conference in April. I was able to present the background to the trial and how it is progressing so far. We had helpful feedback, and look forward hopefully to presenting another update at the next conference.

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

Sincerely