

Dear friends and colleagues,

I am really pleased to say that we reached the recruitment target for the TREATWOLFRAM clinical trial on 1st Nov. 75 children, young people and adults kindly consented to take part in the trial. Some people were sadly not able to take part for medical, logistical or reasons. We closed recruitment to the study with 63 participants.

These include 14 children recruited at Birmingham Women's and Children's Hospital, 11 adults at Queen Elizabeth Hospital Birmingham, 5 at Montpellier, 10 at Paris, 5 at Lodz, and 18 at Almeria.

This is fantastic support from the whole European community, and especially Wolfram Syndrome UK, who are generously supporting UK participants to travel to Birmingham.

This is the first international trial of a treatment for Wolfram, and the largest. Most importantly, we have the right number of participants to give a definite answer to the question: does sodium valproate treatment slow disease progression in Wolfram?

I know I can say on behalf of all the clinical trial team that we really appreciate the time and effort that people are making to take part in this study. This is a lengthy process and can be frustrating. However, by having a gold standard clinical trial, we will have really strong evidence to support the adoption of the treatment into the clinic.

We have an independent Data Monitoring Committee that meet every 6 months. They look at all the data and know who is taking the treatment and who is taking the placebo. If that group see either a really strong signal that the medicine has stopped the disease process, or that it does not work, they can stop the trial immediately.

Realistically I expect the trial to run to Autumn 2024, and for us to have results to share then.

If anybody would like to ask more questions about the study, or their participation, please don't hesitate to get in contact with me, Dr Renuka Dias, Dr Ben Wright, directly or via Tracy

I wish everybody a peaceful and safe Christmas

sincerely

Timothy Barrett [he/him]

Consultant paediatric endocrinology and diabetes