

Dear friends and colleagues,

I am pleased to say the TreatWolfram clinical trial is progressing well. Recruitment ended on 1st Nov 2021, and 63 participants are under follow-up. The first young people who were recruited at Birmingham Children's Hospital, have now completed the trial and had their last visits. We have a new trial coordinator, Ruth Evans, who has taken over from Amandip Malhi and is doing a great job. Our clinical trials team, under Darren Barton's leadership, is brilliant and working hard to ensure that each study site has all resources needed to support people taking part.

We are well over half way through the trial now, thanks to the brilliant efforts of people taking part, and the clinical trial staff. We have another Data Monitoring Committee meeting in the Autumn; this committee, chaired by Professor Karen Morrison, will see all the 'unblinded' data, and hopefully confirm that the trial should continue.

Once we get to the end of the trial, our statisticians and the Data Monitoring Committee will let us know if there is clear evidence that the treatment slows progression in Wolfram.

Thank you to all the participants for their work in taking part, and our clinical trials team around Europe. I will post another update in the Autumn

Best wishes

Tim

Update from Dr Ben Wright

People with Wolfram continue to be reviewed as per the trial protocol at the Queen Elizabeth Hospital. Precautions continue to be undertaken as per hospital COVID guidelines. There have

been some changes to the research nurse team and are now joined by Ewa Delmaczynska who will be working together with Sarah Harris. We continue to be very grateful for the time trial participants and their supporters have offered to help complete this trial.