

## UPDATE ON TREATWOLFRAM CLINICAL TRIAL

September 2020

Dear friends and colleagues, to recap, this is a trial of sodium valproate given by mouth, for 3 years, to try and slow down the progress of vision loss in Wolfram. It involves 4 countries in Europe, and is comparing the effects of sodium valproate to placebo. The trial recruited its first patient in January 2019. We currently have 12 recruits at our children's hospital site in Birmingham, looked after by my colleague Dr Renuka Dias. We had a pause due to Coronavirus, but I am very pleased to say that Dr Ben Wright has just recruited our first adult patient at the adult site in Birmingham. With lots of help from our international colleagues, we now have all the regulatory approvals in place in each country. We spoke with Dr Gema Esteban in Spain this week, and she will recruit her first patient in October. We hope to complete recruitment in June 2021.

We now have really accurate and reproducible assessments in each site so that we can pool the results. Our independent data monitoring committee sees all the unmasked results and if there is a strong signal of effect, or signal of no effect, they will let us know without waiting for the end of the trial.

I am very grateful to everyone who is taking part or about to take part in this trial; and we are learning a lot about how to deliver clinical trials in Wolfram, for the future. Wolfram Syndrome UK have been brilliant in supporting families to attend study visits; and we are very grateful to The Snow Foundation, Eye Hope Foundation, and Association Syndrome de Wolfram for support to collect research samples, which will be available to the whole Wolfram syndrome community.

sincerely

Prof Timothy Barrett