

## **TREATWOLFRAM clinical trial update for families**

**Tim Barrett January 2019**

Dear friends,

I am very pleased to say that we have now started the clinical trial and the first young people have been recruited.

As you know, this clinical trial is a test, under stringent scientific conditions, to find out if our proposed medicine can make a difference in people with Wolfram syndrome, by slowing down the progression of the condition. We have chosen vision as the primary outcome, but are also measuring the effects of the medicine on diabetes, general wellbeing, bladder function, mood, and gait and balance.

This clinical trial is carefully regulated by the European Medicines Agency, the NHS Health Research Authority, an NHS ethics committee, and by the hospitals from which we are recruiting patients. We have to do this, to keep people safe, and to give a clear yes or no answer – does it work or not? This is the first clinical trial in Wolfram syndrome, that is collecting all the information needed to satisfy regulators' requirements.

To give a clear answer, we need to recruit 70 children, young people and adults, and follow them up for 3 years to look at the progress of the condition. Two thirds will receive the study medicine and one third the placebo (dummy medicine). This study is called 'double masked' because we won't know which participants are on which medicine, and the participants will not know either.

To help get a clear answer, we have to use strict conditions on who can take part and who cannot. This will be disappointing for some, but I really hope people will understand, that the regulatory agencies will only let us offer this medicine to everyone, if we can provide cast iron evidence that it works. People who are not taking part in the trial will be able to have the medicine if we can prove it works.

At our Wolfram multidisciplinary clinic on 7<sup>th</sup>/8<sup>th</sup> January we were able to recruit the first young people to the trial. They have been randomised to treatment or placebo, and are visiting our hospital again for safety checks this week.

We discovered that it took an enormous amount of work by the clinical and study teams, support services, and clinical trials unit, to get this study open and recruiting. I am incredibly grateful to everyone who worked so hard to allow us to start; and really went the extra mile. I am also so grateful to the young people and their families who consented to participate.

We hope to open the Queen Elizabeth Hospital Birmingham by March; then our international sites. If anyone would like more information about the study, then please do let me know via Wolfram Syndrome UK and I can arrange to post out information sheets.