

Dear friends and colleagues,

Tracy asked me to write a brief update on our progress towards a clinical trial for Wolfram syndrome.

As you know, we were delighted to receive an award from the UK Medical Research Council to conduct the first efficacy trial of a treatment for Wolfram syndrome. This funding allows us to conduct a clinical trial in 4 countries (UK, France, Spain, and Poland) following all the international regulatory and ethical standards.

The treatment we are trying are tablets that you swallow whole, or crushed with food. We want to see if this treatment slows down the progression of the disease.

We started work on the trial in January, and activated the grant on 1st March. So far we have:

1. Written the ethics application
2. Written the patient information sheets- thank you so much to those of you who have helped with this
3. Written a new draft of the clinical trial protocol
4. Identified each of the trial sites that will recruit patients to the trial. For the UK, this will be Birmingham
5. Found an NHS Pharmacy (Guy's and Thomas's Hospital) who will make the placebo tablet and package the treatment and placebo tablets.

We have come up against an issue with the tablets: the medicine we want to use comes in foil packets (blister packs), and you have to press the blister to break open the foil and get the tablet. For the clinical trial, we have to take the tablets out of their foil packages, and put them in air tight plastic containers, identical to the containers for the placebo tablets.

The tablets that we want to use are hygroscopic – that is, they absorb moisture from the air once they are out of their foil packets. This means that they may release the active medicine in a different way, to tablets that are kept in their foil packets.

The regulatory agency wants us to test the stability of the medicine when taken out of its foil packet and placed in a different container. This will take 4 months, as we will want to dispense the medicine to people every 3 months.

This all sounds rather technical, but is essential so that we know the medicine we are using, works.

We hope to have this completed over the Summer, so that we can recruit the first patients to the clinical trial in November.

I will write a monthly blog to keep you up to date with progress

Thank you for your patience and please be assured we are working hard to open this clinical trial later this year

With best wishes to you all

Prof Tim Barrett