

## **UPDATE ON TREATWOLFRAM CLINICAL TRIAL FOR THE WOLFRAM SYNDROME UK NEWSLETTER**

**Tim Barrett June 1<sup>st</sup> 2021**

I am really pleased to say that the TREATWOLFRAM clinical trial has now reached 48 participants! We are grateful for the fantastic support of families, and all the hard work of our international teams, led by Renuka Dias and Ben Wright in Birmingham, Gema Esteban in Spain, Agathe Roubertie and Christophe Orssaud in France, and Wojciech Mlynarski in Poland. Gema in particular has worked hard to recruit 13 participants, despite having had a Covid infection with after-effects. We need to reach 64 participants, but have to close recruitment this Summer, so do please spread the word and ask anyone interested in taking part, to get in touch.

Our independent Data Monitoring Committee met in March, and reviewed all the data collected to the end of February. They are allowed to see which participants are taking the treatment and which the placebo. They also see the outcome measures such as vision. It is too early for them to tell whether the treatment is working; however, they were able to tell us that they have no safety concerns so far.

Our Trial Steering Committee met in April, to review our progress. This is chaired by Professor Marc Peschanski, Director of the I-Stem Institute in France. We discussed the impact of the Covid-19 pandemic on recruitment. Covid has slowed recruitment; however, all sites are open again and recruiting. We know that thankfully, people with Wolfram seem to be able to fight Covid infections as well as anyone else. We have also amended the trial slightly to allow some of the safety visits to be done locally, and our funder, the UK Medical Research Council (MRC) has helped with extra funds to address some of the effects of the pandemic.

The trial has also been affected by BREXIT. This has created extra 'red tape' and costs of shipping the treatment to international sites. Our brilliant Clinical Trials Team, led by Darren Barton and Sabrina Cronier, have worked with the MRC to get these issues sorted out. I am really pleased to say that the MRC has been understanding, and supported the trial with additional funds to ensure that it succeeds.

We have had a change of staff at the Clinical Trials Unit: our clinical trials coordinator Dr Pooja Takhar sadly left in May as she is moving on to a new career in a completely different field. She has worked hard on checking all participants' data is complete, and preparing our committee reports and ethics amendments. Amandeep Malhi has taken her place. She is an experienced clinical triallist who has worked on other studies within the Unit, and I am sure will want to meet families at a future webinar.

One of the many amazing things about this trial is that our imaging expert, Dr Heather Rose, has managed to cross-calibrate MRI scanners from 4 countries, and collect readable brain images from every participating centre. This has not been done before for a rare disease, and will tell us if the treatment is slowing the loss of brain cells.

Finally I would just like to add my massive thanks to all the TreatWolfram group, including the National Coordinating Centres, and each site's clinical and non-clinical staff; a truly multidisciplinary team effort to get this international trial to patients. I'd also like to thank of course Wolfram Syndrome UK; without whose generosity we would not be able to support participants at their study visits. I hope by the next newsletter to be able to tell you that recruitment is complete, and share some anonymous information on spread of ages and backgrounds of the participants.

With best wishes for a safe and healthy Summer