

UPDATE ON CLINICAL TRIAL FOR FAMILIES

March 10th 2020

Dear friends and colleagues,

I am writing to give you an update on where the TREATWOLFRAM clinical trial has reached. So Birmingham Women's and Children's Hospital has been open to recruitment of children since January 2019. I am delighted to say that University Hospitals Birmingham opened at the end of January 2020. We think that Lodz, Poland, will be the first international site to open, later this month. So far, the parents of 14 children have consented for their child to take part, and 12 have been recruited and are being followed up. The first adult patient has also been recruited.

In terms of outcomes, our nurses and clinical trials team are monitoring participants carefully to do safety checks. As you know, neither us nor the participants know who is taking the active medicine or the placebo; but there are about twice as many participants on the active medicine as on the placebo. There is a Data Monitoring Committee (DMC), that will meet in the summer. This is made up of independent doctors, scientists and statisticians. This committee will look at the data and are allowed to know who is taking the active medicine and who is taking the placebo. If there is any signal that the medicine is not safe, they will tell us. Similarly, they will see if the medicine is being effective at slowing the disease process. However, it is highly unlikely that they will be able to comment on effectiveness yet, until there are more participants.

There have been several difficulties with delivering this trial, including ensuring the supply of medicine, making sure all study procedures are completed at the hospital site, that all safety checks are completed, and of course implications of BREXIT. The Clinical Trials Unit are doing a fantastic job in sorting these out, and have worked flat out to keep on top of the research governance paperwork. We have also worked with the sponsor (University of Birmingham) and funder (UK Medical Research Council) to help solve problems as they arise. Dr Renuka Dias at the Children's Hospital and Dr Ben Wright at University Hospitals Birmingham, are being fantastic and looking after the participants as they attend for study visits.

Going forward, we would like to have 70 recruits internationally by the end of the year. We have plans for recruiting children and adults between now and September, and a list of people waiting to be recruited. Clearly there are new uncertainties, with the Coronavirus outbreak. However, our research governance teams are making plans to support families who are taking part in research studies, and I will send more information round as I get it. It is important to say that the active medicine in this clinical trial, does not affect the immune system, so people taking part in the trial are no more or less likely to catch Coronavirus than anyone else.

Finally I would like to thank the fantastic families and participants who are bearing with us and the study visits- without you, we would never find a treatment!