

UPDATE ON TREATWOLFRAM CLINICAL TRIAL – November 2019

We held a Data monitoring committee (DMC) meeting in the summer, and a trial steering committee (TSC) meeting earlier this month. The DMC is chaired by Professor Karen Morrison, an adult neurology professor who used to lead the adult Wolfram service in Birmingham. The DMC reviewed the data collection so far, and made suggestions to improve the recording of visual acuity, the main endpoint of the clinical trial. The TSC is chaired by Professor Marc Peschanski, Director of iSTEM, a large stem cell facility outside Paris, which specialises in neurodegenerative disorders. The TSC reviewed the clinical trial so far and made some very helpful suggestions to help the study going forward.

In response to trial monitors and participant comments, we are changing the trial medication diaries to make them easier to complete. We have to get further ethics approval for this, and we expect to get this during December.

Our UK adult site is not yet open, as we have had to put a new contract in place for blood pregnancy testing. However, we really hope we can resolve this in the next couple of weeks.

Our international sites are progressing well now that contracts have been signed.

We are in discussion with our funders regarding putting plans in place in the event of a 'no deal' Brexit, and we hope this will reach a resolution soon.

Finally, a massive thankyou to everyone who has taken part so far, and I and the rest of the team look forward to seeing you at upcoming visits to Birmingham.

With best wishes for a peaceful Christmas

Tim