

Update November 2018-

I wanted to update you about the status of the clinical trial since the Wolfram family conference in October.

The medicine and placebo.

So Guy's and St Thomas's Pharmacy Manufacturing Unit (GSTT) have sourced a company that will make the medicine; and have been putting a supply contract in place. Meanwhile the clinical trial sponsor, University of Birmingham, have been negotiating their contract with GSTT to pay for the work. This is due to be signed off this week; after which GSTT will make the placebo tablets. This takes 2 weeks. The plan is for GSTT to do the final quality checks, and get the medicine and placebo to the clinical trials pharmacy department at Birmingham Women's and Children's Hospital (BWCH) the week beginning Monday 18 Dec.

Clinical trial site: BWCH (Birmingham Women's and Children's Hospital).

The children's hospital site will be the first to open. The costs for running the study are being finalised; there has been a lot of negotiation. I am really pleased to say that BWCH had its site initiation visit today, with training in the study being provided to the local study team. All being well, the site will be open to recruit participants from w/b Monday 18 Dec. We will start by posting out participant and parent information sheets to all children and young adults under care of BWCH. I had hoped that we could post out patient information sheets soon after the family conference. Unfortunately, the rules around clinical trial governance mean that I am not allowed to post out these sheets until a site has been officially opened. I am sorry about this. Potential participants will have consent taken, study procedures undertaken, and medicine or placebo dispensed, from the first Wolfram MDT clinic in January. We hope to recruit 25 children and young adults to the BWCH site during 2019.

Clinical trial site: University Hospitals Birmingham.

This site will be the second to open; most likely during January or February 2019. The costs for running the study are being negotiated.

We are also discussing how to offer MRI scans and eye tests to adult participants.

International clinical sites:

We expect Poland to be the first site to open, hopefully in February, followed by France, then Spain.

I know the development of this clinical trial has been lengthy, and frustrating for many of you. For that I can only apologise. In mitigation, I am learning that clinical trials are governed by strict rules designed to keep participants' safety the top priority. We are getting really close now, please bear with us, and thank you to those of you who have sent in comments and suggestions.

We will do our best to offer participation to all patients in the first half of 2019.

Sincerely

Tim Barrett

On Behalf of the clinical trials team