Trial Update – February 2018.

Rebecca Storey and Shazia Ahmed, our senior and junior trials coordinators respectively, have been busy writing all the regulatory documents needed for the trial to go ahead. These were submitted to the Health Regulatory Authority in early January. The ethics committee met in early February. This was in Glasgow, but we were allowed to join by telephone conference. The committee was very sympathetic and had only minor requests. Rebecca and I are writing the response, which has to be back with them by Wed. We cannot get complete regulatory sign-off until we have evidence that the study medicine is stable outside it's manufacturer's packaging.

Regarding the study medicine, we have been in weekly contact with the pharmacy manufacturing unit at Guy's and Thomas's Hospital in London. They have outsourced the testing of the medicine to a commercial contract company, Butterworth's. So the problem is that the study medicine comes in foil blister packs. The manufacturer only guarantees the quality of the tablets while they are in their own packaging. For the clinical trial, Guy's and Thomas's Pharmacy have to take the tablets out of the manufacturer's packaging and put them into white polypropylene containers, so that they can be masked (blinded) with the placebo tablets. Unfortunately, these study tablets absorb moisture from the atmosphere when taken out of their foil packaging, which in theory may affect the stability of the active component. Guy's and Thomas's hospital have been trying to test some tablets that they have kept in typical study bottles; but without success. They then outsourced this job to Butterworth's, who found a non-standard method to measure drug stability. However, when they tried to convert this to an approved method, they could not get the measurements to work. This is turning into a major headache, and is the reason the study has been held up. We are having a telephone conference on Monday with the pharmacy team to get an update and plan what we do next.

We have appointed a new study team member, Heather Rose, who will look after the MRI scans for the trial, and do the measurements to see if the study drug is slowing down the brain changes. She is getting the scanners set up in Birmingham for both children and adults to take part.

Rebecca has been in touch with the international sites, in Almeria, Montpellier, Paris and Lodz, to start getting them set up.

Finally I have been asked to speak with the French families in Paris on March 17th to explain to them where the trial is at.