

Dear Families,

Last year the Dubowitz neuromuscular research team at GOSH sent you notification that in order to improve transparency and equitability, the way we are recruiting to clinical trials had slightly changed.

In particular we notified that we had implemented a randomisation system to identify potential eligible patients. I attach the previous notification for your information.

Since then we have continued to have internal discussion on the procedure, we use to recruit children in clinical trials, because the treatment landscape is continuously evolving and we want to try and be as fair as possible.

Back in January 2020 we contacted our ethic board and they agreed that the below was an appropriate adjustment to make the recruiting process.

The slight change that we have made is that if a clinical trial comes to an end due to the study being stopped by the sponsor or governing bodies due to not meeting safety profiles or end points, and should a study with similar inclusion criteria be ready to recruit, we will give these families the opportunity to take part in one new study. The remaining families in the database will be contacted subsequently. The GOSH ethics board agreed that this principle of providing prioritisation of families who fall in this category is appropriate. This does not include the situation when families decide to withdraw from a study or for families who are not compliant with the study protocol and have to be excluded by the sponsor or the principal investigator from its continuation.

The paragraph above describes the general principles that will guide the selection of families for clinical trials in our centre; obviously other aspects dictated by the protocol and the sponsor, including age, functional status and general health of the children, will continue to apply and will be evaluated by the principal investigator of the study before contacting families.

We also wish to clarify how we will notify families of the selection for clinical trials. As we have more than 300 families in our database, and we open several studies every year, each recruiting a relative small number of participants from the entire country (i.e. not only those families attending GOSH), it is not feasible to contact each family for each trial once the recruitment process has finished, and their child was not selected to take part.

We provide regular updates to the DMD community of our ongoing studies (at GOSH by regular leaflets indicating ongoing studies and those open for recruitment; and nationally via the North Star network and, if approved by ethics, also via advocacy groups and the DMD HUB). This information is also regularly posted in the Dubowitz Neuromuscular Centre website (XXXXX).

Once we will have completed the recruitment, we will provide notification via our DNC website, and via the DMD-HUB, so we ask families to regularly check these websites for updates.

I hope this is clear and please let us know if you have any further questions.

Please bear in mind we are currently working on skeleton staff and if you reply to this email there may be a delay to our response.

Kind Regards  
Neuromuscular Research Team