

FOR-DMD – FOR YOU!



Newsletter 2 –8th April 2013

A Message from the Chief Investigators

The NIH are keeping an eye on the performance of all sites; as the first boy was randomised in January 2013, the clock is now ticking on our two-year recruitment period. Likewise, for UK sites, since the study is on the NIHR portfolio, recruitment to time and target will be closely monitored. In the next newsletter, we hope to share with you recruitment targets for each country and over time. In the meantime, please check all available data sources such as your patient registry, clinical lists and databases in order to identify potentially eligible patients. Please update us on your screening and randomization plan in a prompt manner; this will allow us in turn to report to the NIH current status and prospective recruitment. We know that some boys have been waiting for this study to open, so we expect all sites to be able to screen at least one boy within 2 weeks from activation!

Thanks for being a key part of the FOR DMD team!

Katie Bushby and Berch Griggs, FOR-DMD Chief Investigators

Keeping in Touch

Don't forget to visit our web-site <http://for-dmd.org> and to let us know what you think of it; there's a link there for making contact with the study team.

Between 24th February and 3rd April 2013, we have a total of 429 page visits, with 98 unique visitors from 24 different countries. The most popular pages are /information-patients-families/ (78 hits); /information-clinicians/ (24 hits); /faqs/ (23 hits) and /newsletters/ (21 hits).



We are now on Twitter! Follow us @FOR_DMD



Study Approvals and Site Activation



In the US, we now have twelve sites open to recruitment. We welcome the following sites which have opened to recruitment since our last newsletter: Kansas University Medical Center/Children's Mercy Hospital (Dr Barohn and colleagues); SUNY Downstate Medical Center (Dr Anzika and colleagues); Boston Children's Hospital (Dr Kang and colleagues). The University of Utah and University of Minnesota have been added as two new sites; both are awaiting ethical approval & a signed sub-agreement and are expected to open very soon. Unfortunately, UT Southwestern has had to withdraw.



In Canada, we now have two sites open to recruitment. We welcome London Health Sciences Centre Children's Hospital (Dr Campbell and colleagues) which has opened since our last newsletter.



In the UK, we have three sites open to recruitment. Welcome to Birmingham Heartlands Hospital (Dr Roper and colleagues) which has just opened. We also expect Leeds (Dr Childs and colleagues) to open in the week beginning 8th April, followed by sites at Manchester and Glasgow within the next fortnight. Unfortunately, Evelina Children's Hospital has had to withdraw from the study; we are currently in the process of adding Oswestry as a replacement site. We expect that all UK sites will be open to recruitment by the end of April.



In Germany, BfArM approval was obtained on 27th March. Freiburg (Dr Kirschner and colleagues) will be activated in the week beginning 15th April. We expect that all German sites will be open to recruitment by the end of April. Dr Sabine Schneider-Fuchs (sabine.schneider-fuchs@uniklinik-freiburg.de) has taken over coordination of FOR-DMD in Germany.



Favourable ethical opinion has been received. We are currently finalising the site contracts and insurance arrangements. Padua is expected to be the first Italian site to be activated, with the remaining sites coming on board in May. Unfortunately, Rome is no longer able to participate.

Patient Identification and Recruitment

Across all the currently open sites, a total of 14 boys have been screened as of 8th April. There have been five screening failures (in all cases due to inability to produce replicable FVC readings; these boys will have a second FVC attempt or will be re-screened at a later date). Congratulations to Dr Mah and colleagues at Alberta Children's Hospital, our top screening site, with three boys screened to date. Six of the nine boys who have been successfully screened have been randomized, and it is expected that the remainder will be randomized in the coming week. We are also aware that a number of sites have identified further boys who await screening in the near future. **REMEMBER – once your site has been activated for recruitment, we expect the first screening to take place within two weeks of activation.**

UPDATES, HINTS AND FAQs

We are currently updating the MOO to include comments and suggestions from the sites which have already started screening and randomization. If anyone has further comments and suggestions or has identified any ambiguities or discrepancies, please let us know, by email to for.dmd@newcastle.ac.uk with MOO as the subject line.

SCREENING, RANDOMIZATION AND STUDY DRUG ORDERING

Screening and screening failures:

If a subject fails one of the screening procedures (e.g. he is unable to swallow tablets or to perform a reliable FVC) at the first screening visit, the subject can be provided with placebo tablets or an FVC tube to take home to practice. Another screening day can be arranged to check the boy's eligibility.

The Clinical Evaluator Manual (Section 6.1, page 14) provides details regarding how to record and evaluate FVC re-testing between screening days one and two.

If the subject is confirmed to be eligible at the second screening day, this will NOT represent a screening failure. However, if the subject fails one or more screening procedures even at screening day two and, in the opinion of the site PI, will not be able to comply with eligibility criteria in a reasonable time, the visit should be recorded as a screening failure. The subject will be therefore considered as not eligible for the study but can be re-screened at a later date (at the discretion of the PI) if this is considered appropriate by the site PI.

Please remember that re-screening will require parent(s)/guardian(s) to sign a new consent form (and the subject to sign a new assent form if required by local and country specific regulations). In the case of re-screening, the need to repeat screening procedures that might have been performed successfully at the first screening visit should be discussed on a case-by-case basis with the Chief Medical Coordinator, Dr Michela Guglieri (for.dmd@newcastle.ac.uk).

Drug ordering

Please remember to report any issue regarding study drug (e.g. delay in the shipment of study drug or of the unblinding envelope, missing or delayed receipt of the email to confirm study drug ordering) PROMPTLY to us:

William Martens, Tel: ++ 585-275-2483, Bill_Martens@urmc.rochester.edu;

Chris Speed, Tel + 44-191-222-7623, Mobile: +44-7807106293; chris.speed@newcastle.ac.uk).

INVESTIGATIONS

Please note: the eCRF73 (ECHO) has been updated recently. The new document includes all parameters which need to be recorded as already reported in the Manual of Operation (Section 6.20.2, page 76). Please ensure that you are using the correct document and that the most up-to-date version is shared with your Echo Department and Technician. The updated version is included in the screening visit PDF packet of CRFs in EXPeRT ('CRFs for screening visit.pdf' in the CRFs folder in the document sharing area).

Q: Should the head be included or excluded in the total body DEXA scan analysis?

A: As per clinical practice, the DEXA measurement is performed including the head but the analysis is performed excluding the head. With Hologic, in the DXA report there is a line "subtotal" which automatically excludes the head. Please contact us if you have any issue with DXA analysis (Dr Michela Guglieri, Tel: + 44-191-222-7623, Mobile: +44-7807111192, for.dmd@newcastle.ac.uk)

Q: Which assessments and advices should be done during the screening period?

A: Only Physio advice assessment needs to be provided at screening. The dietary and behavioural assessments and advices should be left blank on the screening visit CRF40. When entering the screening visit eCRF40 in EXPeRT indicate that the dietary assessment was not done (with a reason code of 'O' for other, and 'not required at SC' in the reason text field) and check the 'not done' box for each of the behavioural assessments. All three assessments and advices should be performed at all subsequent visits.

Q: At what temperature should blood and urine samples be stored?

Blood and urine samples for bone biomarkers should be stored at -80°C if possible. If the site does not have a -80°C freezer, samples can be stored at -20°C. Samples for biobanking **must** be stored at -80°C. Overall, we would prefer that all samples be stored at -80°C if possible.

PHARMACOVIGILANCE

Please note the FAX number for SAE reporting has been changed. The correct number is **+44 (0)191 580 0717**

TRAINING

Training material is available on Chillibean. Any site personnel who will be involved in the study but did not attend the IM will be required to complete the training on Chillibean. Please let us know (by email to for.dmd@newcastle.ac.uk with Chillibean as the subject line) if anyone at your site needs access to Chillibean

Clinical evaluators who have not attended an IM (or been trained at a site visit) **MUST** contact Michelle Eagle and/or Wendy King to arrange training (contact details in the Clinical Evaluator Manual). Clinical evaluators or Trial Coordinators must e-mail Michelle Eagle or Wendy King directly when they have uploaded a video to Chillibean, so that they can review the video and provide timely feedback.

CONTACTING THE STUDY TEAM

IF YOU HAVE ANY QUERIES, PLEASE DO NOT HESITATE TO CONTACT US!

Contact details are provided in the MOO (section 2) and an updated document (which includes mobile numbers) was sent via email on 15th February, along with the last newsletter. If you have mislaid your copy of this document, we will be pleased to send a replacement – just ask us via for.dmd@newcastle.ac.uk

Contacting the right person for specific issues as identified in the contact details document will guarantee a faster and more accurate response!

A full list of FAQs and responses may be found at <http://for-dmd.org/faqs/>

