

# FOR-DMD – FOR YOU!



Newsletter 6 –23<sup>rd</sup> July 2014

## SIGNIFICANT MILESTONE REACHED

On 4<sup>th</sup> June 2014, we reached a very significant milestone for FOR-DMD, with the randomisation of the 100<sup>th</sup> patient at our Chicago site. Huge congratulations to Nancy Kuntz, Colleen Blomgren and Lauren Webb for helping us to this major milestone.

Reaching this milestone has meant that NIH (NINDS) have released the remaining 50% of the Year 4 budget, which will be used to fund study drug manufacture and distribution, as well as payment to sites for subject visits.

## FOR-DMD DSMB MEETING NOVEMBER 2014

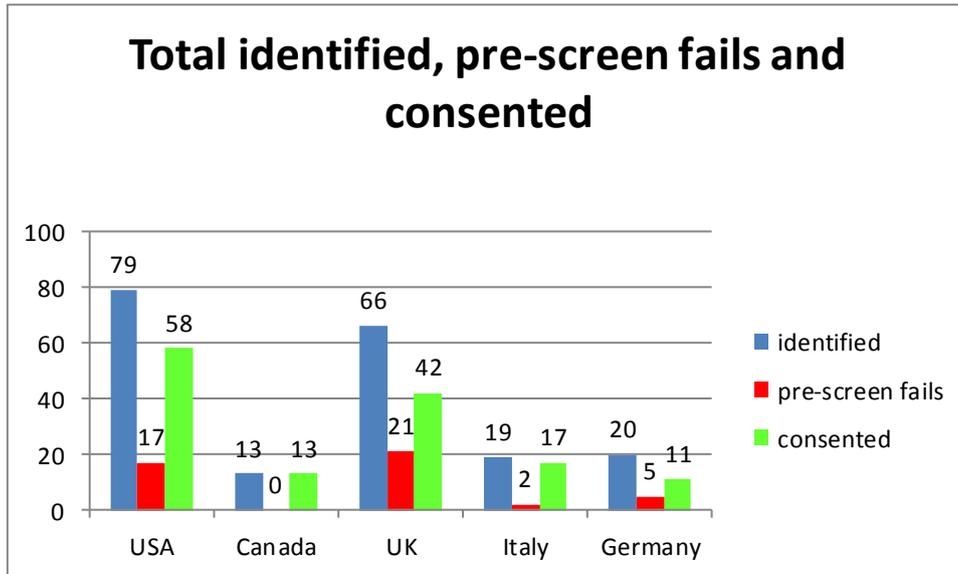
The next meeting of the Data Safety and Monitoring Board for FOR-DMD will take place on 3<sup>rd</sup> November 2014. There will be a major focus on recruitment against target, with progress informing decisions about continuing funding. We need every site to make its best efforts to recruit more patients over the coming months, so that we can demonstrate to the DSMB that reaching our target of 300 patients is a realistic prospect.

## RAISING A MUG TO SUCCESS

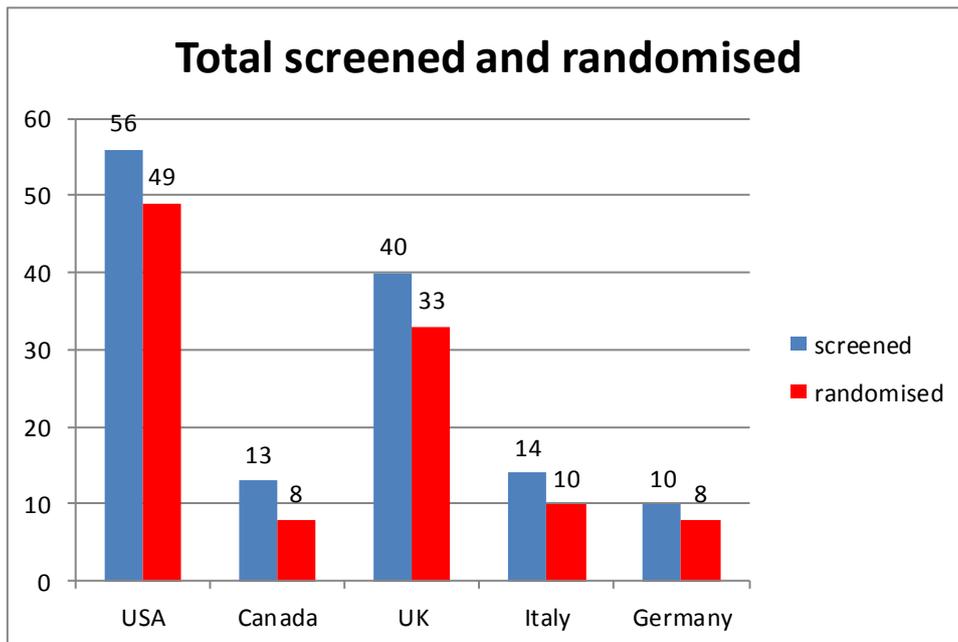


In our last newsletter, we announced the introduction of prizes of FOR-DMD coffee mugs for the sites that randomise every 10<sup>th</sup> patient (i.e. the 80<sup>th</sup>, 90<sup>th</sup>, 100<sup>th</sup> etc) and announced that Dr Horrocks and colleagues in Glasgow had become the first recipients of this prize, for randomising the 80<sup>th</sup> patient. Since then, prizes have been won by Dr Butterfield and colleagues at in Utah (90<sup>th</sup> patient) and Dr Kuntz and colleagues in Chicago (100<sup>th</sup> patient). Special prizes have also been awarded to Dr Flanigan and colleagues in Ohio (for randomising two patients in one day!) and to Dr Schara and colleagues in Essen (for exceptional efforts). Randomisations are currently standing at 109, so we are on the brink of the 110<sup>th</sup> randomisation. **Will your site be the next one to earn a set of mugs?**

## HOW ARE WE DOING?



As of 27<sup>th</sup> July 2014, a total of 197 patients have been identified, including 5 patients at sites that have now withdrawn from the study. There have been 45 pre-screen failures, 5 of these at withdrawn sites. A total of 141 patients have been consented, 8 of whom are currently going through screening.



Also as of 27<sup>th</sup> July 2014, a total of 133 patients have been screened post-consent, with 25 screen failures, an overall screen failure rate of 19%. A total of 108 patients have been randomised. Based on the number of open site months, we should have had 211 patients randomised by now, so we're running behind target. Please do all you can to get us back on track!

## FOR-DMD YouTube VIDEO NOW AVAILABLE



We have produced a video, featuring one of our US participants, explaining why this family decided to take part in the FOR-DMD study and showing one of their study visits at the University of Rochester Medical Center. Also in this video, study co Chief Investigator Dr Robert Griggs and site PI, Dr Emma Cifaloni talk from a clinical point of view about the importance of the study and the benefits of taking part.

The video is available on the the FOR-DMD web-site – [www.for-dmd.org](http://www.for-dmd.org) and on <https://www.youtube.com/watch?v=ZZDuIDpTiIM> . We are working on adding sub-titles in Spanish, Italian and German and will let you know when these are ready.

The purpose of this video is to help patients and families who are thinking to take part in the study to better understand what the study is about and what is required of participants. It has been approved by the University of Rochester Research Subjects Review Board (IRB, ethics committee). As it is publicly available, no additional ethical approval is required in the UK for the video to be used as a new study recruitment tool. We are investigating whether additional approval is required in Germany and Italy and will inform you as soon as possible. For sites in the US and Canada, please check with your local IRB/REC as to whether additional approval is needed for the use of this resource in patient recruitment. Please share this link with current subjects and potential study candidates.

## FOR-DMD IN THE SOCIAL MEDIA

The MDA has mentioned FOR-DMD in several social media posts. The study features on its Facebook page at <https://www.facebook.com/MDANational/posts/10152482592296154>. MDA have also Tweeted about FOR-DMD – see <https://twitter.com/MDAnews/status/481905021953798145> and <https://twitter.com/MDAnews/status/481883956175179776>

Remember that we have our own Twitter account! Follow us @FOR\_DMD. If you are a member of the Twitterati, please re-tweet our messages.

## HAPPY HOLIDAYS

We know that many of you will be heading off on vacation, or taking a well-earned break at home, over the coming weeks. We wish you a restful and enjoyable break and a safe journey if you are travelling. Come back refreshed, and ready for a renewed effort at recruitment from September!



