4th Duchenne CAB Meeting
October 2019

The Duchenne CAB held its 4th meeting from 23 – 26 October in Amsterdam. This time we were able to welcome four companies, one of which requested a full day session with the CAB. There was a slightly different compilation of CAB members this time around. We were happy to include a representative from Israel, as well as a new member from Turkey and two alternates (at different times) from Belgium, who stepped in for our indisposed Belgian member.

Main topics discussed

- There was, as always, a lot of discussion around patient friendlier clinical trials, e.g. Improving the clinical trial experience for patients and families by ensuring a comfortable and welcoming space, access to television and computer games etc.; no long placebo trials (24 weeks acceptable in most cases; maximum 1 year); better randomization to placebo (2:1 or even 3:1); how regulators can be convinced to accept natural history data to supplement placebo data; maximum 2 biopsies; the advantages of NSAA over the 6MWT; unobtrusive and "cool" wearable devices to enhance compliance; the need for a more unified steroid regime to eliminate some of the "noise" in clinical trials ; limiting the number of tests to what is important, to mention just a few
- We spoke to several companies attending about managing patient and family expectations of the different treatments currently in clinical trials by being honest and clear in all communications
- Another topic was around AAV antibody testing in relation to gene therapy, and the importance of education and clarity around this specific issue for patients and families, as well as the need for standardized tests
- Making informed consent forms easier to understand while still including all necessary information
- The importance of Real World Evidence and natural history data and how to promote its acceptance not only by regulators but also by national HTA bodies and payers
- How to best demonstrate the meaning of Real World Evidence in relation to quality of life of patients
- The inability of the EQ-5D to measure the impact of treatment in patients with DMD and the necessity for a disease specific measure
- The lack of multidisciplinary adult care centres for DMD in the majority of countries in the face of the ever greater number of patients transitioning from child to adult care

Duchenne CAB Internal

The Duchenne CAB is now an "autonomous" CAB under the EURORDIS EUROCAB program. This means we are no longer mentored by EURORDIS, but autonomously organize everything ourselves, including moderating the sessions. Feedback from companies and CAB members subsequent to the October session was very positive.

CAB dates 2020

Duchenne CAB dates for 2020 are:

13 – 16 May

7 – 10 October

Inquiries

If you have any questions concerning the Duchenne CAB or any issues you would like to bring to our attention, please contact the Duchenne CAB Coordinator: sally@duchennedatafoundation.org