

Report 9th Duchenne CAB Meeting May 2022

The Duchenne CAB held its 9th round of CAB meetings live in May 2022. As not all CAB members were able to attend in-person, as well as companies still being cautious about travel, the meetings were a (somewhat challenging) hybrid version. Nine Duchenne CAB members were present, three joined online. CAB members from twelve countries met with five companies, with representatives both in the room and online. We were happy to welcome two companies new to the CAB.

For some of the general recommendations given to new companies, please refer to the 8th Duchenne CAB report.

Many of the other topics discussed were a deeper dive into previously debated issues, such as:

- Safety events and mitigation strategies in investigational gene therapy trials, including exclusion of certain mutations and its rationale
- Issues around and policies on pre-approval access and compassionate use for gene therapies – attempting to find a fair and just consensus
- The importance of the language used in education materials and communications to enable families to realistically balance hopes and expectations
- Special considerations for clinical trials in the non-ambulant DMD population and in very young children, including outcomes important to the respective families and patients
- Equity and inclusion in clinical trials – how can we all do better?
- And one of the CAB's priorities: is it acceptable to the community to have placebo arms of 18 months' duration in ambulant patients with DMD approaching the late ambulatory stage? Will this compromise recruitment?

Announcement:

Next official Duchenne CAB dates are 19 – 21 October 2022. We are planning on live meetings, provided the pandemic situation and other factors allow everyone to travel. Depending on necessity there may be a hybrid version.

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:

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