

Report 8th Duchenne CAB Meeting September to November 2021

The Duchenne CAB held its 8th round of CAB meetings between September and November 2021. Due to the COVID-19 pandemic, the meetings were again held virtually via video conference. Twelve Duchenne CAB members from twelve countries met with seven companies – four of them new to interactions with the Duchenne CAB - in eight separate meetings over this period.

Main topics discussed with companies new to the CAB:

- Burden of clinical trials for participants and families and strategies to mitigate these burdens, including:
 - Remote, home or local visits wherever feasible and allowed
 - Concierge service for travel and accommodation booking and/or up-front reimbursement via pre-paid debit card (as far as allowed by local regulations)
 - Care or welcome package on first study visit containing all information to guide participants through their day at the site (schedule, map of hospital etc.), snacks and water, gift if allowed
 - Comfortable space with age-appropriate distractions for participants/Wi-Fi access for parents/caregivers/non-ambulant participants
 - How to organize/order clinical assessments for a trial
 - And many more
- Policies to have in place before initiating a clinical trial, e.g.:
 - Sibling policy
 - Compassionate use/expanded access
 - Recruitment policy
 - Cross-border participation policy (when applicable)
 - Long-term extension studies to guarantee participants' access to drug between end of study and approval and reimbursement
- Biopsies
- Placebo and natural history control arms
- The importance of sharing placebo data and returning individual data to participants

Other topics:

- Safety events and mitigation strategies in investigational gene therapy trials, including exclusion of certain mutations and its rationale
- Feasibility of testing potential candidates for investigational gene therapy trials for neutralizing antibodies prior to functional and clinical screening as a means of reducing the emotional burden on families
- Issues around and policies on pre-approval access and compassionate use for gene therapies
- Might value-based agreements (VBA) be a way to bring gene therapies to more patients around the world
- 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
- Diversity and inclusion in clinical trials
- Outcomes important to families and patients

Announcement:

Next official Duchenne CAB dates are 11 – 14 May 2022. A final decision has yet to be made on whether these will be F2F or virtual due to the unclear omicron situation and travel restrictions.

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