6th Duchenne CAB Meeting
October 2020

The Duchenne CAB held its 6th meeting from 07 – 09 October 2020. Due to the COVID-19 pandemic, the meetings had to be held virtually via video conference. The eleven Duchenne CAB members from eleven different countries met with three companies in four separate meetings during the course of these three days. The CAB also held several interim virtual meetings during the second half of 2020.

Feedback from May 2020 meetings:

- The issue of forgoing baseline biopsies was taken up and discussed with regulators and experts. Unfortunately, the outcome at the present time is that baseline biopsies are currently required in order to demonstrate the % of change in the amount of dystrophin from baseline, with the proviso that they may well no longer be necessary in the future once more data has been collected
- The DMD-QoL Duchenne-specific quality of life measure (developed by the HERCULES Project) will be used as an exploratory endpoint in some of the upcoming trials
- Home visits and assessments are being incorporated into clinical trial protocols, partly to mitigate the impact of the COVID-19 pandemic, but also to reduce the burden of numerous hospital visits
- Entertainment devices (e.g. iPads) will be provided at sites for the duration of infusions (in one trial)
- A sibling protocol will be implemented in one of the gene therapy trials

Some of the main topics discussed in October 2020:

- The need to understand how different companies are addressing the issue of neutralizing antibodies: studies are planned to study seroprevalence (the presence of neutralizing antibodies/NABs) and the potential of plasmapheresis and/or a combination of drugs to decrease NABs
- Precautionary measures to mitigate the risk of serious adverse events in gene therapy protocols to include the non-ambulant population
- Recruitment and retention strategies in clinical trials
- Equity and fairness in access to clinical trials
- Newborn screening for DMD: status and challenges

- Access, early access and compassionate use
- Concern around placebo for a duration of 18/24 months
- Confidentiality in clinical trials to maintain the integrity of the trial; necessity of a statement to this effect to be included in the Informed Consent (ICF)

In addition, there are frequent follow-up calls and communication both between CAB and companies as well as internally

Announcement:

Next Duchenne CAB dates for 2021: 5 – 7 May 2021 and 14 – 16 October 2021. As a result of the continuing uncertainties around the COVID-19 situation, the May meeting will be held virtually. We will re-assess the situation before making a final decision about the October meetings.

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