

3rd Duchenne CAB Meeting May 2019

Amsterdam, May 15 – The Duchenne CAB held its 3rd meeting from 8 – 11 May in Amsterdam with a total of six companies attending. Currently, five of the companies have drugs or biologics in clinical trial. One of the companies met with the CAB to seek advice and discuss their clinical trial design. Below, a short overview of our successes to date and the main topics that are discussed during the Community Advisory Board meetings.

Our successes to date:

- Several companies agreed to limit the number of biopsies to no more than two per trial, unless patients and families agreed to a voluntary third one.
- The majority of companies expressed their willingness to share their placebo data after completion of trials and others have shared placebo data. The purpose of the request to share data is to develop a tool that would reduce the required number of trial participants on placebo or eliminate the need for placebo.
- For one trial, the company agreed to reduce time on placebo to 1 year.
- Several companies are now seriously considering the importance of psychosocial support for patients and families during trials and to consider how to best implement this support.

Main topics discussed:

- Best, most effective ways to communicate with Patient Organizations and trial participants and what specific information would be most helpful.
- Outcome measures: How best to prioritize outcome measures included in the NSAA, which of the activities is most relevant to patients and families in daily life and how best to quantify point changes or differences from 0-1-2 and how that relates to meaningful benefit.
- Patient preferences: Benefit/risk studies to evaluate how much ‘good’ people will expect and how much ‘bad’ they are willing to tolerate in exchange.
- Interactions with regulators: how can the patient voice influence decisions.
- Improving the clinical trial experience for patients and families.

Duchenne CAB Internal:

We proudly announce that Pat Furlong (Founding President and CEO PPMD) was unanimously elected as Chair of the Duchenne CAB.

Announcement:

The 4th Duchenne CAB will take place 23 – 26 October 2019.

Inquiries:

If you have any questions or inquiries concerning the Duchenne CAB, please contact the Duchenne CAB Coordinator: sally@duchennedatafoundation.org.