



Report 13th Duchenne CAB Meeting May 2024

In May 2024, the Duchenne CAB held its 13th round of in-person CAB meetings in Amsterdam, where 10 CAB members from ten countries (two were unable to attend for personal reasons) met with six companies.

Some of the topics discussed were:

- Next generation exon skipping therapies
- Clinical trials to examine the feasibility of strategies for overcoming pre-existing antibodies to AAV that preclude a considerable number of patients from gene therapy
- A proposed strategy to address the current exclusion of deletions in the early exons, specifically exons 8/9
- Some of the issues around studies in patients aged 0-48 months
- Publicly funded early access programs (for gene therapy)
- The necessity of global burden of illness and patient preference studies the results of which can be analysed per country and used to inform local HTA (Health Technology Assessment) processes
- The importance of registries to capture burden of disease and leveraging these as sources of Real World Data

Examples of advice provided by the Duchenne CAB that has been implemented:

- Basing inclusion criteria in clinical trials on function rather than on age
- Placebo duration
- Reduction of number of participants subjected to placebo by applying 2:1 or 3:1 randomization and supplementing with external controls
- Less biopsies, only when absolutely necessary, needle biopsy when feasible
- Increasing use of muscle and cardiac MRI as (exploratory) endpoint

- Comprehensive cross-border policies
- Inclusion of a patient representative on Data Safety Monitoring Boards

Quotes from companies:

- The opportunity to discuss with the Duchenne CAB provides a reality check to how industry may think about clinical studies, and the discussion will certainly be included in the clinical development plan
- The Duchenne CAB has provided great insights into study design parameters, outcomes, informed consent forms and the materials required for patients and their families
- Demonstrating that we have worked with those living with the disease is becoming a critical component of the discussion with regulators and may help shape their thoughts on clinical trial requirements

Announcement:

Next official Duchenne CAB dates are 16-18 October 2024.

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@duchenndatafoundation.org