DUCHENNE

COMMUNITY ADVISORY BOARD

Report 12th Duchenne CAB Meeting October 2023

Some statistics: From May 2018 through December 2023 the Duchenne CAB has held 12 CAB sessions over 3-4 days (May and October). These sessions comprised 48 face-to-face meetings with 18 different companies, covering research and development on exon skipping therapies, gene therapies, gene editing and small molecules. In addition there have been 16 virtual meetings over this period.

Over and above these meetings, the Duchenne CAB Chair and Coordinator also consulted with an ethics group to discuss issues around placebo in gene therapy clinical trials.

In October 2023, the Duchenne CAB held its 12th round of in-person CAB meetings in May in Amsterdam, where 11 CAB members from eleven countries (one online as unable to travel) met with five 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 diseaseand 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 15-17 May 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@duchennedatafoundation.org