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Report 11th Duchenne CAB Meeting January - June 2023

The first 6 months of 2023 have been very busy for the Duchenne CAB. The first virtual meeting was held in January, followed by one in March, one in April and one in June.

In addition, the Duchenne CAB held its 11th round of in-person CAB meetings in May in Amsterdam, where 11 CAB members from eleven countries met with five companies.

Some of the topics discussed were:

- Patient experience mapping
- Clinical trials in BMD and the differences to DMD in care management and recruitment strategies
- Necessity to change the language around BMD and DMD: BMD and DMD have different trajectories with their own challenges; language that suggests changing DMD to BMD is dismissive of BMD patients
- Progress made to date in gene therapies; planned studies in non-ambulant DMD patients intended to broaden the label, and CAB concerns about the ethics of an 18-month placebo period in a clinical trial focused on older non-ambulant DMD patients when the natural history of the disease is well understood
- Update on seroconversion studies and strategies to overcome pre-existing antibodies to AAV to enable treatment of patients who screen-fail due to pre-existing antibodies and explore the potential to re-dose patients who have received a gene therapy treatment in the future
- The possibility of adding gene therapy-treated cohorts to future non-AAV-based studies

- The potential necessity for a third biopsy in some studies: rationale and how to educate patients/families, as well as the possibility of an optional rather than mandatory third biopsy or staggering the second biopsy across different time points
- The importance of a comprehensive cross-border policy for patients and families, covering all aspects of relocation required for participation in the clinical study and subsequent planning and timing for returning to the country of origin
- Next generation exon skipping and the potential of initiating platform trials to shorten development time and enable addressing the rarer exon skipping eligible variants (mutations)
- Pros and cons of the NSAA – which items are meaningful to patients and how specific measures translate to real world evidence

Announcement:

Next official Duchenne CAB dates are 18-20 October 2023.

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

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