



## **Report 14th Duchenne CAB Meeting October 2024**

From 16-18 October 2024, the Duchenne CAB held its 14<sup>th</sup> 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. There were also two virtual meetings with two companies before and after the in-person meetings.

We discussed exon skipping studies with three companies, gene therapy with three companies and small molecules with two companies.

### **Some of the topics discussed were:**

- Study protocols and reducing burden for patients and families
- Decreasing burden of trials by offering local testing and blood draws to reduce number of site visits
- Recruitment and retention strategies
- Paediatric Investigation Plans (including younger patients and the non-ambulant population with DMD in studies)
- Expanded access and compassionate use programmes
- New insights into outcome measures including use of wearable devices and video assessments
- Qualitative interviews and patient reported outcome measures
- Sharing individual data with participants
- Access and reimbursement plans
- Strategies to overcome pre-existing AAV antibodies
- Study materials and informed consent forms (ICF) Policy and external program priorities impacting the DMD drug development continuum
- The need for standardization of dystrophin expression readouts

**Quotes from companies:**

- Our conversations around outcomes and data needs were invaluable. As we consider our plans for future approvals and longer term regulatory requirements, we will need to make sure we continue to partner and stay aligned, as we want to make sure we are pulling through the learnings of which outcomes are important and how these outcomes relate to daily life
- It was a great CAB. Only thing is, that when presenting data, we have to be really aware that the parents are incredibly educated and we need to make sure we are super prepared for all the feedback and questions from the group. If a specific slide does not resonate with the group, we should accept the feedback rather than try to convince them otherwise. We also have to be careful with the language when presenting data. I think it would be great to present the key learnings of the CAB to colleagues and discuss where we could improve.

**Announcement:**

Next official Duchenne CAB dates are 7-9 May 2025.

**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\\_cab@worldlduchenne.org](mailto:sally_cab@worldlduchenne.org)