

## Report 10<sup>th</sup> Duchenne CAB Meeting October 2022

The Duchenne CAB held its 10<sup>th</sup> round of CAB meetings face to face in October 2022 in Amsterdam. Once again, the meetings were a hybrid version. Ten Duchenne CAB members were present, two joined online. CAB members from twelve countries met with four companies, some with representatives both in the room and online.

Discussion topics focussed on:

- Gene therapy, gene editing and next-generation exon-skipping
- Access to and reimbursement of approved drugs and the importance of accurate data on incidence and prevalence as well as the burden of illness to enable clinical development and access.
- Global access and barriers to equity
- Planning for potential post-authorization safety and efficacy studies
- Immune response challenges and mitigation strategies in gene therapy
- The challenges of long-term follow-up for gene therapy post-approval
- The necessity of newborn screening for DMD
- Potential impact of vamorolone, if approved and adopted as the standard of care, on future clinical trials
- Sharing data back to clinical trial participants and their families
- Considerations and ideas on initiating new clinical trial sites as the DMD landscape becomes more crowded

### **Announcement:**

The next official Duchenne CAB dates are 10-12 May 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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