

DUCHENNE



COMMUNITY ADVISORY BOARD

GOALS, OBJECTIVES & BENEFITS

Sally Hofmeister, Duchenne CAB Coordinator

The **Duchenne CAB**

is committed to providing its accumulated experience and knowledge in the global endeavour to accelerate development of effective treatments for DMD

Duchenne CAB Objective



Via its CAB, the Duchenne Community is committed to enhancing research and development by sharing its extensive knowledge and experience of living with DMD in all phases of development, from preclinical, clinical, post marketing to access and reimbursement, in order to accelerate access to effective treatments for DMD worldwide.

Duchenne CAB Organization



The **Duchenne CAB** was launched in February 2018 as an independent advisory body. It was set up by **Duchenne Data Foundation** and mentored by EURORDIS under the EURO CAB program.

The **Duchenne CAB** is an independent international board of patient experts from 12 different countries

It meets bi-annually in Amsterdam, after initiating a call of interest and inviting selected industry partners to individual sessions 6 months prior to each meeting

Ad hoc advice is available when required upon prior agreement

Five core elements of the **Duchenne CAB**



Confidentiality: to promote trust between all participants and provide a safe harbour for meaningful interactions with all stakeholders.

Transparency: to guarantee impartiality and integrity in order to maximize the value and impact of the advice given to industry partners.

Sharing: to promote exchange of ideas, knowledge, best practices and data in a "safe" environment, in order to accelerate global drug development in DMD.

Optimization of R&D: to ensure optimal, meaningful and successful trials for all DMD boys and young men.

Joint plan & action: to streamline cooperation between stakeholders, de-risk development and decrease cost from basic research to clinical trials, market approval and access in a global environment.

Benefits of working with the **Duchenne CAB**



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- Attain first hand knowledge of what it means to live with DMD for patients, families and caregivers
 - Be informed of patient needs and preferences, including benefit-risk, from an early stage of development
 - Receive unbiased advice from the Duchenne CAB in order to create meaningful clinical trials
 - Incorporate the community's opinions and preferences and receive assistance to develop optimal clinical trials that better fit patient needs, thereby improving patient recruitment, adherence, and retention

Benefits of working with the **Duchenne CAB** contd.



- Understand and manage the DMD community's expectations with regard to treatments, expanded access, compassionate use and sibling protocols
- Collect meaningful data sets that fulfil clinical, regulatory and HTA requirements
- Resolve issues pertaining to tissue samples, genetic material and data ownership
- Ensure dissemination of accurate information to the media and the DMD community
- Review ICFs and help develop educational materials

Duchenne CAB Impact – some examples



- Max. 2 biopsies per trial unless participants voluntarily assent to more
- Stress the meaning of placebo for participants and families
- 2:1 randomization ratio can influence recruitment and retention
- Lessen the burden of trials
- Sharing placebo data to add to the body of natural history data; sharing individual data with trial participants
- “Best practice” guidance on trial termination

- **“Great insights into study design, parameters, outcomes, ICFs and education materials”**
- **“As we entered the space only six months ago the initial meeting was extremely helpful to obtain early guidance”**
- **“Important to know needs and experience from patient/family point of view”**
- **“We cannot do excellent development of products without the input of the end-user”**

Thank you!

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