Nothing about us without us!

The added value of involving patients/patient representatives in clinical research

Sally Hofmeister
World Duchenne Organization (WDO)
Duchenne Community Advisory Board (CAB) Coordinator

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Disclaimer:

The opinions expressed in this presentation and on the following slides are those of the presenter and do not necessarily reflect those of WDO or the Duchenne CAB as a whole.

The presenter's experience is mostly in the area of one particular rare disease (Duchenne Muscular Dystrophy or DMD) and may not always apply to more common diseases or all other rare diseases.

Patients know best …

Patients and their caregivers live with their particular disease on a daily basis, so they know best:

- Which aspects or symptoms of the disease most require therapeutic intervention to improve their well-being and quality of life (pain, fatigue, loss of motor function, respiratory or cardiac function etc.)
- What needs to change to ensure a meaningful difference to their daily lives (less pain, more energy, retaining motor function, less reliance on ventilators/other devices, protecting the heart from deterioration)
- What their expectations are (cure/stabilization/slower disease progression)

Patient involvement is vital …

Patient/patient representative involvement throughout all stages of drug development are vital:

- to ensure that researchers and pharmaceutical companies address the issues most important to patients
- to enable the design of optimal, patient-friendly clinical trials that:
  - better meet the needs and preferences of those affected
  - minimize the burden of participation in clinical trials
- to increase the chance of success for the company and the patients

Added value …

De-risking and accelerating clinical development through to approvals - well-designed, patient-friendly trials mean:

- less time-consuming amendments
- faster recruitment
- better adherence to the study protocol
- better retention rates

➢Reduce cost and time to potential market approval

How can patients be involved?

Regulatory and payer mandates for patient engagement in drug development are becoming more and more common and have evolved from a “nice-to-have” to a critical imperative,

BUT …

Patient engagement should not be a mere tick-box exercise!

So how? Some examples:

- Company-initiated focus groups/workshops/Ad-Boards
- Community-initiated advisory boards (CABs)

Community Advisory Boards ...

- Initiated by international patient organizations
- CAB members recruited from member organizations
- CAB led by patient representatives with expertise
- CAB determines which companies it meets with
- Agenda set up collaboratively
- CAB moderates the discussion
- CAB establishes the outcomes and makes concrete recommendations
- Company pays a “fee for service”

Advantages of CABs...

- CAB members chosen carefully to represent the whole of the community, all stages of disease
- CAB members undergo training in research and development, clinical trials, regulatory procedures, ethics, HTA etc.
- Non-biased discussion and advice
- Continuity – at least 2-year commitment
- Frequent two-way follow-up between meetings on developments/implementation of advice

The Duchenne CAB

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

Core elements: confidentiality, trust, transparency, sharing knowledge, ideas, best practices

- Set up by WDO as independent CAB in 2018 (WDO is the legal body)
- Bi-annual meeting sessions in Amsterdam with up to 6 companies
- International: trained patient experts from 12 countries
- 12 sessions comprising 64 individual meetings with 18 companies

Some advice implemented and topics discussed …

- Patient preferences on duration and ratio of placebo randomization
- Benefit-risk preferences; managing expectations
- Biopsies – necessity/number/method
- In-/exclusion criteria and relevant outcome measures/endpoints
- Cross-border policies
- Sibling policies
- Expanded access/compassionate use policies
- Reducing burden of CTs: home/local hospital visits where feasible; home infusions
- Access to drug during gap between MA and commercial access
- Sharing aggregate and individual CT results with patients

Added value: what companies say …

- 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 regarding the clinical development plan
- The Duchenne CAB has provided great insights into: study design parameters, outcomes, ICF and education required for patients/families
- Demonstrating 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

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Thank you!

Contact info Duchenne CAB Coordinator:

sally@duchennedatafoundation.org

sally.hofmeister@worldduchenne.org