







PROJECT HERCULES: CONSTRUCTION OF A NEW PREFERENCE-BASED MEASURE OF QUALITY OF LIFE FOR DUCHENNE MUSCULAR DYSTROPHY (DMD)

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Objectives

Duchenne muscular dystrophy (DMD) is a rare progressive lifelimiting paediatric neuromuscular disease. There is no known cure, so interventions focus on slowing progression and improving quality of life (QoL). Evidence suggests that existing preferencebased measures (PBMs) may be inadequate for assessing QoL in DMD. Project HERCULES is developing a new PBM in DMD.

Methods

Here we describe the results of stages 1 and 2a of a 3-stage PBM development process. In Stage 1, we undertook 18 semi-structured interviews with people with DMD of varying ages. We used framework analysis to identify themes and an initial descriptive system. In Stage 2a, cognitive debriefing was done with 10 patients, 10 parents, and 8 clinicians to refine a draft questionnaire.

Feelings and emotions

1) Worry, anxiety, stress, and uncertainty; 2) Annoyance, frustration, and anger; 3) Happiness, sadness, and depression; 4) Loneliness; 5) Coping and adaptation

Social relationships

6) Friendships; 7) Assistants; 8) Family; 9) Romantic; 10) Social participation; 11) Way treated by others

Physical aspects

12) Getting around; 13) Upper body function; 14) Accessibility; 15) Pain and discomfort; 16) Fatigue; 17) Sleep; 18) Communication and being understood

Autonomy

19) Choice and control; 20) Knowledge/awareness; 21) Dependency on others; 22) Perceived burden on others

Identity

23) Belonging and community; 24) Age-appropriate identity; 25) Confidence/self-image; 26) Personal identity

Daily activities

27) Work and school; 28) Hobbies/leisure activities; 29) Self-care; 30) Activities of daily living; 31) Planning and lack of spontaneity; 32) Adaptation to physical changes

Healthcare services

- 33) Devices and equipment; 34) Assistive technology;
- 35) Burden of healthcare; 36) Access to healthcare provisions; 37) Medicines; 38) Support from others

ITEM GENERATION

Reading level appropriate Not double-barrelled item Not double negative

No jargon Use language from interviews Makes sense to participants

> Taps into current QoL Does not assess a trait Responsive over time

Easy to complete Relevant to all Appropriate to ask

Ensure coverage Minimise overlap Reduce redundancy

Psychometrically sound Sensitive to change (HTA) **Ensure monotonicity**

> **Predictor** of QoL

Draft DMD-QoL items

- I felt unhappy / I felt sad / I felt upset.
- I felt annoyed / I felt angry.
- I felt lonely.

I felt worried.

- Added at debriefing: I felt embarrassed.
- Added at debriefing: I was worried about the future.
- Added at debriefing: I felt I could cope with things.
- I was happy with the people around me / I felt okay with the people around me / I enjoyed being with the people around me.
- I could take part in things with my friends / I could do the things I wanted with my friends.
- I felt left out / I felt accepted / I felt I fit in
- I liked the way I was treated by people.
- I found it hard to talk to people / I had problems talking to people / I found it difficult to talk to people.
- I was happy with the friends I had.
- I felt happy with the people who were helping me.
- Added at debriefing: I was happy with the people I had to help me.
- I was in pain / I felt in pain.
- I felt tired.
- I found it hard to get around / I had problems getting around / I found it difficult to get around.
- I found it hard to use my arms / I had problems using my arms / I found it difficult to use my arms.
- I found it hard to use my hands / I had problems using my hands / I found it difficult to use my hands.
- Added at debriefing: I found it hard to use my fingers.
- Added at debriefing: I found it hard to breathe.
- I could take part in the things I wanted to / I could join in with the things I wanted to / I did the things I wanted to / I could do the things I wanted to.
- I had as much choice as I wanted.
- I felt okay having to ask people for help.
- I was happy with the things I could do myself.
- Added at debriefing: I could do the things I wanted to by myself.
- I felt good about myself.
- Added at debriefing: I felt good about my appearance.
- I did the things I enjoyed / I could do the things I enjoy.
- Added at debriefing: I found it hard to eat.

Figure 1. Mapping qualitative themes onto items using principles of item generation. 43 items went to cognitive debriefing. Bold items chosen after cognitive debriefing for 27-item draft measure.

Results and Conclusions

Seven QoL domains were identified as important in Stage 1. A draft 43-item questionnaire was developed. In Stage 2a, the draft questionnaire was refined following cognitive debriefing. This included an assessment of content validity, item wording, response options, and instructions. This resulted in a 27-item draft measure to be tested in a national psychometric survey (see Figure 1). The draft questionnaire has high content validity. Key challenges included a sensitive subject matter and differing views as to which items should be included in the new PBM.



















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