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Quality of life in Duchenne muscular dystrophy (DMD): initial findings and protocol for a new preference-based measure



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Aims

- Duchenne muscular dystrophy (DMD) is a rare inherited neuromuscular disorder that predominantly affects boys and men (1:3800 to 1:6300 live births).
- The disease causes progressive muscle weakness, impaired ambulation and motor functioning, and cardiovascular and respiratory problems.
- The aim of this research was to identify quality of life (QoL) themes relevant to people with DMD and their families and use them to inform a protocol for a new preference-based measure (PBM) of QoL for DMD.

Methods and Results

- A rapid review of MEDLINE, Embase, CINAHL and trial registries between 2010 and 2016 was conducted.
- Studies measuring QoL in patients with DMD and/or their parents or carers were included and coded to extract themes relevant to QoL. Themes were pooled and grouped into a thematic framework (Figure 1).
- A mixed-methods protocol has been developed for producing a new condition-specific PBM of QoL in DMD, with input from key clinical and academic stakeholders (Figure 2).

Figure 1 QoL themes identified from the rapid review. Forty-five studies were identified featuring 36 measures of QoL covering 5 domains.

Physical

- Physical QoL
- Health behaviour
- Sleep
- Pain
- Daily activities

Psychological

- Psychological QoL
- Happiness
- Depression
- Anxiety
- Coping
- Communication

Social

- Social QoL
- Participation
- Friends
- Relationships
- School/work

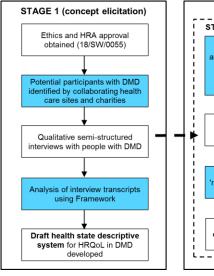
Wellbeing

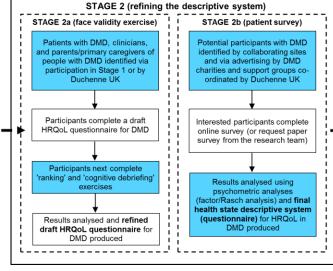
- Wellbeing QoL
- Independence
- Dignity
- Energy/fatigue

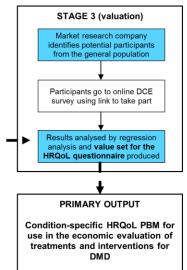
Other

- Accessibility/ wheelchair use
- Healthcare service provision
- Treatment/ therapy effects

Figure 2 Research project protocol diagram. Design stages omitted. DMD: Duchenne muscular dystrophy; DCE: discrete choice experiment; HRA: Health Research Authority; HRQoL: Health-related quality of life; PBM: preference-based measure.







Conclusions

- The review highlighted multiple QoL themes in DMD, which are not adequately addressed with generic preference-based measures.
- We present a protocol for a condition-specific PBM in DMD. Challenges include recruitment in a rare disease and producing a universal measure.

Funding

This work is funded by Duchenne UK under the Project Hercules (Health Research Collaboration United in Leading Evidence Synthesis) stream.



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