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

Philip Powell[1,2], Jill Carlton[1], Donna Rowen[1], Lesley Uttley[1], Helen Buckley Woods[1], John Brazier[1]

[1]School of Health and Related Research, University of Sheffield, Regent Court, 30 Regent Street, Sheffield, S1 4DA, UK

[2]Department of Economics, University of Sheffield, 9 Mappin Street, Sheffield, S1 4DT, UK

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.

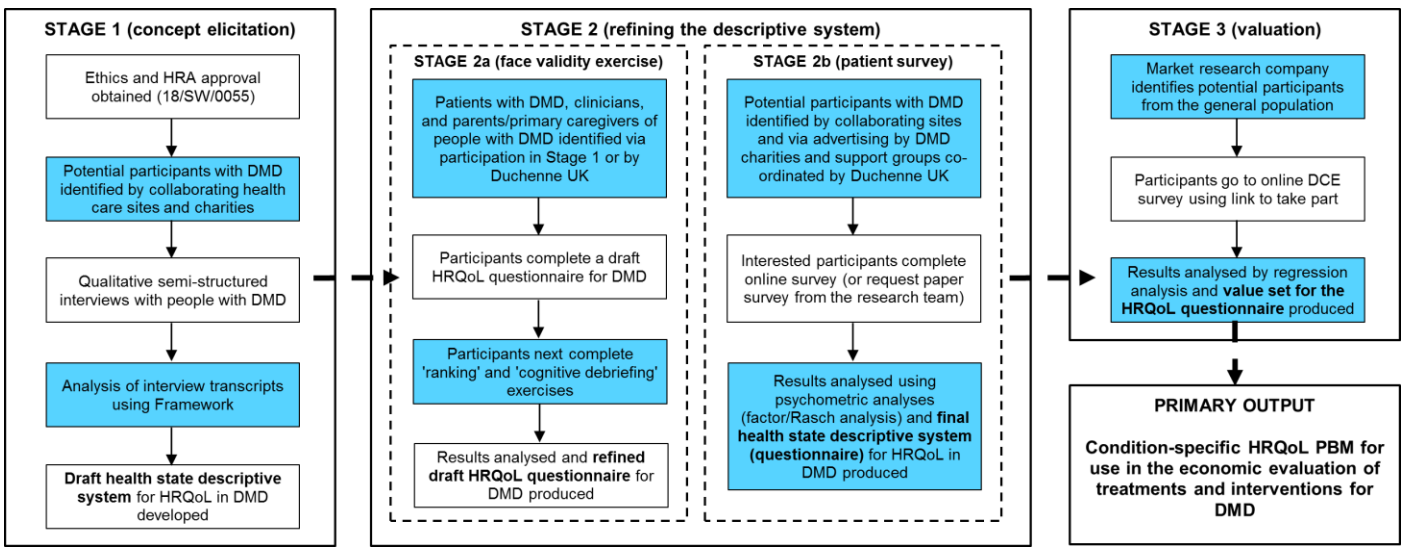
Methods and Results

- 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	Psychological	Social	Wellbeing	Other
Physical QoL	Psychological QoL	Social QoL	Wellbeing QoL	Accessibility/ wheelchair use
Health behaviour	Happiness	Participation	Independence	Healthcare service provision
Sleep	Depression	Friends	Dignity	Treatment/ therapy effects
Pain	Anxiety	Relationships	Energy/fatigue	
Daily activities	Coping	School/work		
	Communication			

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.

Contact us:

p.a.powell@sheffield.ac.uk

www.scharrheds.blogspot.co.uk

www.sheffield.ac.uk/heds

@scharrheds

www.facebook.com/scharrsheffield