Measuring quality of life for economic evaluation in Duchenne muscular dystrophy (DMD) from childhood to adulthood: Protocol for a new preference-based measure

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Objectives

- **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 is to identify **quality of life (QoL) themes** relevant to people with DMD and their families and to develop a new **preference-based measure (PBM)** of QoL for DMD.

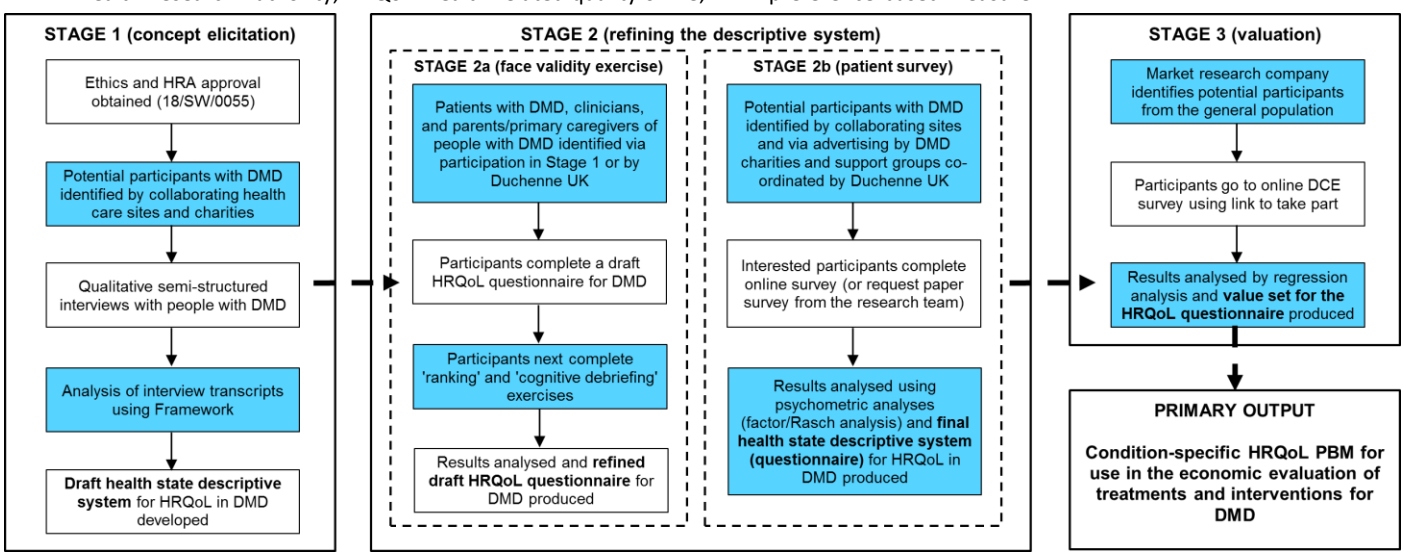
Methods

- A **rapid review** of MEDLINE, Embase, CINAHL and trial registries between 2010 and 2016 was conducted.
- A **mixed-methods protocol** has been developed for producing a new condition-specific PBM of QoL in DMD, with input from the rapid review, and key clinical and academic stakeholders (**Figure 1**).
- Research challenges include producing a single measure of QoL appropriate for **all stages of the disease and across age groups**, and patient recruitment and engagement in a **rare disease**.

Results

- 45 studies on QoL were identified in a review, with 36 measures of QoL across 5 domains: **physical** (e.g. daily activities); **psychological** (e.g. happiness); **social** (e.g. participation); **wellbeing** (e.g. dignity); and **other** (e.g. treatment effects).
- 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 1**).
- The protocol includes: 1) **concept elicitation** via interviews with 20 boys and men with DMD; 2) **face validity exercises** and quantitative surveying to **refine the descriptive system**; and 3) **valuation and modelling** using a DCE_{TTO} survey.

Figure 1 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

- An initial 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**.

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