Measuring quality of life in people with Duchenne muscular dystrophy: developing a new patient reported outcome measure

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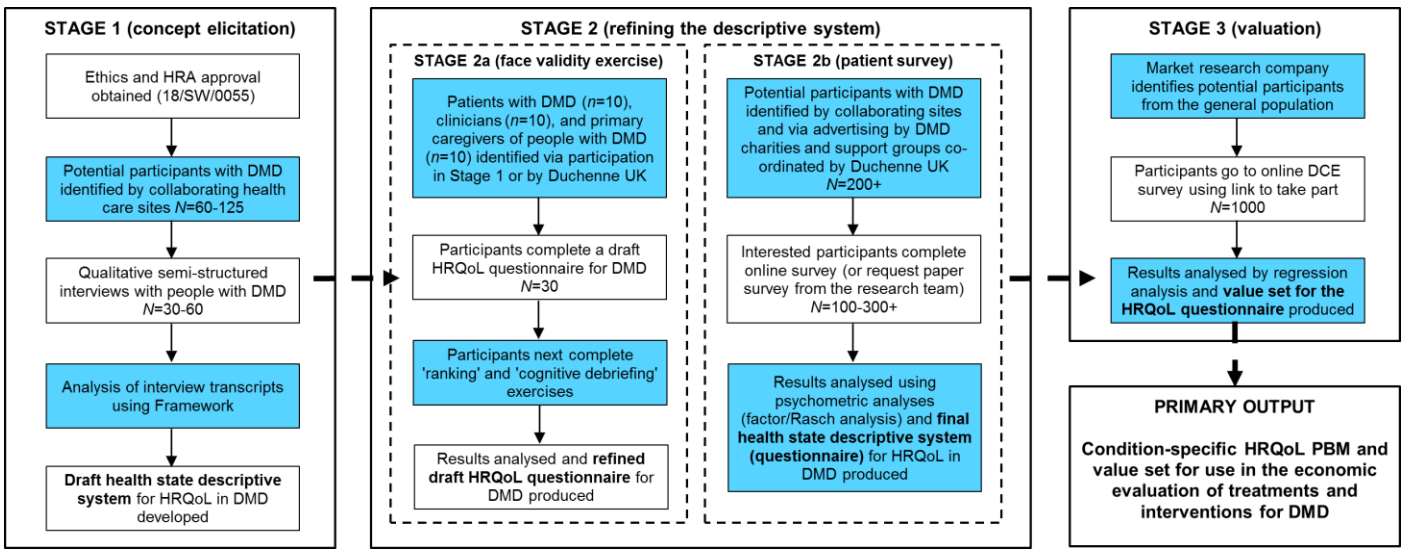
Background

- **Duchenne muscular dystrophy (DMD)** is a rare inherited neuromuscular disorder that predominantly affects boys and men.
- The disease causes progressive muscle weakness, impaired ambulation and motor functioning, and cardiovascular and respiratory problems.
- There is concern that generic **preference-based measures (PBMs)** of **health-related quality of life (HRQoL)** lack specificity to assess aspects of HRQoL especially important to people with DMD.

Aims

- Using **qualitative interviews** and **review methods** develop a **draft descriptive system** for measuring HRQoL in people with DMD.
- Using **face validity** and **psychometric analyses** with DMD patients, carers and clinicians, produce a **refined descriptive system (questionnaire)** for measuring HRQoL in DMD.
- Design and conduct a **valuation study** using a **discrete choice experiment with duration (DCE_{TTO})**, with the adult UK public to produce a **utility value set**.

Figure 1 Research project process 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



Methods and analysis

- **Stage 1 – concept elicitation**: 30 to 60 boys and men (aged 7+ years) will take part in a **semi-structured qualitative interview** about their HRQoL. They will be recruited via five UK NHS sites. The data will be analysed using **Framework**.
- **Stage 2 – refining the descriptive system**: 10 patients, 10 carers, and 10 clinicians will assess the **face validity** of the descriptive system (questionnaire) from the analysis in Stage 1. The draft questionnaire will be administered alongside a selection of generic PBMs online to 100-300+ boys and men with DMD (aged 7+ years) recruited from NHS sites and via Duchenne UK. **Psychometric analyses (factor and Rasch analyses)** will be used to refine the draft questionnaire.
- **Stage 3 – valuation and econometric modelling**: 1000 adults from the UK public will take part in an **online DCE_{TTO} survey** to value health states from Stage 2. The data will be analysed with **regression analysis** to produce utility values.

Ethics and dissemination

- This study has a **favourable ethical approval** from the NHS South West – Central Bristol Research Ethics Committee (REC 18/SW/0055).
- The primary output is a **condition-specific PBM** for assessing HRQoL in DMD. Results will be disseminated in **open access publications**.

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