

Development of a New Quality of Life Measure for Duchenne Muscular Dystrophy Using Mixed Methods

The DMD-QoL

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Study Question

To develop a patient-reported outcome measure (PROM) to evaluate quality of life (QoL) in boys and men with Duchenne muscular dystrophy (DMD).

What Is Known and What This Paper Adds

Health-related QoL in people with DMD is impaired compared to the general population, but there is no optimal measure of QoL in DMD for use in clinical care and research studies. This study developed a new 14-item QoL PROM for patients with DMD that has demonstrable content and face validity.

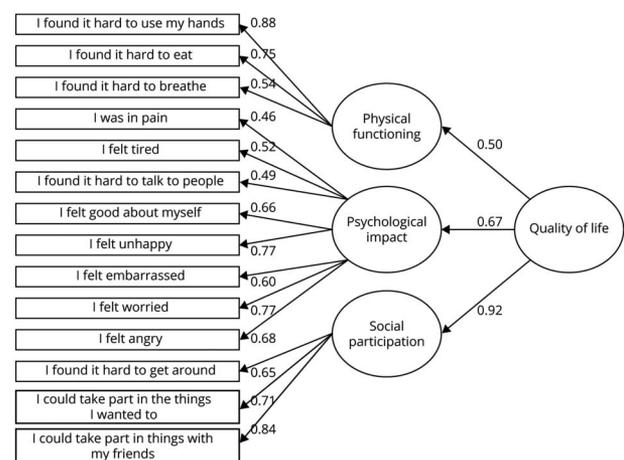
Methods

For this instrument development study, the investigators relied on a 3-step procedure. The first step involved using a framework analysis to generate draft items based on semistructured qualitative interviews with 18 male patients with DMD recruited through 5 UK National Health Service sites and the charity Duchenne UK. These interviews occurred in 2018. The second step involved conducting cognitive debriefing interviews with 10 patients, 8 clinicians, and 10 parents of patients. The interviews occurred in early 2019. The objective of these interviews was to select and refine a reduced item list. The third step involved conducting an online survey of 102 UK-based patients and parents and collecting stakeholder input between late 2019 and early 2020. The objective in the third step was creating the final questionnaire, which was the primary outcome. Patient and public involvement and engagement was embedded throughout the process.

Results and Study Limitations

The initial draft of the QoL PROM featured 47 items, but the interviews during the second step led to a reduced list of 27 items. The final QoL PROM had 14 items. This finalized QoL PROM is appropriate for 7- to 9-year-old patients via proxy report and for ≥10-year-old patients via self-report or proxy

Figure Items from the Novel QoL PROM



The 14 items featured in the newly developed QoL PROM.

report. The final measure showed good psychometric properties. The QoL PROM's limitations include a lack of attention to QoL aspects like sexual relationships that are important to adults but inappropriate for children, the need for validation in an independent dataset, and the need for cross-cultural validation with patients from outside the UK.

Study Funding and Competing Interests

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A draft of the short-form article was written by M. Dalefield, a writer with Editage, a division of Cactus Communications. The corresponding author(s) of the full-length article and the journal editors edited and approved the final version.

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