

Examination of Content Validity of the EQ-5D/Y in Duchenne Muscular Dystrophy

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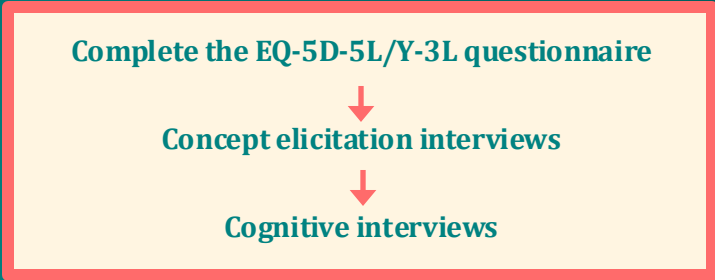
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Objectives

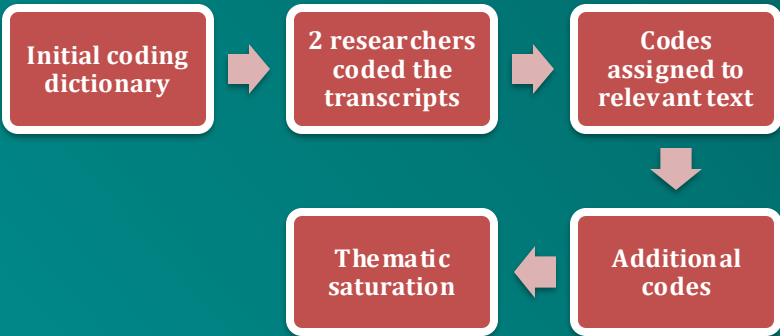
- Evaluate the content validity of the EQ-5D-5L and EQ-5D-Y-3L in adult and pediatric DMD patients.
- Explore the potential bolt-on items for EQ-5D-5L and EQ-5D-Y-3L in adult and pediatric DMD patients.

Methods

- Participants:** 15 adults (≥18 yrs) and 15 pediatric patients (8-15 yrs) from two DMD patient associations in China.
- Study design:** Web-based and semi-structured interviews
- Procedure:**



- Data analysis**



Conclusion

- Both EQ-5D-5L and EQ-5D-Y-3L demonstrated acceptable relevance, comprehensiveness, and comprehensibility in patients with DMD.
- These measures may lack certain dimensions needed for patients with rare diseases. Testing some bolt-ons for this population would be valuable.

Results

EQ-5D-5L			EQ-5D-Y-3L		
Health and overall QoL	Physical Health	Muscle weakness limits activities and self-care, leading to reliance on others.	Health and overall QoL	Physical Health	Dependence on assistance for basic tasks, difficulty walking, and potential school withdrawal.
	Mental Health	Patients face anxiety, depression, and loneliness, especially when comparing themselves to peers.		Mental Health	Mixed feelings; some are anxious about peer teasing and experience irritability from medications.
	Social Well-being	DMD restricts social interactions, increasing feelings of isolation and embarrassment.		Social Well-being	While most have friends, they often lack deep connections, and their interactions tend to be limited to passive activities.
Comprehensiveness	Mobility	The definition needs clarification regarding whether assistive devices should be included in the item and how upper limb strength should be assessed.	Comprehensiveness	Mobility	The definition needs clarification regarding whether assistive devices should be included in the item.
	Self-Care	The example is inappropriate, suggest to include using the restroom and eating independently.		Looking after Myself	The scope should extend to more complex daily tasks, such as managing personal hygiene, meal preparation, and maintaining household responsibilities.
	Usual Activities	Examples are insufficient, social engagement should be included.		Doing Usual Activities	Fail to account for the critical role of social engagement and educational participation in supporting the development of children with DMD.
	Pain/Discomfort	Patients' high pain levels remain inadequately addressed; the source of pain is unclear.		Pain/Discomfort	Discomfort levels may not be accurately measured, as the definition is not clear.
	Anxiety/Depression	Mental health requires more attention, particularly through adding measures for loneliness and emotional states.		Feeling worried, sad, or unhappy	Insufficient focus on emotional challenges unique to children with DMD, e.g., loneliness and stigma.
Suggestions	Longer Time Frame	Prefer "for the past few weeks" to "today" for assessments.	Suggestions	Bolt-on(s)	Muscle strength-related activities; Sleep issues; Impact on schooling
	Bolt-on(s)	Add social support to better capture social integration. Add cognition to capture its impact on quality of life.			

Suggestions for future studies

Assessment of psychometric properties

- In a large sample of DMD/neuromuscular disease patients
 - H1: Dimensions of the EQ-5D capture HRQoL aspects for DMD patients
 - H2: The questionnaire is comprehensible across ages and cognitive levels
 - H3: Measure HRQoL across different stages of disease progression

Explore bolt-on items

- Fatigue
- Social relationship;
- Cognition;
- Loneliness
- Schooling and learning difficulties
- Sleep

Performance in clinical setting

