The reliability and validity of the Greek version of the Pediatric Quality of Life Inventory™ 3.0 Duchenne muscular dystrophy module in children with Duchenne muscular dystrophy.

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Background

- Duchenne Muscular Dystrophy (DMD) is a rare condition that belongs to a wider group of diseases related to dystrophin, a protein found in muscle fibers, responsible for muscle function[1].
- In DMD, dystrophin is not expressed partially or completely, due to many and different mutations in the relevant gene, resulting in the gradual deterioration of vital functions[1].
- Measuring health-related quality of life (HRQL) in a disease of this nature, with multifactorial management, which includes complex care by doctors of different specialties (neurologists, developmentalists, cardiologists, orthopedics, pulmonologists) and other therapists (occupational therapists, physical therapists, psychologists, nutritionists, nurses), is considered very crucial, in order for decision makers to improve the provision of services for these patients[2].
- PedsQL 4.0 Generic Core is widely accepted and used in HRQL studies including those in DMD patients. PedsQL 4.0 Generic Core and PedsQL 3.0 DMD Module have been used together in order to get more consolidated results in HRQL studies in Duchenne people[3].
- A Greek translation of the PedsQL™ 3.0 DMD module is available. However, a validated Greek language version of the PedsQL™ 3.0 DMD module is not yet available[4].

Objective

The objective of the study was twofold: firstly, to assess the psychometric properties of the Greek linguistic adaptation of the PedsQL™ 3.0 DMD module in children aged 5–18 years with DMD, and secondly to make the Greek version available for use in evaluating treatment efficacy and HRQoL outcomes.

Methods

Study design and Sample

- Following ethics committee approval by the University of Peloponnese and the involved hospitals, a cross-sectional study was conducted between September 2022 and July 2023.
- Seventy-nine children with DMD and their parents/caregivers were recruited from the AHEPA University Hospital, Neuromuscular clinic and University Hospital of Patras, Neuromuscular clinic during their annual scheduled DMD clinic visit or by phone call through MDA-Hellas Registry, a patients' organization for neuromuscular diseases.
- The linguistic adaptation of the PedsQL™ 3.0 DMD module into the Greek language was approved by the inventor and is described in detail in a previous publication[4].

Measures, procedures and data analysis

- The PedsQL™ 3.0 DMD module consists of a child self-report and parent proxy report with 18 items in four domains, comprising "Daily Activities" (5 items), "Treatment Barriers" (4 items), "Worry" (6 items) and "Communication" (3 items). Child self-reports vary according to age group: 8–12 years (children) and 13–18 years (adolescents).
- Parent proxy reports vary according to age groups: 5–7 years (young children), 8–12 years (children) and 13–18 years (adolescents).
- The questionnaire employs a 5-point response scale, whereby respondents indicate the frequency with which each item has been problematic over the past month. The scale ranges from 0; indicating that the item has never been problematic, to 4: indicating that it has been an almost constant problem. Items are reverse scored and linearly transformed to a 0–100 scale (0 = 100, 1 = 75, 2 = 50, 3 = 25, 4 = 0), with a higher score indicating a better HRQoL
- Data were analyzed with Statistical Package for the Social Sciences (SPSS)20. Descriptive statistics were generated for demographic of children with DMD and clinical variables and are reported as mean and SD values for continuous variables and frequencies/proportions for categorical variables.

Results

- Baseline demographic and clinical data are shown in Table 1. A total of seventy-nine children with DMD and their parents/caregivers were enrolled in the study.
- The number of eligible questionnaires included in the analysis was seventy (27 child self-reports and 43 parent proxy reports). The mean age of the patients at the time of data collection was 12.52 years (SD = 2.97) (ranged between 8 to 19 years).
- The mean age at the time of diagnosis was 3.58 years (SD = 2.83,ranged 0 to 10 years). One-third (32.3%) of the patients were non-ambulatory, around half of whom (51.6%) undergoing steroid treatment, and 72.1% had inherited the disease maternally.
- The age range of the Duchenne boys (both the ones been interviewed and the ones not) was from 6-17 with an average age of 10 years old while the age range for the boys been interviewed was from 9-17 with an average age of 12 years old.

Table 1.Demographic data

Variable	Mean	SD
Age of onset [years]	3.58	2.83
Age at time of the evaluation [years]	12.52	2.97
	Mean	**SD**
Variable	N	[%]
Age distribution 5–7	7	20.6%
Age distribution 8–12	16	47.1%
Age distribution 13–18	11	32.4%
Non-ambulatory patients	10	32.3%
On steroid treatment	16	51.6%
Positive family history	31	72.1%

- The internal consistency reliability of the scale was assessed using Cronbach's alpha coefficient. Both the child self-report and parent proxy report total scores surpassed the minimum reliability threshold of 0.7 (child report total score α = 0.8, parent report total score α = 0.89).
- To assess the correlations between items and their subscales, the Spearman correlation coefficient was used. It is found that all items had moderate to good correlations with their hypothesized subscales for the child report and good to excellent correlations for the parent report.
- A subset of children (n = 7) and parents (n = 10) completed the Greek version of the PedsQL™ 3.0 DMD module a second time. ICCs for test-retest reliability showed excellent agreement for the total score for the child self-report (ICC = 0.92) and the parent proxy report (ICC = 0.81)(Table 2).
- Specifically, there was good agreement for most subscales for the child self-report questionnaire and the parent proxy report questionnaire, except for the treatment barriers subscale, which showed moderate agreement in both reports. The communication subscale demonstrated poor agreement in both the child self-reports and the parent proxy reports.
- Furthermore, the ICCs were employed to evaluate the degree of concordance between the responses provided by the parents and those given by the children. The level of agreement between the responses provided by parents and their children was deemed to be satisfactory for the total score (ICC = 0.8) and for two of the four subscales (daily activities and treatment barriers, ICC 0.8 and 0.76, respectively). Moderate agreement was observed for the subscale communication (ICC = 0.35), while the agreement for the subscale worry was poor (ICC = 0.43) (Table 3).

Table 2. Test-retest reliability of the Greek version of the PedsQL™ 3.0 Duchenne muscular dystrophy module

Scale	Intraclass correlation coefficient (95% CI)
Child self-report	
Total (18)	0.92 (0.53–0.99)
Daily activities (5)	0.81 (−0.11–0.97)
Treatment barriers (4)	0.44 (−0.39–0.88)
Worry (6)	0.71 (−0.67–0.95)
Communication (3)	0.07 (−0.67–0.74)
Parent proxy report	
Total (18)	0.81 (0.33–0.94)
Daily activities (5)	0.68 (−0.13–0.91)
Treatment barriers (4)	0.4 (−0.35–0.87)
Worry (6)	0.8 (0.31–0.94)
Communication (3)	0.24 (−0.39–0.68)

Table 3. Parent–child agreement of the Greek version of the PedsQL™ 3.0 Duchenne muscular dystrophy module

Scale	Intraclass correlation coefficient (95% CI)
Total (18)	0.8 (0.57–0.91)
Daily activities (5)	0.76 (0.47–0.89)
Treatment barriers (4)	0.7 (0.32–0.86)
Worry (6)	0.35 (−0.43–0.7)
Communication (3)	0.43 (−0.25–0.74)

Conclusions

In conclusion, the evidence presented in this study suggests that the PedsQL™ 3.0 DMD module Greek version constitutes a disease-specific instrument with satisfactory psychometric properties for measuring HRQoL in pediatric patients with DMD.

Furthermore, it can also be employed as a reliable and valid outcome measure to assess the efficacy of treatments in both research and clinical practice.

References

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