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# 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

## Abstract

**Introduction:** This cross-sectional study aimed to evaluate the reliability and validity of the Greek version of the Pediatric Quality of Life Inventory™ (PedsQL™) 3.0 Duchenne muscular dystrophy (DMD) module.

**Methods:** The Greek version of PedsQL™ 3.0 DMD was completed by children with DMD and their parents/caregivers during their annual clinical visit to a hospital setting. Internal consistency reliability (Cronbach's $\alpha$) and reproducibility (ICC, intraclass correlation coefficients) of the DMD module were assessed, and test-retest reproducibility was evaluated after 6 to 8 months. Known-group validity was evaluated by comparing scores between different patient groups.

**Results:** A total of 79 children with DMD and their parents/caregivers were enrolled in the study. Internal consistency reliability was confirmed as Cronbach's $\alpha$ was $> 0.70$ (total score: child $\alpha = 0.8$, parent/caregiver $\alpha = 0.89$) and the ICC exceeded 0.6 (for the total score of the child report 0.92 and 0.81 for the parent/caregiver report). Construct validity of PedsQL™ 3.0 DMD module was confirmed. The mean quality of life total score for child self-report was 76.29 ± 13.27 among the ambulatory patients and 56.91 ± 13.27 among the non-ambulatory patients ($p < 0.001$). The mean quality of life score for the parent/caregiver proxy report was 70.64 ± 20.75 among ambulatory patients and 52.15 ± 22.54 among non-ambulatory patients ($p < 0.001$). The child self-reports were in good agreement with the parent/caregiver proxy reports for most subscales (ICC range 0.49–0.81, 0.57–0.91).

**Conclusions:** The PedsQL™ 3.0 DMD module is valid and reliable in Greek patients with DMD for measuring disease-specific health-related quality of life (HRQoL).

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**Keywords:** health-related quality of life, Duchenne muscular dystrophy, PedsQL, Greek language, psychometric properties

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## Introduction

Duchenne muscular dystrophy (DMD) is a rare condition primarily affecting young children, particularly boys, with an incidence rate ranging from 15.9 to 19.5 per 100,000 newborn males in the USA and UK, respectively [1]. The global pooled birth prevalence stands at 19.8 per 100,000 newborn males [2]. DMD is the most prevalent neuromuscular disorder [3] associated with dystrophin, a protein indispensable for muscle function. In DMD patients, dystrophin expression is absent; resulting in the progressive decline in muscle function [4, 5]. The initial impact is on movement, with ambulation typically lost between the ages of 10 and 14. This is followed by a loss of upper body mobility [4, 5]. Subsequently, the weakness of smooth muscles irreversibly impairs respiratory and cardiac function, with cardiac or respiratory failure being the leading cause of death among DMD patients [4, 5]. The progressive loss of motor function places a considerable burden on both patients and their families [6, 7]. This burden encompasses physical, psychosocial, and school-related functioning [6–10]. Assessing disease progression and treatment efficacy relies on patient-reported outcomes like health-related quality of life questionnaires [11–13]. Many health-related quality of life (HRQoL) instruments are available, comprising generic core instruments that evaluate HRQoL across diverse populations and disease-specific modules comprising items pertinent to the conditions of specific patients [14].

The Pediatric Quality of Life (PedsQL™) Inventory 4.0 Generic Core Scales questionnaire, comprising both child self-report and parent proxy-report, is designed to assess HRQoL in children and adolescents aged 2–18 in relation to their physical, emotional, social, and school functioning over the previous 1 month (https://www.pedsql.org/about_pedsql.html). The PedsQL™ 3.0 DMD module has been designed to assess HRQoL in children with DMD from the age of 5 to 18 years in relation to their daily activities, treatment barriers, worry, and communication over the previous 1 month. Currently, the Greek language version of the PedsQL™ 4.0 Generic Core scale is available for the general population [15, 16]. In addition, a Greek translation of the PedsQL™ 3.0 DMD module is available [17]. However, a validated Greek language version of the PedsQL™ 3.0 DMD module is not yet available. 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.

## Patients and methods

### Study design and patient population

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 [17]. The PedsQL™ 3.0 DMD module questionnaire was administered in person in the neuromuscular clinics to children with DMD and their parents/caregivers, or by phone call. Subject selection was performed by a researcher using purposive sampling. Inclusion criteria were: 1) confirmed DMD or BMD diagnosis by either genetic testing or muscle biopsy 2) age 5 to 18 years. A written informed consent was obtained from all study patients/caregivers.

### Measures and procedures

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. The PedsQL™ 3.0 DMD module questionnaire was administered to children with DMD and their parents/caregivers, separately in a non-interactive manner. If a child was unable to read, a non-healthcare researcher read the questionnaire and recorded the child's responses using the scale. Baseline demographic and clinical data were collected from medical records. The initial completion of the questionnaires occurred at varying times for each participant. Consequently, the interval

between the initial and subsequent responses to the questionnaire ranged from six to eight months.

### Statistical analysis

Data were analyzed with Statistical Package for the Social Sciences (SPSS) version 20.0. The demographic data of the patients were reported with descriptive statistics, such as percentages, means, standard deviations, and ranges. The feasibility of the questionnaire was assessed using the percentage of missing data. The percentage of scores at the extremes of the scaling range, that is, the "ceiling effect" (the maximum possible score) and the "floor effect" (the minimum possible score) [18], were determined. The ceiling and floor effect provide information on the distribution of the scale. Surveys with small floor or ceiling effects (≤ 15%) are considered to have acceptable measurement standards, while surveys with moderate floor or ceiling effects (> 15%) are considered less precise measurements. The internal consistency reliability of the Greek version PedsQL™ 3.0 DMD scale was determined by calculating Cronbach's $\alpha$ coefficient [19, 20]. Scales with reliability ≥ 0.70 are considered satisfactory and are recommended for comparing patient groups.

The item-subscale correlations for the Greek version PedsQL™ 3.0 DMD were determined at baseline using the Spearman correlation coefficient, which is a number between −1 and 1 and measures the linear association of two variables indicating the strength and the direction of a relationship between them. Good scaling is achieved if the correlation between an item and its hypothesized subscale is stronger than its correlation with other subscales. The test-retest reliability of the Greek version of the scale was assessed for a subset of the sample (n = 17) using the intraclass correlation coefficient (ICC) [21, 22]. The intraclass correlation coefficient is a descriptive statistic that describes to which extent two different groups are likely to resample. The range of intraclass correlations is from −1 to 1, with higher values indicating better agreement. ICC ≤ 0.4 is designated as indicating poor to fair agreement, 0.41–0.60 as moderate agreement, 0.61–0.8 as good agreement, and 0.81–1 as excellent agreement. ICC was also used for the determination of agreement between child self-reports and parent proxy reports for the Greek version PedsQL™ 3.0 DMD scale. Construct validity was assessed between ambulatory and non-ambulatory children and between children who were receiving steroids and those who were not receiving them, by using the independent sample t-test to compare first evaluation scores [3]. Furthermore, construct validity was also assessed by Pearson correlation among the Greek version of PedsQL™ 3.0 DMD scales and the PedsQL 4.0 Generic Core scales [23]. Correlation is designated as small (0.1–0.29), medium (0.3–0.49), and large (≥ 0.5).

Additionally, a comparison analysis of the PedsQL™ 3.0 DMD scale by age group was conducted using non-parametric statistical tests, as the Shapiro–Wilk test indicated a lack of normality for some subscales. Mann–Whitney U test was employed for the child self-report, which included two age groups, while the Kruskal–Wallis test was used for multiple comparisons for the parent proxy reports, which included three age groups.

## Results

### Demographic and clinical characteristics

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.

### Feasibility

The item-level missing response rate for the child self-report was 4.86%. Among twenty-two children aged 8–12 years, six were unable to complete the self-report questionnaire due to intellectual or physical disabilities. Similarly, among fifteen children aged 13–18 years, four could not complete the self-report

**Table 1.Demographic data**

| Variable | Mean | SD | Range |
|---|---|---|---|
| Age of onset [years] | 3.58 | 2.83 | 0–10 |
| Age at time of the evaluation [years] | 12.52 | 2.97 | 8–19 |
| **Variable** | **N** | **[%]** | |
| **Age distribution** | | | |
| 5–7 | 7 | 20.6% | |
| 8–12 | 16 | 47.1% | |
| 13–18 | 11 | 32.4% | |
| Non-ambulatory patients | 10 | 32.3% | |
| On steroid treatment | 16 | 51.6% | |
| Positive family history | 31 | 72.1% | |

SD — standard deviation

**Table 2. Internal consistency of the Greek version of the PedsQL™ 3.0 Duchenne muscular dystrophy module**

| Scale* | N | Cronbach's α | Mean | SD | Floor effect [%] | Ceiling effect [%] |
|---|---|---|---|---|---|---|
| **Child self-report** | | | | | | |
| Total (18) | 27 | 0.80 | 69.12 | 17.32 | 0 | 3.7 |
| Daily activities (5) | 27 | 0.72 | 69.07 | 23.12 | 0 | 11.1 |
| Treatment barriers (4) | 27 | 0.80 | 72.20 | 22.41 | 0 | 14.8 |
| Worry (6) | 27 | 0.66 | 70.17 | 18.11 | 0 | 3.7 |
| Communication (3) | 27 | 0.73 | 63.27 | 30.33 | 7.4 | 14.8 |
| **Variable** | | | | | | |
| Total (18) | 43 | 0.89 | 65,05 | 22.72 | 0 | 4.7 |
| Daily activities (5) | 43 | 0.87 | 56.16 | 32.42 | 7 | 14 |
| Treatment barriers (4) | 43 | 0.56 | 70.54 | 26.20 | 4.7 | 20.9 |
| Worry (6) | 43 | 0.87 | 65.79 | 24.96 | 0 | 11,6 |
| Total (18) | 43 | 0.89 | 65.05 | 22.72 | 0 | 4.7 |

*Number of items per subscale; SD — standard deviation

for the same reasons. Consequently, the parent report was completed for these ten patients. The item-level missing data rate for the parent proxy report questionnaire was 2.58%.

## Reliability

### *Internal consistency reliability*

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 $\alpha = 0.8$, parent report total score $\alpha = 0.89$) (Table 2). A ceiling effect was observed in the parent proxy response in the "Communication" subscale.

### *Item-subscale correlations*

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 (the * mark indicates correlation statistically significant at 0.01 level and ** at 0.05 level). Table 3 presents the Spearman correlation coefficient between the items and the subscale scores.

### *Test-retest reliability*

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). 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 (Table 4).

## Parent–child agreement

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 5).

## Construct validity

Construct validity was evaluated between the ambulatory and non-ambulatory patients, in addition to evaluation between patients receiving steroids and those who were not, using the independent samples t-test to compare the scores obtained in the initial evaluation (Tables 6–8). The means for all subscales were higher for ambulatory patients and for patients not receiving steroids. The total score of all subscales demonstrated a significant correlation with ambulatory status in both the child self-report and the parent proxy report. These findings align with the expected outcomes, as non-ambulatory patients tend to exhibit

**Table 3. Spearman correlation coefficient between the items and the subscale scores of the Greek version of the PedsQL™ 3.0 Duchenne muscular dystrophy module**

| Subscales/items per subscale | Child | | | | Parent | | | |
|---|---|---|---|---|---|---|---|---|
| | A*** | T*** | W*** | C*** | A*** | T*** | W*** | C*** |
| **Daily activities** | | | | | | | | |
| Trouble eating with a fork and knife | 0.570** | 0.607** | 0.299 | 0.107 | 0.800** | 0.557** | 0.376* | 0.438** |
| Hard to write or draw with a pen or pencil | 0.532** | 0.251 | −0.015 | 0.128 | 0.780** | 0.502** | 0.310* | 0.396** |
| Hard to put on my clothes | 0.800** | 0.551** | 0.396* | 0.570** | 0.861** | 0.511** | 0.531** | 0.557** |
| Hard to use the toilet without help | 0.823** | 0.473* | 0.417* | 0.488** | 0.785** | 0.431** | 0.604** | 0.508** |
| Need more time than others to complete tasks | 0.462* | 0.099 | 0.327 | 0.437* | 0.783** | 0.375* | 0.493** | 0.545** |
| **Treatment** | | | | | | | | |
| Hard to take medicines | 0.376 | 0.576** | 0.192 | −0,18 | 0.399* | 0.481** | 0.088 | 0.215 |
| Physical therapy or daily stretching hurts | 0.460* | 0.799** | 0.178 | −0.174 | 0.383* | 0.641** | 0.152 | −0.008 |
| Hard to be responsible for my medicines or physical therapy | 0.506** | 0.863** | 0.437* | 0.326 | 0.231 | 0.718** | 0.2 | 0.285 |
| Hard to manage my muscle problem | 0.618** | 0.744** | 0.610** | 0.423* | 0.530** | 0.749** | 0.457** | 0.366* |
| **Worry** | | | | | | | | |
| Worry about my muscle problem | 0.224 | −0.03 | 0.722** | 0.245 | 0.448** | 0.373* | 0.795** | 0.254 |
| Worry whether or not my medicines are working | 0.105 | 0.14 | 0.641** | 0.128 | 0.421** | 0.348* | 0.707** | 0.216 |
| Worry about my family | 0.151 | 0.167 | 0.686** | 0.276 | 0.337* | 0.185 | 0.724** | 0.394** |
| Worry about needing help from others | 0.561** | 0.515** | 0.674** | 0.501** | 0.595** | 0.472** | 0.803** | 0.397** |
| Worry about not being accepted by others | 0.457* | 0.364 | 0.519** | 0.35 | 0.521** | 0.344* | 0.837** | 0.311* |
| Worry about being treated differently from others my age | 0.355 | 0.497* | 0.562** | 0.139 | 0.486** | 0.365* | 0.864** | 0.321* |
| **Communication** | | | | | | | | |
| Hard for me to tell the doctors and nurses how I feel | 0.492** | 0.245 | 0.375 | 0.769** | 0.576** | 0.311* | 0.309* | 0.836** |
| Hard for me to ask the doctors and nurses questions | 0.484* | 0.092 | 0.434* | 0.793** | 0.581** | 0.3 | 0.384* | 0.873** |
| Hard for me to explain my muscle problem to other people | 0.387* | 0.101 | 0.112 | 0.741** | 0.477** | 0.484** | 0.410** | 0.728** |

*Correlation statistically significant at 0.01 level
**Correlation statistically significant at 0.05 level
***A — daily activities; T — treatment; W — worry; C — communication

lower scores for HRQoL compared to their ambulant counterparts [3, 5]. The results of the independent t-tests concerning ambulation are presented in Table 6. Regarding the results for patients undergoing steroid treatment (Table 7), these were expected to be in reverse. It is recognized that the introduction of steroids in the management of DMD has significantly delayed disease milestones, such as the loss of ambulation and upper trunk use, to older ages [5].

To interpret the preceding results, a further analysis was conducted, including age-group stratification, as shown in Table 8. In the 5–7 age group, the total scores for both steroid and non-steroid patients were roughly equivalent. However, the daily activities and treatment barriers subscale scores were significantly higher for the steroid group, whereas the worry and communication subscale scores were not. For the 13–18 age group, the steroid group had higher

**Table 4. 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) |

*Number of items per subscale; CI — confidence interval

**Table 5. 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) |

*Number of items per subscale; CI — confidence interval

total and subscale scores in the parent proxy reports, a pattern not seen in the child self-reports. Conversely, in the 8–12 age group, the steroid group exhibited lower scores in both child and parent proxy reports.

### Differences in PedsQL™ 3.0 Duchenne muscular dystrophy scores between age groups

The mean scores on the PedsQL™ 3.0 DMD by age group are displayed in Table 9. In the child self-report questionnaires, total score and mean scores in all subscales apart from "Worry", were higher for the group of 8–12 years than for the group of 13–18 years old. Shapiro–Wilk's test of normality indicated that the subscale communication did not follow the normal distribution. Using the independent samples Mann–Whitney U test, the distributions are the same across age groups.

Parents of the 13–18-year-old group reported significantly lower scores in both the total and all subscales scores than the parents of the 8–12-year-old group. Parents of the 5–7-year-old group reported higher scores for the total score and all the subscales apart from daily activities in comparison to the parents' reports of the 8–12-year-old group. The results of the Shapiro–Wilk test of normality indicated that the three subscales: daily activities, treatment, and communication did not follow the normal distribution. The Kruskal–Wallis test for independent samples revealed that the distributions were identical across all age groups.

The Greek version of the PedsQL 4.0 Generic Core questionnaire was used to examine the construct validity of the Greek version of PedsQL™ 3.0 DMD [23]. The PedsQL 4.0 Generic Core questionnaire is a generic HRQoL instrument that can discriminate among children of the same age group, a pediatric subpopulation with a chronic disease. The Greek version of it has been validated by Gkoltsiou et.al [15]. Prior research has demonstrated that patients presenting with disease-specific symptoms or an impaired health status exhibited markedly diminished PedsQL 4.0 Generic Core scores in comparison to healthy individuals. The Greek version of the PedsQL™ 3.0 DMD module satisfied the construct validity criteria (Table 10).

## Discussion

To facilitate multinational collaboration, it is essential that validated HRQoL questionnaires are available in multiple languages. The measurement of HRQoL provides valuable information that can be employed in the assessment of treatment or the identification of the most efficacious medicines. The PedsQL™ 3.0 DMD module has now been validated by Uzark et al. [24] and Thongsing et al. [3] in patients with DMD and has been employed for the measurement of health-related quality of life in pediatric patients with DMD and the documentation of the factors affecting it. The present study confirms that the Greek version of the PedsQL™ 3.0 DMD module is a valid and reliable instrument for evaluating HRQoL in the pediatric DMD population. Nevertheless, some children were unable to complete the questionnaires due to the coexistence of an intellectual disability or accelerated deterioration of their health status. No floor effects were identified for any of the subscales. However, a ceiling effect was observed for the "Communication" subscale of the parent proxy report, indicating that these parents had established effective communication with their children.

Cronbach's coefficient alpha exceeded 0.7 for internal consistency in all subscales, in both the child self--report and the parent proxy report demonstrating an acceptable reliability as well as for group comparisons.

**Table 6. Construct validity of the Greek version of the PedsQL™ 3.0 Duchenne muscular dystrophy module, known-groups method comparing ambulatory and non-ambulatory patients**

| Scale* | Ambulatory | | Non-ambulatory | | | | 95% CI | |
|---|---|---|---|---|---|---|---|---|
| Child self-report | Mean | SD | Mean | SD | P | Difference | Low | Upper |
| Total (18) | 76.29 | 13.27 | 56.91 | 17.04 | 0.00 | 19.38 | 7.28 | 31.48 |
| Daily activities (5) | 80 | 14.14 | 50.5 | 24.09 | 0.00 | 29.50 | 11.41 | 47.59 |
| Treatment barriers (4) | 81.77 | 19.57 | 56.88 | 18.27 | 0.00 | 24.9 | 9.01 | 40.78 |
| Worry (6) | 71.25 | 21.19 | 68.33 | 11.98 | 0.69 | 2.92 | −12.20 | 18.03 |
| Communication (3) | 71.08 | 24.85 | 50 | 35.36 | 0.08 | 21.08 | −2.78 | 44.94 |
| Parent proxy report | | | | | | | | |
| Total (18) | 70.64 | 20.75 | 52.15 | 22.54 | 0.01 | 18.49 | 4.21 | 32.77 |
| Daily activities (5) | 63.67 | 31.38 | 38.85 | 28.88 | 0.02 | 24.82 | 4.25 | 45.39 |
| Treatment barriers (4) | 76.08 | 23,33 | 58.17 | 28.91 | 0,04 | 17.9 | 0.95 | 34.86 |
| Worry (6) | 72.92 | 21.68 | 49.36 | 24.99 | 0.00 | 23.56 | 8.34 | 38.78 |
| Communication (3) | 70 | 31.07 | 69.23 | 34.26 | 0.94 | 0.77 | −20.71 | 22.25 |
| Child self-report | | | | | | | | |

*Number of items per subscale; CI — confidence interval; SD — standard deviation

**Table 7. Construct validity of the Greek version of the PedsQL™ 3.0 Duchenne muscular dystrophy module, known-groups method comparing patients receiving steroids to patients not receiving steroids**

| Scale* | Steroid | | Non-steroid | | | | 95% CI | |
|---|---|---|---|---|---|---|---|---|
| | Mean | SD | Mean | SD | P | Difference | Low | Upper |
| Child self-report | | | | | | | | |
| Total (18) | 66.72 | 15.46 | 72.6 | 19.96 | 0.4 | −5.88 | −19.92 | 8.16 |
| Daily activities (5) | 67.5 | 21.53 | 71.36 | 26.18 | 0.68 | −3.86 | −22.82 | 15.09 |
| Treatment barriers (4) | 72.79 | 19.68 | 71.25 | 27.35 | 0.87 | 1.54 | −17.49 | 20.56 |
| Worry (6) | 68.57 | 17.31 | 72.5 | 19.84 | 0.59 | −3.93 | −18.75 | 10.88 |
| Communication (3) | 56.25 | 31.7 | 73.48 | 26.3 | 0.15 | −17.23 | −41.16 | 6.69 |
| Parent proxy report | | | | | | | | |
| Total (18) | 64.29 | 19.76 | 65.77 | 25.68 | 0.83 | −1.48 | −15.64 | 12.68 |
| Daily activities (5) | 59.17 | 26.24 | 53.3 | 37.81 | 0.56 | 5.87 | −14.15 | 25.89 |
| Treatment barriers (4) | 69.64 | 21.77 | 71.43 | 30.53 | 0.83 | −1.79 | −18.32 | 14.75 |
| Worry (6) | 60.67 | 22.76 | 70.68 | 26.47 | 0.19 | −10.01 | −25.25 | 5.23 |
| Communication (3) | 71.43 | 32.34 | 68.18 | 31.67 | 0.74 | 3.25 | −16.47 | 22.96 |

*Number of items per subscale; CI — confidence interval; SD — standard deviation

Test-retest responses demonstrated good to excellent agreement in daily activities and worry for both child-self report and parent/caregiver proxy report as well as in total score, which suggest that the PedsQL™ 3.0 DMD module Greek version is stable and reliable for child-self report and parent proxy report in total score, daily activities and worry. Similar findings were noted in the Thongsing et al. [3] study for the child self-report while on the parent proxy report, all scores varied between excellent to very good. In both the child-self report and parent proxy report in the subscale treatment barriers and communication showed poor to fair agreement. This finding is similar to the Uzark et al. [24] study, in which the agreement between parents and their children was in general poor.

## Parent–child agreement

A notable degree of consensus has been achieved in the parent–child agreement correlation with an ICC of 0.8 except for the worry and communication subscales which are moderate. This may be attributed to the possibility that parents may overestimate their

**Table 8. Construct validity of the Greek version of the PedsQL™ 3.0 Duchenne muscular dystrophy module, known-groups method comparing patients receiving steroids to patients not receiving steroids by age group**

| Scale* | Children's age group | | | | | | | | | | | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|
| | 5–7 | | | | 8–12 | | | | 13–18 | | | |
| | Steroid | | Non-steroid | | Steroid | | Non-steroid | | Steroid | | Non-steroid | |
| | Mean | SD | Mean | SD | Mean | SD | Mean | SD | Mean | SD | Mean | SD |
| **Child self-report** | | | | | | | | | | | | |
| Total (18) | | | | | 68.04 | 11.14 | 75 | 14.03 | 62.77 | 26.69 | 71.23 | 23.66 |
| Daily activities (5) | | | | | 69.58 | 17.64 | 85 | 12.25 | 61.25 | 33.26 | 63.57 | 29.54 |
| Treatment barriers (4) | | | | | 74.65 | 21.79 | 76.56 | 27.18 | 67.19 | 11.83 | 67.71 | 29.43 |
| Worry (6) | | | | | 66.08 | 18.5 | 61.88 | 18.49 | 76.04 | 11.97 | 78.57 | 19.16 |
| **Parent proxy report** | | | | | | | | | | | | |
| Total (18) | 73.61 | 29.46 | 74.44 | 21.84 | 63.62 | 16.96 | 75.34 | 22.41 | 62.15 | 30.1 | 54.74 | 27.27 |
| Daily activities (5) | 60 | 42.43 | 53 | 37.52 | 59.5 | 23.34 | 69.64 | 31.17 | 57.5 | 37.97 | 42 | 41.38 |
| Treatment barriers (4) | 84.38 | 22.10 | 78.75 | 24.84 | 66.67 | 22.49 | 83.33 | 14.61 | 73.44 | 20.65 | 60.63 | 37.74 |
| Worry (6) | 77.08 | 32.41 | 84.17 | 17.03 | 60.78 | 20.4 | 75.12 | 22.47 | 52.08 | 29.76 | 60.83 | 30.75 |
| Communication (3) | 75 | 11.79 | 85 | 18.07 | 70 | 30.67 | 73.81 | 30.59 | 75 | 50 | 55.83 | 34.93 |

*Number of items per subscale; SD — standard deviation

**Table 9. Comparison of the Greek version of the PedsQL™ 3.0 Duchenne muscular dystrophy module scores by age group**

| Scale* | Children's age group | | | | | |
|---|---|---|---|---|---|---|
| | 5–7 | | 8–12 | | 13–18 | |
| | Mean | SD | Mean | SD | Mean | SD |
| **Child self-report** | | | (N = 16) | | (N = 11) | |
| Total (18) | | | 69.78 | 11.83 | 68.15 | 23.83 |
| Daily activities (5) | | | 73.44 | 17.49 | 62.73 | 29.27 |
| Treatment barriers (4) | | | 75.13 | 22.29 | 67.5 | 22.97 |
| Worry (6) | | | 65.03 | 17.97 | 77.65 | 16.27 |
| Communication (3) | | | 65.63 | 22.75 | 59.85 | 39.93 |
| **Parent proxy report** | (N = 7) | | (N = 22) | | (N = 14) | |
| Total (18) | 74.21 | 21.51 | 67.35 | 19.14 | 56.86 | 27.13 |
| Daily activities (5) | 55 | 35.36 | 62.73 | 25.77 | 46.43 | 39.63 |
| Treatment barriers (4) | 80.36 | 22.37 | 71.43 | 21.61 | 64.29 | 33.47 |
| Worry (6) | 82.14 | 19.5 | 65.34 | 21.64 | 58.33 | 29.6 |
| Communication (3) | 82.14 | 16.27 | 71.21 | 29.96 | 61.31 | 38.76 |

*Number of items per subscale; SD — standard deviation

children's feelings of worry or may even express their own feelings of worry regarding the condition of their children. Moreover, the level of concordance between child and parent responses is higher in the age group 8–12 years than in the age group 13–18 years. It is evident that the parents' scores are considerably lower than those of their children. This discrepancy may be attributed to two factors. Firstly, parents may be more aware of the progression of the disease, which could result in elevated anxiety levels. Secondly, older children who have been dependent on a wheelchair for years may have become more accustomed to it and therefore be better equipped to cope with it than their parents. The findings of the present study align with those of the Thongsing et al. [3] study, in which the Thai version of the PedsQL 3.0 DMD questionnaire

**Table 10. Pearson's product-moment correlations among the Greek version of the PedsQL™ Scales for Duchenne muscular dystrophy**

| Scale | Total score DMD | Daily activities | Treatment | Worry | Communication | Total score GC | Physical health summary score | Psychosocial score | Emotional score | Social score | School score |
|---|---|---|---|---|---|---|---|---|---|---|---|
| **Total score DMD** | 1 | 0.900** | 0.735** | 0.762** | 0.709** | 0.782** | 0.690** | 0.699** | 0.494** | 0.739** | 0.564** |
| **Daily activities** | 0.900** | 1 | 0.593** | 0.571** | 0.569** | 0.760** | 0.747** | 0.594** | 0.415** | 0.696** | 0.437** |
| **Treatment** | 0.735** | 0.593** | 1 | 0.429** | 0.316** | 0.582** | 0.436** | 0.527** | 0.266* | 0.544** | 0.533** |
| **Worry** | 0.762** | 0.571** | 0.429** | 1 | 0.427** | 0.672** | 0.570** | 0.701** | 0.595** | 0.662** | 0.440** |
| **Communication** | 0.709** | 0.569** | 0.316** | 0.427** | 1 | 0.414** | 0.376** | 0.405** | 0.224 | 0.420** | 0.429** |
| **Total score GC** | 0.782** | 0.760** | 0.582** | 0.672** | 0.414** | 1 | 0.825** | 0.826** | 0.653** | 0.837** | 0.567** |
| **Physical health summary score** | 0.690** | 0.747** | 0.436** | 0.570** | 0.376** | 0.825** | 1 | 0.444** | 0.420** | 0.570** | 0.145 |
| **Psychosocial score** | 0.699** | 0.594** | 0.527** | 0.701** | 0.405** | 0.826** | 0.444** | 1 | 0.762** | 0.856** | 0.804** |
| **Emotional score** | 0.494** | 0.415** | 0.266* | 0.595** | 0.224 | 0.653** | 0.420** | 0.762** | 1 | 0.521** | 0.374** |
| **Social score** | 0.739** | 0.696** | 0.544** | 0.662** | 0.420** | 0.837** | 0.570** | 0.856** | 0.521** | 1 | 0.603** |
| **School score** | 0.564** | 0.437** | 0.533** | 0.440** | 0.429** | 0.567** | 0.145 | 0.804** | 0.374** | 0.603** | 1 |

*Correlation statistically significant at 0.01 level (2-tailed); **correlation statistically significant at 0.05 level (2-tailed); DMD — Duchenne muscular dystrophy; GC — generic core

was validated. In the aforementioned study, there was a high level of agreement, classified as good to excellent, in the total score and the subscales "Daily Activities" and "Treatment Barriers". However, moderate agreement was observed for the subscales "Worry" and "Communication" in the Uzark et al. [24] study. Additionally, poor to moderate agreement was reported between children with DMD and their parents, indicating that the disparate perspectives warrant further investigation. It has been observed that there is often less than optimal agreement between children's self-reports and parents' proxy reports in the context of health-related QoL questionnaires for children with and without chronic illness.

## Known-group validity

A known-group validity assessment was conducted using the known-group method, with comparisons made between ambulatory and non-ambulatory groups, and between steroid and non-steroid groups. These comparisons were used to assess construct validity, and the results demonstrated that the instrument is capable of discriminating between the groups. In these assessments, the anticipated difference in favor of the ambulatory group was observed, which suggests that ambulatory patients report higher HRQoL. The results demonstrated that all subscale scores and the total scores of ambulatory patients and their parents were higher than the respective scores of non-ambulatory patients and their parents, thereby supporting the hypothesis that the loss of ambulation is associated with a reduction in quality of life. The results of both studies conducted by Thongsing et al. [3] and Uzark et al. [24] yielded comparable outcomes.

Furthermore, it was observed that non-steroid users reported improved HRQoL which is somewhat incongruous with the actual situation. This is because the introduction of steroid use in the therapeutic management of Duchenne muscular dystrophy has led to a notable benefit, namely an increase in the mean age at which ambulation is lost. To address this apparent paradox, a further assessment was conducted across age groups. The results of the age-group analysis yielded disparate outcomes. In the 5–7 age group, in which all the boys are still ambulant, the total score indicates a slight advantage for the non-steroid group. However, in the subscales "Daily Activities" and "Treatment Barriers", the group receiving steroids exhibited higher scores than the non-steroid group. Conversely, the subscales "Worry" and "Communication" indicate a slight advantage for the non-steroid group. In the 8–12 age group, the majority of scores indicated a preference for the non-steroid group. In the 13–18 age group, there is a discrepancy between

the responses of children and parents. In the case of children, the scores indicate a preference for non-steroid users, whereas in the case of parents, the scores indicate a preference for steroid users. The results in the 5–7 group are indicative of the psychological disposition of parents of newly diagnosed children, at least in the context of Greece.

On the one hand, there are those who are firmly convinced of the benefits of steroid administration, even in the pro-symptomatic phase of the disease. This group may exhibit an excessive level of concern regarding the long-term implications of the disease. On the other hand, some will only administer steroids to their children when the symptoms are well established. However, they tend to adopt a more optimistic outlook as long as they observe their child ambulating. In order to interpret the results in the 8–12 age group, it is essential to conduct a qualitative assessment of the sample. As DMD is a heterogeneous disease, the combination of the different ambulatory and yes/no steroid statuses of each participant is a significant factor. This could explain why non-steroid users report better HRQoL. For the oldest group of 13–18, the contradictory views expressed by parents and children are in line with the level of general disagreement as was highlighted by the child-parent agreement test. Former studies show similar differences in favor of the non-steroid groups [25, 26].

The mean total score was 69.12 on the child self-report and 65.05 on the parent proxy report, with all subscales scores higher in the present study than that reported by Wei et al. [26] in Canada and Thongsing et al. [3] in Thailand. The subscale scores in the present study are either higher or comparable to those in the Canadian study. A comparison of the subscales cores with those of the Uzark et al. [24] study (Ohio, USA) reveals a mixed out come. In certain subscales, the scores are higher in the present study, whereas in others, the scores are higher in the American study. It is noteworthy that the present study exhibits the highest score in the daily activities subscale when compared to the aforementioned three studies in the child self-report, a finding that is not corroborated by the respective parent proxy reports. In the latter, the highest score across all four studies is observed in the American study [24].

The results of the present study demonstrated markedly elevated levels of agreement between parents and children in comparison to the findings of the American study [24]. Additionally, notable similarities were observed between the present results and those of the Thai study in the same field [3]. In the present study, it appears that children with DMD assess their HRQoL at a higher level than their parents do on their behalf, a pattern that is also evident in the Canadian study [26]. In the other two studies, there is a discrepancy between parents' estimation of their children's HRQoL and their children's self-report on approximately half of the subscales. The four studies exhibit several demographic characteristics in common, including the mean age of the patients (11.7 years in the present study and the Thai study, 10.7 years in the Canadian study and 10.4 years in the USA study) and in terms of ambulatory status (57.7% in the present study, 60.7% in the Thai study, 72.4% in the Canadian study and 58% in the USA study). The majority of children exhibit a positive outlook on their HRQoL, which is shaped by their limited awareness of the disease and its progression. Conversely, parents tend to adopt a more pragmatic and concerned stance. Furthermore, the present study examined the criterion-related validity of the PedsQL™ 3.0 DMD module in conjunction with another validated questionnaire, the PedsQL 4.0 Generic Core [15, 19] which was administered in the same population. It should be noted that the present study is subject to certain limitations, including the relatively small sample size, which was drawn from only two of the four pediatric neuromuscular clinics.

## 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.

## Article information and declarations

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### *Data availability statement*

Data will be made available upon reasonable request.

### *Ethics statement*

The Ethics Committee of the University of Peloponnese approved the health-related quality of life study; the Scientific Boards of AHEPA Hospital of Thessaloniki and the Hospital of Patras approved also the health-related quality of life study. All participants have provided their written informed consent.

### *Author contributions*

EK, AK, and EC contributed to the study conception and design. Material preparation, data collection, and analysis were performed by EK, and statistical analysis by GM. The first draft of the manuscript was written by EK and all authors commented on previous versions of the manuscript. All authors read and approved the final manuscript.

### *Conflict of interest*

All authors declare no conflict of interest.

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### *Supplementary material*

None.

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