Validation of the Pediatric Quality of Life Inventory™, Neuromuscular Module, version 3.0 in Spanish for Argentina

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ABSTRACT

Objective: To assess the psychometric properties of the Pediatric Quality of Life Inventory™ (PedsQL™ 3.0), Neuromuscular Module, version in Spanish for Argentina, for children aged 2-18 years with neuromuscular disease.

Population and methods: Observational, analytical, prospective validation study conducted in Hospital Garrahan between March 19th, 2019 and March 9th, 2020. The retest questionnaire was administered 10-15 days later to validate it among patients who reported a stable condition.

Results: A total of 185 children and their parents participated. In terms of the questionnaire's feasibility, its content was easily understood by participants. Its reliability was acceptable, with an internal consistency of 0.82 among children and 0.87 among parents and a retest intraclass correlation coefficient of 0.70 among children and 0.82 among parents. In relation to the construct validity, 8 of the 11 hypotheses established (72.7 %) were confirmed.

Conclusion: The questionnaire's psychometric properties were validated.

Key words: *quality of life, neuromuscular disease, pediatrics, validation study.*

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ACRONYMS AND ABBREVIATIONS

95 % CI: 95 % confidence interval.
DMD: Duchenne muscular dystrophy.
GROC: global rating of change.
HRQoL: health-related quality of life.
IC: informed consent.
ICC: intraclass correlation coefficient.
IQR: interquartile range.
MDC: minimal detectable change.
NMD: neuromuscular disease.
OPPCf: overall perception of patient condition as per family member.
OPPCk: overall perception of patient condition as per kinesiologist.
PedsQL™ 4.0: Pediatric Quality of Life Inventory™ version 4.0.
PedsQL™ NM 3.0: PedsQL™ Neuromuscular Module version 3.0.
SD: standard deviation.
SEM: standard error of measurement.
SMA: spinal muscular atrophy.
T0: baseline time for questionnaire administration.
T1: second questionnaire administration or retest.
VAS: visual analogue scale.

INTRODUCTION

Neuromuscular diseases (NMDs) make up a heterogeneous group of disorders of the peripheral nerves and muscles. They are characterized by variable degrees of muscle weakness, most are of genetic origin, present with a chronic and progressive course, and cause higher or lower levels of disability.[1] The concept of health-related quality of life (HRQoL) refers to the impact of a disease on a patient's opinion of their own well-being, considering the individual perception of their physical and psychosocial limitations.[2]

From a comprehensive perspective, the assessment of children with chronic conditions includes the use of generic and specific instruments to assess their HRQoL. One of those instruments available is the generic questionnaire called Pediatric Quality of Life Inventory™, version 4.0 (PedsQL™ 4.0), which has been validated for the Argentine pediatric population.[3] The PedsQL™ Neuromuscular Module version 3.0 (PedsQL™ NM 3.0) is also available to assess specifically children with such diseases. Several studies demonstrated that this questionnaire is reliable to measure HRQoL in patients with NMD aged 2-18 years.[4-11]

Hospital Garrahan caters for more than 400 children from across the country who have genetically determined NMD as part of a multidisciplinary program that was started in 2006.

More recently, the importance of considering HRQoL as an outcome variable in research studies or in the clinical follow-up of children with NMD has been recognized.

Given the relevance of having a specific, validated instrument to measure HRQoL in our country, the objective of this study was to assess the psychometric properties of the PedsQL™ NMv3.0, version in Spanish for Argentina, in children aged 2-18 years with NMD.

MATERIALS AND METHODS

This was an observational, analytical, and prospective study to assess the psychometric properties of the PedsQL™ NM 3.0, version in Spanish for Argentina, translated and culturally adapted to the Argentine population by MAPI Research Trust, who authorized its use.

This study was approved by the Research Ethics Review Committee of Hospital Garrahan on March 19th, 2019 (no. 1147). Results are reported based on the Consensus-based Standards for the Selection of Health Measurement (COSMIN).[12,13] The study was conducted in accordance with the ethical and legal regulations governing personal data (Law 25326).

Argentine children with NMD aged 2-18 years and their parents were included after a prior visit to their physical therapist or kinesiologist and the signature of the informed consent (IC) or assent, as applicable.

Patients with other chronic conditions, or who were experiencing an acute event, or had end-stage disease, or did not understand the questions in the questionnaire for their age or a younger group were excluded.

Questionnaires with more than 50 % of unanswered questions were left out.[14,15]

The PedsQL™ NM 3.0 consists in self-administered surveys for pre-adolescents (8-12 years old) and adolescents (13-18 years old) to assess three domains. The first domain is called "About my neuromuscular disease" and has 17 questions about the problems caused by the disease for the child and/or their family. The 3 questions in the domain called "Communication" ask about communication difficulties. The 5 questions in the domain called "About our family resources" address the problems caused by NMD on family functioning. The version aimed at young children (5-7 years old) is administered by an adult and includes only the first domain. The section for parents includes the 3 domains. In the case of toddlers (2-4 years old), only parents complete the questionnaire.

For each item, children and parents answer to what extent it was a problem in the past months, based on a 5-point Likert-like scale, where 0 means "never" and 4, "almost always." The score is reversed and turned into a 0-100 scale, where 0 = 100, 1 = 75, 2 = 50, 3 = 25, and 4 = 0. The version aimed at children aged 5-7 years includes 3 answer options based on emoticons (not at all, sometimes, always). The domain and total scores are estimated by dividing the sum of the scores of all items by the total number of answered items. A higher score suggests less problems.

At baseline, demographic characteristics, disease data, and the level of education of the caregiver completing the questionnaire were collected.

Construct validity refers to the extent to which the questionnaire's score correlates to other measurements. Eleven hypotheses were established with different levels of correlation between the PedsQL™ NM 3.0 and its "About my neuromuscular disease" domain and the following instruments:

PedsQL™ 4.0: Generic questionnaire divided by age groups and administered and scored similarly to the PedsQL™ NM 3.0.[3]

Overall perception of patient condition as per family member (OPPCf): This corresponds to the caregiver's assessment of the child's status in the past month based on a 0-10 visual analogue scale (VAS), where 0 means "very bad" and 10, "very good".[3]

Overall perception of patient condition as per kinesiologist (OPPCk): Modified version of the instrument used by Roizen M.[3] Based on a clinical

and functional assessment, 2 kinesiologists established how NMD affected the patient since their most recent visit using a VAS, where 0 means "not affected at all" and 10, "affected a lot".

Vignos scale: Functional assessment of lower limbs made up of 10 items, with a score of 1 for patients who are able to walk and climb stairs without assistance and 10 for those who are bed-bound.[1,16]

Brooke scale: Functional classification of upper extremity movement into 6 levels, where 1 means that the patient can abduct the arms in a full circle until the touch above the head and 6, that the patient has no useful function of the hands.[1,16]

A pilot test was done in 30 subjects as training for operators on how to administer the instruments and assess the level of agreement in terms of OPPCk between the kinesiologists based on the intraclass correlation coefficient (ICC) and the corresponding 95 % confidence intervals (CIs). The result was acceptable, with an ICC of 0.82 (0.66-0.91).[17,18]

The study was conducted in two moments. During the visit in person or time zero (T0); after the IC was signed and demographic data were collected, the PedsQL™ NM 3.0 was administered simultaneously to children and parents. The other instruments were administered subsequently.

Time one (T1) or retest consisted in the administration of the PedsQL™ NM 3.0 10-15 days after T0 only to patients who had a stable condition, as per the Global Rating of Change (GROC) based on a 3-category ordinal scale.[19-21] A family member was asked the following: "How is your child doing from the last assessment: better, the same or worse?" Subjects who answered "the same" were considered stable. T1 was performed via a telephone messaging system to reduce the number of visits to the hospital.[22]

In relation to the questionnaire's feasibility, the content validity was assessed in the first 32 subjects who were included and their caregivers using questions about its content and writing, the need for assistance, and the time taken to complete it. It was established that at least 80 % of participants had to complete the questionnaire for it to be considered valid and representative of the target population.[23-25] For the assessment of interpretability, the floor and ceiling effects were considered if more than 15 % of all participants obtained the minimum or maximum value in the questionnaire's items during T0.[26]

In relation to the questionnaire's reliability, the internal consistency was assessed using Cronbach's alpha for the PedsQL™ NM 3.0 during T0. A value between 0.7 and 0.95 was deemed acceptable.[26,27] A Cronbach's alpha value >0.7 allows to compare groups, but for individual use, it should be > 0.9.

For the test-retest reliability, the group of stable patients was considered based on the GROC during T1.[22] The ICC and its respective 95 % CIs were estimated.[12] A two-way random effects model (ICC: 2.1) was used. An ICC ≥ 0.70 was deemed an acceptable reliability.[26]

The error of measurement was described as the standard error of measurement (SEM) ($SD \times \sqrt{1 - ICC}$)[27] and as minimal detectable change (MDC) ($SEM \times 1.96 \times \sqrt{2}$).[28-31]

The differences between each measurement between T0 and T1 versus the mean value of both measurements were described using Bland Altman plots, with the corresponding 95 % CIs.[32-34]

To assess the construct validity, Pearson's or Spearman's correlation coefficient was used, as applicable. Coefficients > 0.50, between 0.50 and 0.30, and < 0.30 were considered strong, moderate, and poor, respectively.[35] The construct validity was considered acceptable if there was an agreement with at least 75 % of the hypotheses established.[26] Such property was also assessed by comparing known groups. Comparisons were done using Student's t test for independent samples or the Mann-Whitney U test, as applicable. Based on the investigators' clinical experience and the bibliography,[4-6] different hypotheses were proposed to assess the differences by age, ambulation, and ventilatory support requirement. The hypotheses are reported with their corresponding testing in the results section.

Continuous outcome measures with a normal distribution were reported as mean and standard deviation (SD) or as median and interquartile range (IQR). Categorical outcome measures were reported as number and percentage. To establish the sample distribution, the Shapiro-Wilk or Kolmogorov-Smirnov test was used, as applicable. A value of $p < 0.05$ was considered significant. Data were analyzed using the IBM SPSS Macintosh software, version 24.0 (IBM Corp., Armonk, NY, USA).

RESULTS

Between March 19th, 2019 and March 9th, 2020, 335 participants were recruited; of these, 71 did not meet the inclusion criteria and 77 were excluded. A total of 187 children and their family were assessed.

Two children aged 2-4 years, who had undergone a tracheostomy and were receiving continuous ventilatory support and were therefore unable to communicate, were excluded because their parents could not report their status and complete the questionnaire. A total of 24 (13.0 %) participants did not take part in the T1 assessment (*Figure 1*). This group was included in the assessment of all psychometric properties except for the longitudinal analyses.

Table 1 describes the characteristics of participants.

The feasibility analysis included 32 subjects. Six of them were females and their median age was 10 years (IQR: 5-14). The mean time to complete the questionnaire was 4 minutes and 21 seconds (SD: 1 minute and 28 seconds). In general, the content of the questionnaire was easily understood by participants. Only 9 required assistance and had the questionnaire completed by a family member because of their age.

In relation to the impression caused by the questionnaire, 17 children considered it asked about situations they experienced on a daily basis, and 29 family members considered it encompassed the most important aspects of their child's condition. Also, 21 children indicated that they understood the questions; and 30 family members, that they were well written.

In relation to internal consistency, Cronbach's alpha was acceptable, with a value of 0.82 and 0.87 for children and family members, respectively. *Table 2* shows the coefficients by domain for the different age groups during T0.

The test-retest reliability assessment included 161 participants (95.8 %), who reported having a stable condition. The median number of days between T0 and T1 was 15 (IQR: 13-19). The ICC (2.1) for the child version was 0.704 (95 % CI: 0.670-0.780) and, for the parent version, 0.829 (95 % CI: 0.773-0.871); all these values were considered acceptable. *Table 2* reports the ICCs

Figure 1. *Flow chart*

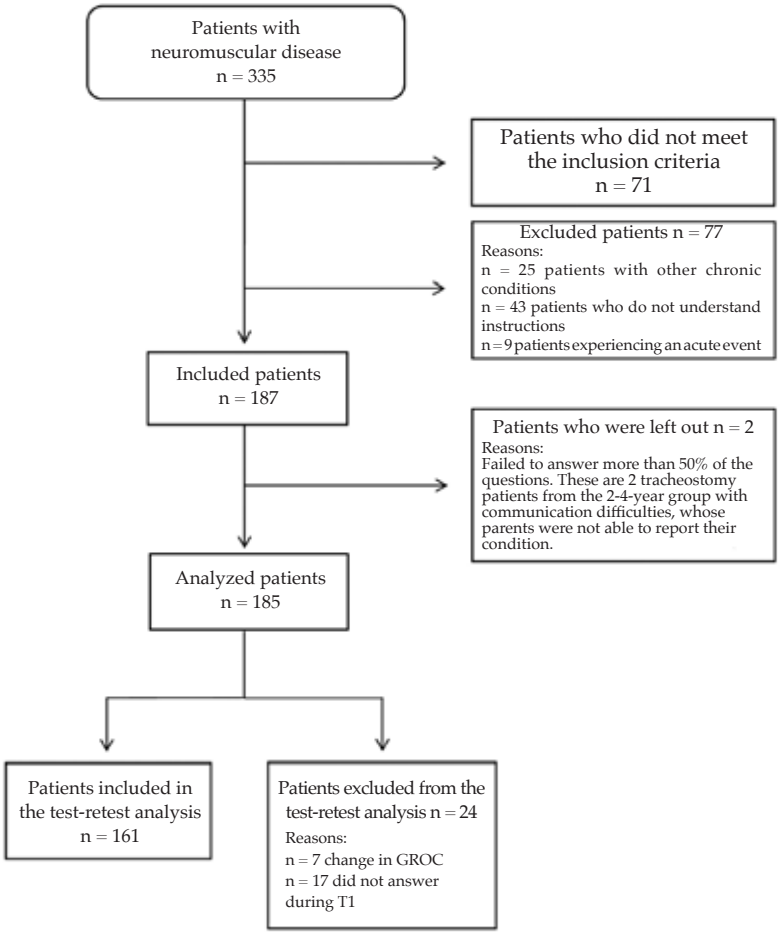


TABLE 1. *Characteristics of participants*

Demographic data	**All n = 185**	**Completed T1 n = 161**	**Excluded T1 n = 24**	***p* value**
Male sex, n (%)	118 (63.8)	102 (63.4)	16 (66.7)	0.753
Age, median (IQR), years	10 (6-13)	10 (6-13)	8 (6-12.75)	0.72
Age in years by group, n (%)				0.549
2-4	28 (15.1)	26 (16.1)	2 (8.3)	
5-7	38 (20.5)	31 (19.3)	7 (29.2)	
8-12	64 (34.6)	55 (34.2)	9 (37.5)	
13-18	55 (29.7)	49 (30.4)	6 (25.0)	
Place of origin, n (%)				
CABA	13 (7.0)	13 (8.1)	0 (0)	
Greater Buenos Aires	83 (44.9)	73 (45.3)	10 (41.7)	
PBA and other provinces	89 (48.2)	75 (46.6)	14 (58.3)	
Level of education, n (%)				0.881
None	12 (6.5)	10 (6.2)	2 (8.3)	
Regular	164 (88.6)	143 (88.8)	21 (87.5)	
Special	3 (1.6)	3 (1.9)	0 (0)	
Home schooling	6 (3.2)	5 (3.1)	1 (4.2)	
Mother as accompanying family member, n (%)	165 (89.2)	142 (88.2)	23 (95.8)	0.479
Level of education of accompanying family member, n (%)				0.6
Incomplete primary education	4 (2.2)	4 (2.5)	0 (0)	
Complete primary education	52 (28.1)	43 (26.7)	9 (37.5)	
Complete secondary education	76 (41.1)	68 (42.2)	8 (33.3)	
Complete tertiary/university education	53 (28.6)	46 (28.6)	7 (29.2)	
Clinical characteristics	**All n = 185**	**Completed T1 n = 161**	**Excluded T1 n = 24**	***p* value**
Health coverage, n (%)	163 (88.1)	142 (76.7)	21 (87.5)	0.701
Age at symptom onset, median (IQR), in months	12 (6-36)	12 (6-36)	12 (4.5-45)	0.871
Age at diagnosis, median (IQR), in months	36 (18-72)	36 (18-72)	48 (23-72)	0.287
Diagnosis, n (%)				0.337
DMD/BMD	54 (29.2)	47 (29.2)	7 (29.2)	
SMA	63 (34.1)	57 (35.4)	6 (25)	
Other types of muscular dystrophy	26 (14.1)	21 (13)	5 (20.8)	
Myopathies	20 (10.8)	19 (11.8)	1 (4.2)	
Other	22 (11.9)	17 (10.6)	5 (20.8)	
Taking medication for their condition, n (%)	109 (58.9)	98 (60.8)	12 (50)	0.568
Ambulatory, n (%)	94 (50.8)	82 (50.9)	12 (50)	0.932
Tracheostomy, n (%)	9 (4.9)	4 (2.5)	5 (20.8)	0.002
Ventilatory support, n (%)				0.489
None	146 (78.9)	125 (77.6)	21 (87.5)	
Partial	34 (18.4)	31 (19.2)	3 (12.5)	
Total	5 (2.7)	5 (3.10)	0 (0)	
Generic PEDsQL for children, median (IQR), score (*)	67.4 (54.3-78.3)	67.4 (54.5-78.3)	70.1 (47.8-78.5)	0.727
Generic PEDsQL for family members, median (IQR), score	61.4 (50-72.8)	61.9 (51.6-72.8)	56.9 (42.9-67.9)	0.139
Vignos scale, median (IQR), score	6.5 (3-9)	6.5 (3-9)	7.5 (3-9)	0.59
Brooke scale, median (IQR), score	2 (1-3)	2 (1-3)	2.5 (2-3)	0.627
OPPC as per child aged 5-7 years, median (IQR), score (**)	2 (2-2)	2 (2-2)	2 (2-2)	0.786
OPPC as per child older than 7 years, median (IQR), score (***)	9 (7-10)	9 (7-10)	9.5 (8-10)	0.178
OPPC as per kinesiologist, median (IQR), score	1 (0-3)	1 (0-3)	1 (1-3)	0.749
OPPC as per family member, median (IQR), score	8 (7-9)	8 (7-9.25)	8 (7-9)	0.484

T1: retest; CABA: Autonomous City of Buenos Aires; IQR: interquartile range; DMD: Duchenne muscular dystrophy; BMD: Becker muscular dystrophy; SMA: spinal muscular atrophy; PEDsQL: Pediatric Quality of Life Inventory™; OPPC: overall perception of patient condition.
* Estimated based on n = 157 (children aged 5-18 years).
** Estimated based on n = 38 (potential value range: 0-2).
*** Estimated based on n = 119 (potential value range: 0-10).

(2.1) for the overall score and the score by domain of the PedsQL™ NM 3.0, together with SEMs and MDCs. The Bland-Altman plots for both versions are shown in *Figure 2*.

In relation to construct validity, *Table 3* shows Spearman's correlation coefficients for the comparison between both versions of the PedsQL™ NM 3.0 and the selected correlation criteria. In general, 8 of the 11 hypotheses established were confirmed (72.7 %). *Table 3* reports the results of the comparison between known groups. The hypotheses about differences in terms of HRQoL according to parents of children who could ambulate or not and those related to ventilatory support were corroborated for both versions.

Table 4 shows the overall score and the score by domain of the PedsQL™ NM 3.0 for the different age groups for both the child and parent versions. In relation to interpretability, the mean score of the PedsQL™ NM 3.0 among the 157 children who completed the questionnaire during T0 was 73.78 points. The average score among the 185 family members who completed the questionnaire during T0 was 67.7 points. The ceiling effect was achieved by 2 patients and 2 parents.

DISCUSSION

The validation of the psychometric properties of the PedsQL™ NM 3.0 were adequate for both the child and parent versions.

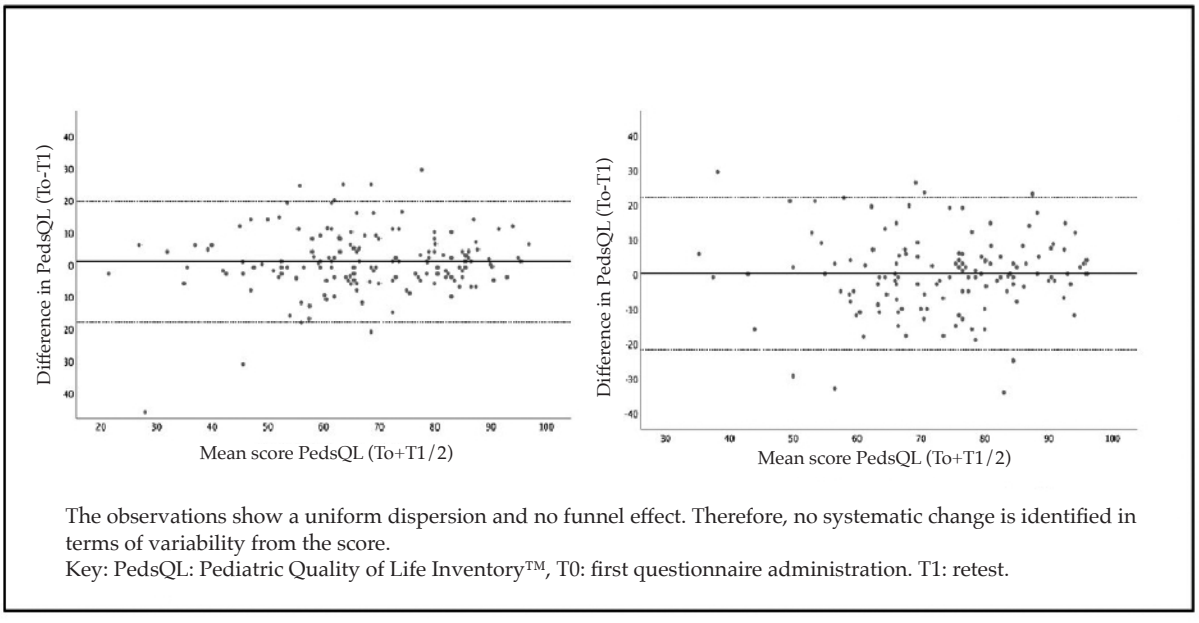
Both versions of the questionnaire showed an acceptable internal consistency, similar to what has been reported by other authors.[4-10]

The test-retest results were acceptable and demonstrated that the questionnaire is reliable to assess clinically stable patients. The child version showed a lower reliability, mainly in the "Communication" and "About my family resources" domains. This was similar to other questionnaire validations, which may limit its use in the assessment of this specific aspects.[4-6,10]

In relation to construct validity, a strong correlation was observed between the PedsQL™ 4.0 and the PedsQL™ NM 3.0, child and parent versions, as observed in other validations.[4-6] The generic version provides more information on the social, emotional, and child education aspects. Most likely, using both scales may allow to perform a more comprehensive assessment of HRQoL.

NMDs are chronic, disabling conditions. Children with NMD develop adaptations and/or compensations that are not equally perceived

FIGURE 2. *Brand-Altman plots, PedsQL™ Neuromuscular Module. Left: parent version. Right: child version*



The observations show a uniform dispersion and no funnel effect. Therefore, no systematic change is identified in terms of variability from the score.
Key: PedsQL: Pediatric Quality of Life Inventory™, T0: first questionnaire administration. T1: retest.

by their caregivers. Similar to other validations, the scores obtained by parents were lower than that of their children.[4,5,7-9] Considering this, there was a poor correlation between the OPPCf and the child version of the questionnaire, which was not corroborated, although the value was close to the cutoff point. The poor correlation observed between the Vignos and Brooke scales and the functional domain "About my neuromuscular disease" and the differences noted when comparing HRQoL between ambulatory and non-ambulatory children indicate that patients are adapted to their condition and do not perceive it as an obstacle.[36-38] Respiratory involvement

TABLE 2. *Internal consistency and test-retest reliability*

Child version								
PEDsQL™ Neuromuscular Module	**n**	**Chronbach's alpha**	**n**	**T0 mean (SD)**	**T1 mean (SD)**	**ICC (95 % CI)**	**SEM**	**MDC 95**
Overall	109	0.822	135	74.0 (14.7)	73.7 (14.4)	0.704 (0.67-0.780)	8	22.17
2-4 years	NA	NA	NA	NA	NA	NA	NA	NA
5-7 years	37	0.861	31	73.8 (17.6)	70.2 (17.0)	0.717 (0.494-0.852)	9.36	25.95
8-12 years	58	0.843	55	74.6 (15.5)	74.4 (15.3)	0.745 (0.599-0.843)	7.82	21.69
13-18 years	51	0.789	49	73.5 (11.7)	75.1 (11.2)	0.716 (0.548-0.829)	6.23	17.28
About my neuromuscular disease	149	0.801	161	75.2 (14.9)	74.4 (14.9)	0.769 (0.725-0.862)	7.16	19.85
2-4 years	NA	NA	NA	NA	NA	NA	NA	NA
5-7 years	37	0.861	31	73.8 (17.6)	70.2 (17.0)	0.717 (0.494-0.852)	9.36	25.95
8-12 years	59	0.756	55	76.6 (14.9)	75.4 (15.5)	0.674 (0.499-0.796)	8.51	23.58
13-18 years	53	0.761	49	74.4 (13.2)	76.0 (12.4)	0.769 (0.625-0.862)	6.34	17.58
Communication	118	0.738	161	68.9 (27.3)	71.3 (26.5)	0.547 (0.318-0.716)	18.37	50.93
2-4 years	NA	NA	NA	NA	NA	NA	NA	NA
5-7 years	NA	NA	NA	NA	NA	NA	NA	NA
8-12 years	63	0.701	55	68.8 (27.8)	70.5 (27.1)	0.614 (0.416-0.756)	17.27	47.87
13-18 years	55	0.784	49	68.9 (27)	72.1 (26.2)	0.547 (0.318-0.716)	18.17	50.37
About my family resources	114	0.624	161	72.6 (20.9)	73 (20.4)	0.648 (0.450-0.785)	12.43	34.45
2-4 years	NA	NA	NA	NA	NA	NA	NA	NA
5-7 years	NA	NA	NA	NA	NA	NA	NA	NA
8-12 years	62	0.72	55	71.8 (24.3)	73.3 (22)	0.660 (0.477-0.788)	14.17	39.27
13-18 years	52	0.382	49	73.4 (16.6)	74.1 (15.7)	0.648 (0.450-0.785)	9.85	27.3
Parent version								
Overall	156	0.873	161	68.5 (16.7)	67.6 (15.8)	0.829 (0.773-0.871)	6.9	19.14
2-4 years	21	0.736	26	69.1 (17.9)	68.6 (16.2)	0.710 (0.449-0.859)	9.64	26.71
5-7 years	34	0.915	31	68.8 (18)	67.3 (17.7)	0.906 (0.816-0.953)	5.52	15.29
8-12 years	54	0.828	55	70.7 (14.2)	69.1 (14.8)	0.837 (0.737-0.902)	5.73	15.89
13-18 years	47	0.899	49	65.4 (17.8)	65.6 (15.6)	0.832 (0.721-0.902)	7.29	20.22
About my neuromuscular disease	161	0.83	161	70.1 (16.6)	68.9 (16.2)	0.833 (0.778-0.875)	6.78	18.8
2-4 years	22	0.729	26	73.5 (13.1)	72.1 (15.5)	0.763 (0.540-0.886)	6.37	17.68
5-7 years	34	0.891	31	69.6 (19.3)	68.1 (18.3)	0.932 (0.864-0.966)	5.03	13.95
8-12 years	58	0.784	55	71.3 (15.5)	69.8 (15.5)	0.802 (0.683-0.879)	6.89	19.11
13-18 years	47	0.85	49	67.2 (17.5)	66.8 (16)	0.811 (0.687-0.889)	7.61	21.09
Communication	180	0.85	161	64.7 (32.3)	61.8 (32.1)	0.686 (0.595-0.759)	18.1	50.17
2-4 years	25	0.931	26	73.5 (39.1)	52.9 (40.8)	0.692 (0.425-0.849)	21.7	60.15
5-7 years	38	0.832	31	68.3 (30.2)	71.5 (27.8)	0.693 (0.455-0.839)	16.73	46.38
8-12 years	62	0.741	55	69.5 (28.6)	64.1 (27.7)	0.675 (0.501-0.796)	16.3	45.19
13-18 years	55	0.897	49	57.6 (33.1)	58 (33)	0.680 (0.494-0.806)	18.72	51.9
About my family resources	178	0.694	161	67 (21.4)	66.7 (21.8)	0.740 (0.661-0.803)	10.91	30.2
2-4 years	27	0.489	26	68.7 (18.7)	66.7 (21.8)	0.726 (0.479-0.867)	9.79	27.13
5-7 years	36	0.791	31	66.1 (20.9)	61.5 (22.5)	0.659 (0.408-0.819)	12.2	33.8
8-12 years	62	0.604	55	69.4 (20.3)	69.9 (20.3)	0.782 (0.653-0.867)	9.48	26.27
13-18 years	53	0.753	49	63.9 (24.1)	66.6 (23)	0.758 (0.609-0.856)	11.85	32.86

SD: standard deviation. SEM: standard error of measurement. ICC: intraclass correlation coefficient.
MDC: minimal detectable change. PEDsQL: Pediatric Quality of Life Inventory™. NM: neuromuscular. NA: not applicable.
T0: first questionnaire administration. T1: retest.

appears to have a greater impact on HRQoL since significant differences were observed in the score between the child version and the version for parents of children with ventilatory support.

The correlation between the parent version and the OPPCf did not have the expected results, possibly because the family member reported on the child's condition in the past month,

Table 3. *Construct validity: correlation hypothesis and testing of the child and parent versions*

Child version											
PedsQL NM	**n**	**Generic PedsQL**	**n**	**Vignos scale**	**n**	**Brooke scale**	**n**	**OPPC as per kinesiologist**	**n**	**OPPC as per family member**	**n**
		H1: Strong positive correlation								H2**:** Poor positive correlation	
Overall	157	**0.624**							157	0.306	157
2-4 years		NA									NA
5-7 years	38	0.663							38	0.203	38
8-12 years	64	0.643							64	0.314	64
13-18 years	55	0.587							55	0.377	55
				H3: Poor negative correlation		**H4: Poor negative correlation**		**H5: Poor negative correlation**			
About my neuromuscular disease			157	**-0.169**	157	**-0.175**	157	**-0.152**			
2-4 years				NA		NA		NA			
5-7 years			38	0.01	38	-0.056	38	-0.361			
8-12 years			64	-0.314	64	-0.33	64	-0.016			
13-18 years			55	-0.169	55	-0.141	55	-0.142			
Parent version											
PedsQL NM	**n**	**Generic PedsQL**	**n**	**Vignos scale**	**n**	**Brooke scale**	**n**	**OPPC as per kinesiologist**	**n**	**OPPC as per family member**	**n** **PedsQL NM child**
		H6: Strong positive correlation								H7: Strong positive correlation	**H11: Moderate positive correlation**
Overall	185	**0.691**							185	0.293	185 **0.446**
2-4 years	28	0.428							28	-0.067	NA NA
5-7 years	38	0.816							38	0.452	38 0.439
8-12 years	64	0.732							64	0.086	64 0.439
13-18 years	55	0.71							55	0.5	55 0.444
				H8: Moderate negative correlation		**H9: Moderate negative correlation**		H10: Moderate negative correlation			
About my neuromuscular disease			185	**-0.475**	185	**-0.437**	185	-0.291			
2-4 years			28	-0.368	28	-0.382	28	-0.509			
5-7 years			38	-0.381	38	-0.377	38	-0.505			
8-12 years			64	-0.624	64	-0.51	64	-0.025			
13-18 years			55	-0.449	55	-0.479	55	-0.265			

OPPC: overall perception of patient condition. PedsQL: Pediatric Quality of Life Inventory™. NM: neuromuscular.
NA: not applicable. H: hypothesis.
In bold, 8 of the 11 (72.7%) hypotheses corroborated with the PedsQL Neuromuscular Module.
All correlation measurements were described based on Spearman's Rho correlation coefficient.

TABLE 3. *(Continued)*

Construct validity. Comparison of known groups			
H12: The overall score is higher among children aged 2-13 years compared to those in the 14-18-year sub-group.			
PedsQL NM, child version	n	Median (IQR)	
Children aged 2-13 years	114	74.5 (62.5-86)	$p = 0.984$
Children aged 14-18 years	43	75 (66-84)	
H13: The overall score is higher among parents of children aged 2-13 years compared to those in the 14-18-year sub-group.			
PedsQL NM, parent version	n	Median (IQR)	
Parents of children aged 2-13 years	142	69.5 (60-81)	$p = 0.155$
Parents of children aged 14-18 years	43	67 (49-81)	
H14: The overall score is lower among non-ambulatory children compared to ambulatory children.			
PedsQL NM, child version	n	Mean (SD)	
Non-ambulatory	73	72.7 (15.3)	$p = 0.43$
Ambulatory	84	74.7 (15.7)	
H15: The overall score is lower among parents of non-ambulatory children compared to those of ambulatory children.			
PedsQL NM, parent version	n	Median (IQR)	$p < 0.001$
Non-ambulatory	91	62 (51-71)	
Ambulatory	94	77.5 (67-86)	
H16: The overall score is lower among children requiring ventilatory support compared to those who did not.			
PedsQL NM, child version	n	Median (IQR)	
Ventilatory support requirement	32	68.8 (61.8-80.7)	$p = 0.03$
No ventilatory support requirement	125	77 (66-87)	
H17: The overall score is lower among parents of non-ambulatory children compared to those of ambulatory children.			
PedsQL NM, parent version	n	Median (IQR)	$p < 0.002$
Ventilatory support requirement	39	60 (51-72)	
No ventilatory support requirement	146	71 (60.7-82.2)	

PEDsQL: Pediatric Quality of Life Inventory™. NM: neuromuscular. H: hypothesis. IQR: interquartile range. SD: standard deviation.

considering bonding and emotional aspects not included in this questionnaire. Also, the OPPCk and the "About my neuromuscular disease" domain did not achieve the expected correlation. This may be explained because the former compares the patient's current condition to the latest visit 6 months ago, whereas the latter compares it to the past month. Bach observed that caregivers' perception about the HRQoL of children with SMA type I is significantly higher than that perceived by their physicians.[38]

Unlike the version for Spain,[7-9] the mean scores were >60 points, except for the "Communication" domain of the parent version for the 13-18-year group, which scored 55 points. As suggested by Girabent-Farré, this may be related to psychosocial and communication aspects typical of adolescence.[9] No differences were observed between both versions when exploring HRQoL based on age.

This study has certain limitations. The retest was not done in person because almost half of patients did not live near the hospital. Therefore, it is not possible to warrant that children's answers were completed by themselves, especially in the group aged 5-7 years, who need an adult to make the questions without interfering with the children's answers.

In addition, the sample of patients aged 2-4 years and 5-7 years was smaller than the rest. This may be because one of the inclusion criteria was a prior assessment and many NMDs are usually diagnosed in the preschool age. Consistent with this, participants' median age was 10 years.

This study also has strengths. Based on these results, there is a valid instrument available to make an objective measurement of HRQoL in the Argentine pediatric population with NMDs. The conduct of this study at a national, public, referral hospital allowed to recruit children from across the country, in spite of the low prevalence of these conditions in the general population.

For future studies, it is important to assess the use of the PedsQL™ NM 3.0 in the follow-up of children with NMDs and/or as a measurement instrument in new scientific projects developed in Argentina.

CONCLUSIONS

The PedsQL™ NM 3.0 questionnaire in Spanish for Argentina, child and parent versions, shows acceptable reliability, internal consistency, and construct validity values. It is recommended to compare groups using the overall score or the first domain "About my neuromuscular disease" in clinical or research settings. ■

TABLE 4. *Interpretability assessment*

Child version					
PEDsQL™ Neuromuscular Module	**n values**	**Mean (SD) n (%)**	**Min.-Max. n (%)**	**Floor**	**Ceiling**
Child					
Overall	157	73.78 (15.5)	14.7-100	0 (0)	2 (1.3)
2-4 years	NA	NA	NA	NA	NA
5-7 years (*)	38	70.6 (20.3)	14.7-100	0 (0)	2 (5.3)
8-12 years (*)	64	75.0 (14.8)	36-99	0 (0)	0 (0)
13-18 years (*)	55	74.5 (12.1)	51-96	0 (0)	0 (0)
About my neuromuscular disease	157	74.7 (15.7)	14.7-100	0 (0)	2 (1.3)
2-4 years	NA	NA	NA	NA	NA
5-7 years (*)	38	70.6 (20.3)	14.7-100	0 (0)	2 (5.3)
8-12 years (*)	64	76.7 (14.2)	36.8-98.5	0 (0)	0 (0)
13-18 years	55	75.4 (13.4)	48.5-97.0	0 (0)	0 (0)
Communication	118	69.5 (27.4)	0-100	2 (1.7)	29 (24.6)
2-4 years	NA	NA	NA	NA	NA
5-7 years	NA	NA	NA	NA	NA
8-12 years	64	70.0 (27.9)	0-100	2 (3.1)	16 (25.0)
13-18 years	55	69.0 (27.2)	8.3-100	0 (0)	13 (23.6)
About my family resources	18	74.0 (20.5)	20-100	0 (0)	19 (16.1)
2-4 years	NA	NA	NA	NA	NA
5-7 years	NA	NA	NA	NA	NA
8-12 years	64	73.1 (23.5)	20-100	0 (0)	12 (18.7)
13-18 years	55	75.1 (16.6)	40-100	0 (0)	7 (12.7)
Parent version					
Overall	185	67.7 (17.0)	5-100	0 (0)	2 (1.1)
2-4 years	28	68.1 (17.7)	5-88.1	0 (0)	0 (0)
5-7 years (*)	38	65.9 (18.9)	25-100	0 (0)	1 (2.6)
8-12 years (*)	64	71.0 (13.5)	35-96	0 (0)	0 (0)
13-18 years (*)	55	64.9 (18.6)	20-100	0 (0)	1 (1.8)
About my neuromuscular disease	185	69.4 (16.8)	176-100	0 (0)	2 (1.1)
2-4 years (*)	28	72.0 (13.8)	48.5-92.6	0 (0)	0 (0)
5-7 years (*)	38	67.1 (20.0)	17.6-100	0 (0)	1 (2.6)
8-12 years (*)	64	71.3 (14.8)	30.9-97	0 (0)	0 (0)
13-18 years	55	67.5 (18.0)	22-100	0 (0)	1 (1.8)
Communication	185	64.0 (32.4)	0-100	16 (8.6)	42 (22.7)
2-4 years	28	63.6 (38.3)	0-100	5 (17.8)	8 (28.6)
5-7 years	38	65.1 (31)	0-100	3 (7.9)	7 (18.4)
8-12 years	64	70.6 (27.4)	8.3-100	0 (0)	17 (26.6)
13-18 years	55	55.7 (34.6)	0-100	8 (14.5)	10 (18.2)
About my family resources	185	65.7 (22.2)	10-100	0 (0)	15 (8.1)
2-4 years (*)	28	67.8 (18.4)	35-100	0 (0)	2 (7.1)
5-7 years (*)	38	62.1 (23.5)	15-100	0 (0)	2 (5.3)
8-12 years (*)	64	70.4 (19.5)	15-100	0 (0)	6 (9.4)
13-18 years (*)	55	61.5 (25.2)	10-100	0 (0)	5 (9.1)

IQR: interquartile range; DMD: Duchenne muscular dystrophy; BMD: Becker muscular dystrophy; SMA: spinal muscular atrophy; PEDsQL: Pediatric Quality of Life Inventory™; NM: neuromuscular.
(*) Values are described as mean and standard deviation (SD). NA: not applicable.

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