REVISTA CHILENA DE PEDIATRÍA
Sociedad Chilena de Pediatría
www.scielo.cl

Rev Chil Pediatr. 2017;88(1):100-106
DOI: 10.1016/j.rchipe.2016.07.003

ORIGINAL ARTICLE

Upper limb functional assessment scale for children with Duchenne muscular dystrophy and Spinal muscular atrophy

Escala de evaluación funcional de extremidades superiores en niños con distrofia muscular de Duchenne y Atrofia músculo espinal

Raúl G. Escobar[a,b], Nayadet Lucero[b], Carmen Solares[c,d], Victoria Espinoza[c,d], Odalie Moscoso[b], Polín Olguín[c,d], Karin T. Muñoz[b,e], Ricardo Rosas[c,d,*]

[a]Neurology Unit, Pediatrics Division, School of Medicine, Pontificia Universidad Católica de Chile, Santiago, Chile
[b]Neurorehabilitation and Neuromuscular Disease of Childhood laboratory, Clinical Hospital, Red de Salud UC-CHRISTUS, Santiago, Chile
[c]School of Psychology, Pontificia Universidad Católica de Chile, Santiago, Chile
[d]Inclusion Technology Development Center, Pontificia Universidad Católica de Chile (CEDETi UC), Santiago, Chile.
[e]School of Kinesiology, Pontificia Universidad Católica de Chile, Santiago, Chile

Received: 1-5-2016; Accepted: 13-7-2016

Abstract

Introduction: Duchenne muscular dystrophy (DMD) and Spinal muscular atrophy (SMA) causes significant disability and progressive functional impairment. Readily available instruments that assess functionality, especially in advanced stages of the disease, are required to monitor the progress of the disease and the impact of therapeutic interventions. **Objective:** To describe the development of a scale to evaluate upper limb function (UL) in patients with DMD and SMA, and describe its validation process, which includes self-training for evaluators. **Patients and Method:** The development of the scale included a review of published scales, an exploratory application of a pilot scale in healthy children and those with DMD, self-training of evaluators in applying the scale using a handbook and video tutorial, and assessment of a group of children with DMD and SMA using the final scale. Reliability was assessed using Cronbach and Kendall concordance and with intra and inter-rater test-retest, and validity with concordance and factorial analysis. **Results:** A high level of reliability was observed, with high internal consistency (Cronbach a = 0.97), and inter-rater (Kendall W = 0.96) and intra-rater concordance (r = 0.97 to 0.99). The validity was demonstrated by the absence of significant differences between results by different evaluators with an expert evaluator (F = 0.023, P > .5), and by the factor analysis that showed that four factors account for 85.44% of total variance. **Conclusions:** This scale is a reliable and valid tool for assessing UL functionality in children with DMD and SMA. It is also easily implementable due to the possibility of self-training and the use of simple and inexpensive materials.

Keywords:
Neuromuscular diseases; Scale; Function; Duchenne muscular dystrophy; Spinal muscular atrophy; Upper limb.

Correspondence:
Raúl G. Escobar
rescobar@med.puc.cl

Ricardo Rosas
rrosas@uc.cl

Introduction

Neuromuscular diseases (NMD) are those that affect the peripheral nervous system control resulting in muscular control loss. In pediatric age, most NMDs lack curative treatment and involve a significant functional compromise, leading to progressive disability. Any neurological rehabilitation program that helps these patients requires instruments to monitor functionality, prevent secondary disorders, provide a common language among professionals involved, and especially to evaluate the effect of different therapies.

Among pediatric NMDs, Duchenne muscular dystrophy (DMD) and spinal muscular atrophy (SMA) are the most common, with DMD as the most prevalent and both progressive and highly disabling[1,2]. Diverse function assessment tools have been described for DMD and SMA[3–5]. Among these instruments are those that evaluate motor function aspects of daily life activities and which, have a greater clinical benefit by including life-limiting concepts, highlighting the most relevant functional aspects of the patient.

Among the instruments described are those based on self-report [6–8], which provide information that allows the individual's ability to interact with society and his/her level of independence to be measured, but they have important limitations as they are not able to control non-matching variables. On the other hand, there are instruments that are based on the observation of an experienced evaluator, using standard procedures and following precise instructions and defined materials. These characteristics make them more objective and useful in evaluating more accurately the evolution of the disease, in addition to deliver adequate final criteria that is crucial when evaluating the effectiveness of therapeutic interventions. However, some authors argue that the application of these instruments at different centers and without adequate training of evaluators can affect their reliability [9,10]. The above challenges the use of these instruments, which becomes even more difficult when considering the need for translations when the instruments are originally in other languages[10].

In recent years, the emergence of new therapies for DMD and SMA has led to a series of clinical trials that required functional evaluation tools to assess their effectiveness [1–13]. This has helped us to know the validity and reliability of these instruments, which, however, have been focused on early stages of the disease prior to the inability of walking [10,14–16]. There are only a few evaluation tools to monitor the functionality after the inability of walking. Strength monitoring, through manual dynamometry, can give an objective account of the progression of strength loss after walking loss or before this occurs[17,18]. However, these assessments provide limited information on overall patient functionality and have important application limitations in more advanced stages of the disease. Upper limb function evaluation (UL) has been proposed as an adequate alternative for this purpose, therefore, instruments that aim to evaluate the effect of the loss of strength on UL have been developed [6,19–24].

The development of instruments to evaluate the evolution of NMDs, such as DMD and SMA, which provide objective information to assess the impact of therapeutic interventions, especially in post-loss stages, is crucial for a proper management of these patients. These instruments are of key importance and should be accessible to professionals in charge of patients with NMDs, without resulting in excessive medical expenses. These instruments should be designed with inexpensive and easily acquired materials, have manuals and instructions that promote self-learning and be available in the language of the evaluator and the population to be evaluated.

The objectives of this study are to report the development of a scale specifically made to evaluate UL function, its application in patients with DMD and SMA, and describe its validation process, which includes self-training for evaluators.

Patients and Method

Design and development of the scale

Initially, other scales specifically designed or adapted to evaluate NMDs functionality were reviewed[5,6,21–23,25–28]. Afterwards, a group of experts (pediatric neurologist specialized in NMDs, pediatric neurologist specialist in neurorehabilitation, occupational therapist, kinesiologist), with extensive experience in the management of pediatric patients with NMDs, reviewed the items intended to evaluate UL functionality, choosing an initial list of 17 items. Then, 4 additional items were added, aiming to reflect limitations in activities of daily living (ADL) in non-walking patients with NMDs. The list included a total of 21 items, which were applied to 8 healthy children (between 5 and 12 years of age) and then to 4 children with NMD (between 10 and 16 years of age). Eight items were modified according to the evaluations made creating the final list.

The final version of the scale included 21 items, which were grouped into 4 dimensions, similar to the one proposed by Mayhew[29]. Each item has a score from 0 to 5, except 5 of them that score between 0 and 4. The scale has a total score that ranges between 0 and 120.

Subsequently, a detailed manual about the application of the scale and the implementation of the necessary kit was written. In order to have a model, the scale application to a healthy adult was filmed. This

video was watched by 4 therapists, and based on their comments, modifications to the manual were made to obtain consistency between the images and the instructions.

The occupational therapist (OT), who participated in the scale design, evaluated a total of 10 patients with NMD (between 10 and 19 years of age), in 2 sessions, each separated by 2 weeks. These sessions were filmed so that the implementation of the scale could be scored by other evaluators. Prior to the scale implementation, the OT applied Barthel's index (IB) to each of the patients in the first session [30]. IB index is a 10-item scale that measures functional independence in the domains of personal care and mobility. The total score ranges between 0, total dependence, to 100, total independence. The validity and reliability of IB has been clearly established[31,32].

Evaluator training

After completing the above, 5 experienced therapists working with children with neurological disabilities (2 OT and 3 kinesiologists), became self-trained in the application of the scale using the manual and video (both available at: http://www.cedeti.cl/recursos-tecnologicos/escala-de-funcionalidad/funcionalidad-enfermedades-neuromusculares/). The frequency and time for review of the material was determined by each therapist; also, they were able to ask the OT questions.

Evaluator reliability

After the self-training phase, the evaluators received the assessment of each of the children performed by the OT, in addition to a set of the scale application guidelines. Evaluators performed the assessments consecutively, finalizing one process before starting the next. Each of the 5 evaluators applied the scale to the 10 patients, for a total of 50 evaluations (5 evaluations for each patient). After 8 weeks of the first round, evaluators repeated the assessments in the same way, completing 2 evaluations (test, re-test) for each of the 10 patients.

Patients

The sample was non-randomized and formed by 10 subjects, 8 DMD and 2 SMA, who had been followed up for at least 4 years in the Neurorehabilitation and Pediatric Neuromuscular Diseases Unit of Catholic University of Chile, all with confirmed diagnosis by genetic-molecular study. The mean age of subjects was 12.8 years (range: 9.4 to 19.1). One of the patients with SMA was female. Three of the 10 participants presented independent ambulation, DMD and under corticoid treatment. The other 5 patients with DMD had suspended corticosteroids since the inability of walking, at least a year earlier.

All participants completed their baseline assessments safely and without difficulty. The average time of the evaluation was 20 min (range: 15 to 23) and there was no evidence of fatigue in any of the subjects.

The study was approved by the ethics committee of the School of Medicine of the Pontifical Catholic University of Chile.

Statistical Analysis

Validity and reliability of the scale were evaluated with various analyzes detailed in the *Results* section. For all statistical tests, p values of less than 0.05 were considered significant. Statistical package SPSS® version 22 was used for analyzes.

Results

Reliability of scale

The first source of reliability is a measure of internal consistency analyzed by Cronbach's alpha. The result obtained is a = 0.97, showing a very high internal consistency.

The second is a measure of objectivity that helps us to determine how consistently judges evaluate the same cases using the scale. To do this, 5 judges were presented a total of 3 videos with fictitious cases. The evaluations were submitted to a Kendall coefficient of concordance W, obtaining a result of W = 0.96, a high and very significant concordance among judges ($p < 0.01$).

The third is a test-retest measure among judges. Six judges evaluated 10 videos of real cases two months apart. The average Pearson correlation between the first and second evaluation was between 0.97 and 0.99 (table 1), indicating a high consistency among judges' assessments.

Validity of the scale

The first evidence, related to validity of the scale content, is assured by the process of development of its items by experts, which was previously described in the section of *Patients and method*. In addition, content analysis was performed by a group of experts outside the team in charge of development.

The second source of validity, considered as evidence of concurrent validity, is the concordance of the scale results among judges, with the evaluation carried out by the evaluator 1, who is expert judge, regarded as the measurement pattern. When comparing the means of the 5 judges with this expert, a non-significant difference was obtained ($F = 0.023$, $p > 0.5$).

A third source of evidence, which supports concurrent validity, is obtained by comparing the results of the scale with IB, which showed an average score of 48

Table 1.

Evaluador	S1	S2	S3	S4	S5	S6	S7	S8	S9	S10	TS
Evaluador 1	0,99	0,98	1,00	1,00	0,99	0,79	0,97	1,00	1,00	0,88	0,98
Evaluador 2	0,99	1,00	0,67	0,96	0,98	0,79	0,94	0,92	0,95	0,89	0,97
Evaluador 3	0,98	0,98	1,00	0,97	0,99	0,99	0,95	0,85	1,00	0,81	0,98
Evaluador 4	1,00	0,97	0,94	0,99	0,97	0,98	0,98	1,00	1,00	0,95	0,99
Evaluador 5	0,95	1,00	1,00	0,94	0,92	0,98	0,95	0,98	0,97	0,98	0,97
Evaluador 6	0,99	0,97	0,45	0,99	0,97	1,00	0,94	0,99	1,00	0,77	0,97

En la tabla se detalla el índice de correlación de Pearson (r) entre la primera y la segunda evaluación de los sujetos para cada evaluador. En la columna "TS" se muestra la correlación entre la primera y segunda evaluación del conjunto de todos los sujetos para cada evaluador. S1: sujeto 1; S2: sujeto 2; S3: sujeto 3; S4: sujeto 4; S5: sujeto 5; S6: sujeto 6; S7: sujeto 7; S8: sujeto 8; S9: sujeto 9; S10: sujeto 10; TS: todos los sujetos.

Table 2.

Ítem	Componente de funcionalidad			
	Proximal	Media	Distal	Mixta
Abducción hombros hasta la altura de los hombros (D)	0,91	0,32	0,11	0,02
Abducción hombros sobre la altura de los hombros (D)	0,95	0,22	0,12	0,01
Flexión de hombros hasta la altura de los hombros (D)	0,94	0,26	0,12	0,01
Flexión de hombros sobre la altura de los hombros (D)	0,95	0,24	0,12	0,02
Abducción hombros hasta la altura de los hombros (I)	0,91	0,32	0,11	0,02
Abducción hombros sobre la altura de los hombros (I)	0,95	0,22	0,12	0,01
Flexión de hombros hasta la altura de los hombros (I)	0,94	0,26	0,12	0,01
Flexión de hombros sobre la altura de los hombros (I)	0,95	0,24	0,12	0,02
Manos a la boca	0,28	0,77	0,40	0,01
Trasladar peso desde los muslos a la mesa o a la altura de los hombros con las 2 manos	0,35	0,84	0,26	–0,01
Levantar y trasladar latas	0,25	0,72	0,39	0,09
Rasgar papel	0,45	0,06	0,75	0,17
Desplazar peso de un círculo a otro	0,41	0,79	0,20	0,10
Trazar trayecto en hoja	0,19	0,87	0,06	–0,23
Encender la luz presionando el interruptor	–0,20	–0,36	0,04	0,43
Agarrar 5 monedas	–0,01	0,40	–0,27	0,55
Levanta con agarre de 3 puntos de apoyo (pinza trípode)	0,05	0,30	0,92	–0,05
Levanta con agarre de 2 puntos de apoyo (pinza término-terminal)	0,09	0,43	0,86	–0,06
Ponerse una camiseta	0,74	0,54	0,23	0,07
Llevar lata llena de bebida a la boca	0,50	0,78	0,30	–0,03
Llevar cuchara a la boca	0,35	0,71	0,49	0,04
Peinarse	0,51	0,68	0,46	0,04
Lavarse los dientes	0,48	0,65	0,40	–0,01
Abrir una botella	0,17	-0,07	0,15	0,71
Abrir la tapa de un recipiente	0,06	0,29	0,92	0,05

Matriz rotada análisis factorial.

among the 10 patients (range 20 to 90). Although IB contains aspects not evaluated by our scale, the correlation was very high and significant (r = 0.93).

Finally, we have evidence of scale development validity through factor analysis (table 2). A Factorial analysis of the scale indicated 4 factors that explain 85.44% of the total variance. The first factor is proximal functionality, the second middle functionality, the third distal functionality and the fourth is of mixed functionality. The first one includes items of shoulder functionality and the process of putting on a T-shirt. The second one involves the processes of bringing hands to the mouth, transfer weight from the thighs to the table or to shoulder height with the two hands, lift and transfer cans, move weight from one circle to another, draw path on a paper, bring a full can to mouth, bring a spoon to the mouth, combing and brushing their teeth. The third one is the process of tearing a piece of paper and lifting an object with 2 point grip and opening the lid of a container. The fourth one consists of grabbing 5 coins and opening a bottle.

It is interesting to note that the most complex items on the scale, those that have significant factor weights in more than one factor, are precisely those that evaluate functionality of daily activities, such as putting on a T-shirt, bringing a full can to the mouth, bringing a spoon to the mouth, combing and brushing the teeth.

Discussion and Conclusions

This article describes an UL function scale evaluation in children with lack of strength secondary to 2 of the most frequent NMDs in pediatric age, DMD and SMA. The application of the scale requires a process of self-training and the use of inexpensive and easily acquired materials to create the stimuli used during the application. This scale proves to be highly reliable and shows a high concordance among and within the evaluators.

The need to have an UL function evaluation instrument in patients with progressive loss of muscle strength, especially in stages near or after the inability of walking, is due to the fact that these children present significant axial and lower extremity lack of muscle strength. This results in that the activities that these patients can perform the best are those that involve the use of their upper limbs, usually not considered in most of the existing scales of functionality[16–18].

In the selection, aspects that were not the exclusive expression of muscular strength, but reflected the ability to perform functional actions were included. We selected those tests already reported in the literature and which seemed to us more representative of strength-associated functionality [5,6,21–23,25–28]. The scale developed showed excellent reliability, with a very high internal consistency (Cronbach's of 0.97).

The fact that the loss of muscular strength and second motor neuron disorders is characterized by a proximal to distal progression should be considered when grouping the items of an evaluation scale for this type of diseases if differentiate degrees of strength expressed in functionality is intended. Mayhew et al. suggested grouping the items in 3 levels: high, middle and distal [29]. However, it is important to consider that the functionality of specific actions is affected by compensatory strategies that each patient develops through the evolution of the disease, and it is not only altered by the lack of strength in certain body parts such as the shoulders, elbows and/or wrists. Therefore, we thought that it was important to consider functionality actions represented by basic ADLs involving different segments of the upper limbs. Our scale was built in 4 dimensions: proximal, middle, distal and mixed functionality.

The factorial analysis of the scale was valid not only when it showed that 4 factors explain 85% of total variance, but also when described the presence of items with significant factorial weights in more than one factor. All these complex items correspond to the mixed functionality dimension. On the other hand, the high scale correlation (r = 0.97) with another instrument widely used in the evaluation of ADLs in DMD, such as IB [33,34], provides further evidence of its validity. In addition, IB has shown an important floor effect when applied to very weak patients with DMD and poor motility, suggesting the need to use instruments capable of adequately evaluating functionality in patients with a high degree of functional compromise [35], something that our scale shows to be capable of doing.

Literature suggests that the use of functional assessment instruments, based on clinical observation, requires specific training of the evaluator to achieve adequate reliability and consistency [9,10,28,36,37]. This aspect limits their use as training is not always readily available to the evaluators. Our scale, applied by self-trained evaluators, showed a high inter-evaluator agreement with a Kendall W coefficient of 0.96 ($p < 0.001$) and an average intra-judges correlation greater than 0.97, proving the effectiveness of the self-learning strategy. The high levels of reliability obtained in the application of the scale make possible to avoid the need for training for a correct application. Another advantage that presents the scale is the easy implementation of the battery used, formed by elements constructed with accessible and low cost materials. Finally, the scale is in Spanish, overcoming another limitation described in the literature regarding the lack of instruments developed in our language and the need of translation of instruments that have been validated in other languages [10].

The limited number of participants may be considered a weakness of the study, especially in the case of SMA. However, this does not detract from the findings, since there was no difference in the results obtained between those patients who maintained the ability to walk with respect to those who had lost it. There was also no difference between children with DMD compared to children with SMA. Therefore, this scale is able to provide objective information on UL functionality in these patients, even at different stages of the evolution of the disease. Future studies involving a greater number of patients, both with DMD and SMA at different stages of the disease, and other types of myopathies with significant strength compromise, especially UL, are crucial.

In summary, this scale is a reliable and valid instrument to evaluate UL functionality in children with DMD and SMA between 9 and 19 years of age. In addition, it is easy to implement due to the possibility of self-training and the use of simple and inexpensive materials.

Ethical Responsabilities

Protection of people and animals: The authors reported that no experiments on either people or animals have been performed.

Confidentiality of personal data: The authors reported that they have followed the protocols of their center regarding the publication of personal data.

Privacy rights and informed consent: The authors have obtained the informed consent from patients and/or subjects referred to in the article. These documents are in the possession of the corresponding author.

Funding

Project funded by Vice-Rectory for Research, UC Interdisciplinary Research Center, Pontifical Catholic University of Chile.

Conflict of interests

The authors declare no conflict of interest.

Acknowledgements

We thank the OT Rodolfo Aviles, Vanesa Bron, Miguel Jorquera, Loreto Quezada, María Paz Salinas y Bernardita Severin; all part of the staff at Neurorehabilitation and Neuromuscular Disease Laboratory, Catholic University, for your help to determine the validity and reliability of the scale.

References

1. Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: Diagnosis, and pharmacological and psychosocial management. Lancet Neurol. 2010;9:77-93.
2. Lunn MR, Wang CH. Spinal muscular atrophy. Lancet. 2008;371:2120-33.
3. Mercuri E, Mayhew A, Muntoni F, et al. Towards harmonization of outcome measures for DMD and SMA within TREAT-NMD. Neuromuscul Disord. 2008;18:894-903.
4. Iannaccone S, AmSMART Group. Outcome measures for pediatric spinal muscular atrophy. Arch Neurol. 2002;59:1445-50.
5. Main M, Kairon H, Mercuri E, Muntoni F. The Hammersmith Functional Motor Scale for children with spinal muscular atrophy: A scale to test ability and monitor progress in children with limited ambulation. Eur J Paediatr Neurol. 2003;7:155-9.
6. Steffensen B, Hyde S, Lyager S, Mattsson E. Validity of the EK scale: A functional assessment of non-ambulatory individuals with Duchenne muscular dystrophy or Spinal muscular atrophy. Physiother Res Int. 2001;6:119-34.
7. Vandervelde L, Van den Bergh PY, Penta M, Thonnard JL. Validation of the ABILHAND questionnaire to measure manual ability in children and adults with neuromuscular disorders. J Neurol Neurosurg Psychiatry. 2010;81:506-12.
8. Vandervelde L, Van den Bergh PY, Goemans N, Thonnard JL. Activity limitations in patients with neuromuscular disorders: A responsiveness study of the ACTIVLIM questionnaire. Neuromuscul Disord. 2009;19:99-103.
9. Mazzone ES, Messina S, Vasco G, et al. Reliability of the North Star Ambulatory Assessment in a multicentric setting. Neuromuscul Disord. 2009;19:458-61.
10. Scott E, Eagle M, Mayhew A, et al. Development of a functional Assessment Scale for ambulatory boys with Duchenne muscular dystrophy. Physiother Res Int. 2012;17:101-9.
11. Voit T, Topaloglu H, Straub V, et al. Safety and efficacy of drisapersen for the treatment of Duchenne muscular dystrophy (DEMAND II): An exploratory, randomized, placebo-controlled phase 2 study. Lancet Neurol. 2014;13:987-96.
12. Bushby K, Finkel R, Wong B, et al. Ataluren treatment of patients with nonsense mutation dystrophinopathy. Muscle Nerve. 2014;50:477-87.
13. Chiriboga CA, Swoboda KJ, Darras BT, et al. Results from a phase 1 study of nusinersen (ISIS-SMNrx) in children with spinal muscular atrophy. Neurology. 2016;86:1-8.
14. Bérard C, Payan C, Hodgkinson Fermanian J. A motor function measure scale for neuromuscular diseases. Construction and validation study. Neuromuscul Disord. 2005;15:463-70.
15. Vuillerot C, Girardot F, Payan C, et al. Monitoring changes and predicting loss of ambulation in Duchenne muscular dystrophy with the Motor Function Measure. Dev Med Child Neurol. 2010;52:60-5.
16. Mazzone ES, Vasco G, Palermo C, et al. A critical review of functional assessment tools for upper limbs in Duchenne muscular dystrophy. Dev Med Child Neurol. 2012;54:879-85.
17. Merlini L, Mazzone ES, Solari A, Morandi L. Reliability of handheld dynamometry

in spinal muscular atrophy. Muscle Nerve. 2002;26:64-70.
18. Stuberg WA, Metcalf WK. Reliability of quantitative muscle testing in healthy children and in children with Duchenne muscular dystrophy using a hand-held dynamometer. Phys Ther. 1988;68:977-82.
19. Lord JP, Portwood MM, Lieberman JS, Fowler WM Jr, Berck P. Upper extremity functional rating for patients with Duchenne muscular dystrophy. Arch Phys Med Rehabil. 1987;68:151-4.
20. Hiller LB, Wade CK. Upper extremity functional assessment scales in children with Duchenne muscular dystrophy: A comparison. Arch Phys Med Rehabil. 1992;73:527-34.
21. Brooke MH, Griggs RC, Mendell JR, Fenichel GM, Shumate JB, Pellegrino RJ. Clinical trial in Duchenne dystrophy. I. The design of the protocol. Muscle Nerve. 1981;4:186-97.
22. Mazzone E, Bianco F, Martinelli D, et al. Assessing upper limb function in nonambulant SMA patients: Development of a new module. Neuromuscul Disord. 2011;21:406-12.
23. Pane M, Mazzone ES, Fanelli L, et al. Reliability of the performance of upper limb assessment in Duchenne muscular dystrophy. Neuromuscul Disord. 2014;24:201-6.
24. Uchikawa K, Liu M, Hanayama K, Tsuji T, Fujiwara T, Chino N. Functional status and muscle strength in people with Duchenne muscular dystrophy living in the community. J Rehabil Med. 2004;36:124-9.
25. Vignos PJ Jr, Spencer GE Jr, Archibald KC. Management of progressive muscular dystrophy in childhood. JAMA. 1963;184:89-96.
26. Nelson L, Owens H, Hynan LS, Iannaccone ST, the AmSMART Group. The gross motor function measure is a valid and sensitive outcome measure for spinal muscular atrophy. Neuromuscul Disord. 2006;16:374-80.
27. Krosschell KJ, Maczulski JA, Crawford TO, Scott C, Swoboda KJ. A modified Hammersmith functional motor scale for use in multi-center research on spinal muscular atrophy. Neuromuscul Disord. 2006;17:693-7.
28. O'Hagen JM, Glanzman AM, McDermott MP, et al. An expanded version of the Hammersmith Functional Motor Scale for SMA II and III patients. Neuromuscul Disord. 2007;17:693-7.
29. Mayhew A, Mazzone ES, Eagle M, et al. Development of the performance of the upper limb module for Duchenne muscular dystrophy. Dev Med Child Neurol. 2013;55:1038-45.
30. Mahoney FI, Barthel DW. Functional evaluation: The Barthel index. Md State Med J. 1965;14:61-5.
31. Jacelon CS. The Barthel index and other indices of functional ability. Rehabil Nurs. 1986;11:9-11.
32. Shinar D, Gross CR, Bronstein KS, et al. Reliability of the activity of daily living scale and its use in telephone interview. Arch Phys Med Rehabil. 1987;68:723-8.
33. Nair KPS, Vasanth A, Gourie-Devi M, et al. Disabilities in children with Duchenne muscular dystrophy: A profile. J Rehabil Med. 2001;33:147-9.
34. Brunberotti MA, Sobreira C, Rodrigues-Júnior AL, et al. Correlations of Egen klassifikation and Barthel Index scores with pulmonary function parameters in Duchenne muscular dystrophy. Heart Lung. 2007;36:132-9.
35. Lue YJ, Lin RF, Chen SS, Lu YM. Measurement of the functional status of patients with different types of muscular dystrophy. Kaohsiung J Med Sci. 2009;25:325-33.
36. Ryan JW, Phillips CY, Prescott PA. Interrater reliability: The underdeveloped role of rater training. Appl Nurs Res. 1988;1:148-50.
37. Castorr AH, Thompson KO, Ryan JW, Phillips CY, Prescott PA, Soeken KL. The process of rater training for observational instruments: Implications for interrater reliability. Res Nurs Health. 1990;13:311-8.