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ASSESSMENT PROCEDURES

Towards a short questionnaire for stepwise assessment of upper limb function, pain and stiffness in Duchenne muscular dystrophy

Mariska M. H. P. Janssen, Alexander C. H. Geurts and Imelda J. M. de Groot

Department of Rehabilitation, Radboud University Medical Center, Donders Centre for Neuroscience, Nijmegen, the Netherlands

ABSTRACT

Purpose: Duchenne muscular dystrophy can lead to upper extremity limitations, pain and stiffness. In a previous study, these domains have been investigated using extensive questionnaires, which are too time-consuming for clinical practice. This study aimed at gaining insight into the underlying dimensions of these questionnaires, and to construct a short questionnaire that can be used for clinical assessment.

Methods: Exploratory factor analysis was performed on the responses of 213 participants to a web-based survey to find the underlying dimensions in the Capabilities of Upper Extremity questionnaire, the ABILHAND questionnaire, and questionnaires regarding pain and stiffness. Based on these underlying dimensions, a stepwise approach was formulated. In addition, construct validity of the factors was investigated.

Results: In total, 14 factors were identified. All had high internal consistency (Cronbach's alpha >0.89) and explained 80–88% of the variance of the original questionnaires. Construct validity was supported, because participants in the early ambulatory stage performed significantly better ($p< 0.001$) than participants in the late non-ambulatory stage.

Conclusion: The factors identified from the set of questionnaires provide a valid representation of upper extremity function, pain and stiffness in Duchenne muscular dystrophy. Based on the factor commonalities, the Upper Limb Short Questionnaire was formulated.

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Duchenne muscular dystrophy; factor analysis; pain; stiffness; upper extremity; upper limb short questionnaire

➤ IMPLICATIONS FOR REHABILITATION

- New insights into the underlying dimensions of upper extremity function, pain and stiffness in Duchenne muscular dystrophy are gained.
- Fourteen factors, with good internal consistency and construct validity, are identified regarding upper extremity function, pain and stiffness in Duchenne muscular dystrophy. Based on these factors, the Upper Limb Short Questionnaire is presented.
- The Upper Limb Short Questionnaire can be used as an identifier of arm-hand limitations and the start of more thorough clinical investigation.

Introduction

Duchenne muscular dystrophy (DMD) is the most common type of muscular dystrophy. DMD is caused by a defect in the dystrophin gene, which is located on the X-chromosome. This defect leads to a shortage or absence of the dystrophin protein, which results in progressive muscle degeneration [1]. As a consequence, boys with DMD experience muscular weakness already in early childhood. Boys with DMD lose the ability to walk around the age of 10 [2] and, without corticosteroid treatment, arm function starts to decrease in the early ambulatory phase [3].

Until now no cure has been found for DMD, however, life expectancy is increasing due to disease retarding treatments like corticosteroids and supportive techniques such as nocturnal ventilation [4]. Currently, median survival of boys with DMD is estimated to be over 30 years [2,4], which means that men with DMD experience functional limitations for the largest part of their lives. Functional limitations in the lower extremities can be compensated fairly well by using a wheelchair. In contrast, limitations in the upper extremities are much harder to compensate. This is unfortunate, since arm function is important to maintain independence in daily life. We previously investigated arm function in boys with DMD during the course of their disease using a web-based questionnaire [3]. We concluded that arm function started to decrease already in the early ambulatory phase and that, despite this loss of arm function, arm supports were rarely used.

In this previous study, upper limb function was measured using three existing questionnaires: the Capabilities of Upper Extremity Questionnaire (CUE) [5], the ABILHAND questionnaire (including few additional questions [6]), and the Brooke scale [7]. These scales were chosen based on unpublished data from our own research group, in which we concluded that these scales are the most appropriate self-report instruments to investigate upper extremity function in teenage boys with DMD. Taken together,

CONTACT Mariska MHP Janssen Mariska.Janssen@radboudumc.nl Radboud University Medical Center, Department of Rehabilitation, Reinier Postlaan 2, 6525 GC Nijmegen, the Netherlands

Supplemental data for this article can be accessed here.

these upper extremity questionnaires consist of more than 60 questions. In addition, questions concerning pain and stiffness were modified from the University of Michigan Upper Extremity Questionnaire [8]. In total, 42 questions about pain and 42 questions about stiffness in the upper extremities were asked.

This large number of questions gives an extensive insight into arm function and experienced pain and stiffness, but it also has disadvantages. The large number of questions can lead to low patient compliance. In addition, using all questions would be too time-consuming for diagnostic purposes in a clinical context. For clinical practice, a short questionnaire would be needed to identify what aspects of arm function the rehabilitation should focus on. Therefore, the primary aim of this study was to gain insight in the underlying dimensions of the above-mentioned set of questionnaires in order to formulate a short questionnaire that clinicians can use for stepwise assessment of upper extremity function, pain and stiffness in patients with DMD. The secondary aim of this study was to investigate the construct validity of the identified factors in boys and men with DMD.

Methods

Participants

This study was part of a larger study in which a total of 344 participants from 14 different countries responded to a web-based questionnaire [3]. The originally English questionnaire was translated into Dutch, Italian, Spanish, German, and French (Tekom, Hoofddorp, the Netherlands) and distributed through patient organizations in the different countries. We excluded respondents that did not agree with the clinical Duchenne phenotype, based on the diagnostic criteria of Emery [9]. Participants were also excluded if the diagnosis was made after the age of 10 years, or when participants who did not use corticosteroids and who were 14 years or older were not wheelchair confined [9]. In total, 213 participants were included. The questionnaires used for data collection were approved by the medical-ethical committee in the Arnhem-Nijmegen region (the Netherlands).

Questionnaires

Arm function was assessed with the Capabilities of Upper Extremity Questionnaire (CUE) [5] and the ABILHAND questionnaire [6]. These questionnaires were chosen based on unpublished data of our own research group, in which we concluded that the above mentioned selfreported questionnaires were most suitable for examining upper extremity function in DMD patients, since no specific upper extremity questionnaires for DMD were available at the time of that study. The chosen questionnaires were widely used for several diagnostic groups. The CUE consists of 17 items (of which 15 items are asked for either hand, yielding a total of 32 items) that examine basic functional activities of the upper extremities. The ABILHAND questionnaire consists of 22 items that assess more complex activities of the upper extremities. Next to the 22 ABILHAND items described by Vandervelde et al. [6], four more items were added (i.e., “eat with a spoon”, “use fork and knife”, “drink a glass of water without straw”, and “use the keyboard of a computer”), because these activities were indicated as very important by boys with DMD (unpublished data from our own research group). This adapted scale will be referred to as ABILHAND-plus.

Pain and stiffness in the segments and joints of the upper limbs were assessed with a scale that was adapted from the University of Michigan Upper Extremity Questionnaire [8]. Pain and stiffness were assessed on three different aspects: frequency, severity, and activity limitations due to pain and stiffness. Frequency was measured on a 7-point scale (range 0–6), whereas severity and activity limitations were measured on an 11-point scale (range 0–10).

Detailed information on the questionnaires used in this study is presented in Supplementary Tables S1–4.

Statistical methods

For the ABILHAND-plus, there were four answer options, including the option “I don’t know”. Since 28.5% of the respondents filled in “I don’t know” for at least one of the items, we chose to replace this value with the average of the remaining items, so the results of these respondents could still be included in the factor analysis. However, when more that one-third of the items was scored as “I don’t know” (which was the case for 2.5% of the respondents), we interpreted these as missing values, because taking the average of the remaining items was considered unreliable.

Exploratory factor analysis was performed to test the underlying dimensions in the questionnaires used. Principal component analysis (PCA) was applied as the extraction method. Orthogonal rotation (varimax) with Kaiser’s criterion, Eigenvalues >1.0, was used to determine the final number of extracted factors. The Kaiser–Meyer–Olkin measure was used to verify the sampling adequacy for the analysis. In addition, Bartlett’s test of sphericity was applied to test whether correlations between items were sufficiently large for PCA.

Cronbach’s alpha was calculated as a measure of internal consistency of each constructed factor. The percentage of variance explained by each factor is also presented.

To test the construct validity of the factors, the hypothesis was formulated that participants in the early ambulatory disease stage performed significantly better than participants in the late non-ambulatory disease stage. Student’s *t*-tests were used to test for statistical differences between the groups defined. The mean group differences with 95% confidence intervals of the factor sum scores are presented.

All statistical analysis were performed using IBM SPSS statistics 20.0 for Windows (SPSS Inc., Chicago, IL).

Results

Participant characteristics

Questionnaires were filled in by patients themselves (20%) or by their parents/legal guardians (80%). Of the 213 included participants, 198 filled in the complete questionnaire, whereas 15 participants ended the questionnaire prematurely. Participants were on average 13 years (range: 1–35 years) and 55% of the participants were wheelchair confined (median age of wheelchair confinement: 10 years, range 1–20 years). The median age of diagnosis was 4 years (range 0–10 years) and 66% of the participants used corticosteroids (currently or in the past). In addition, 49% of the participants had a mild or severe scoliosis. A more detailed description of the participants can be found in Janssen et al. 2014 [3].

Factors

Sample adequacy of the factor analysis was good for all questionnaires (Kaiser–Meyer–Olkin>0.9). In addition, Bartlett’s tests of sphericity indicated that correlations between items were sufficiently large for PCA (p values <0.001). Table 1 represents the descriptive values for the factors identified in the CUE, ABILHAND-plus, pain, and stiffness questionnaires.

In total, 14 factors were identified in the CUE, ABILHAND-plus, and the questionnaires regarding pain and stiffness. The CUE

Table 1. The factors and their characteristics resulting from factor analysis on each questionnaire (CUE, ABILHAND-plus, PAIN, and STIFFNESS).

Factors	N	Eigenvalues	% of explained variance	% of explained variance (total)	Cronbach's Alpha	Kaiser–Meyer–Olkin	Bartlett's tests of sphericity (*p* values)
CUE				86.6		0.91	0.001
Basic hand function	8	22.4	74.7		0.98		
Heavy lifting	10	2.5	8.3		0.98		
Light or no lifting	12	1.1	3.6		0.99		
ABILHAND-plus				80.4		0.97	0.001
Gross hand function	16	19.7	75.7		0.97		
Fine hand function	10	1.2	4.7		0.98		
PAIN				86.3		0.86	0.001
Pain limitations	14	21.4	50.9		0.98		
Pain severity (not shoulder)	12	6.1	14.5		0.97		
Distal pain frequency	6	3.2	7.7		0.93		
Shoulder pain	4	2.0	4.7		0.89		
Proximal pain frequency (not shoulder)	4	1.5	3.5		0.93		
Elbow pain frequency	2	1.1	2.6		0.90		
STIFFNESS				87.9		0.98	0.001
Stiffness frequency	14	27.8	66.1		0.98		
Stiffness limitations	14	4.3	10.3		0.99		
Stiffness severity	14	2.1	5.1		0.98		

resulted in three factors, of which the corresponding items were related to "basic hand function", "heavy lifting", and "light or no lifting". The internal consistency of each factor was high (Cronbachs' α: 0.98–0.99). With respect to the ABILHAND-plus, two factors were identified, of which the corresponding items were related to "gross hand function" and "fine hand function". For both factors, the internal consistency was high (Cronbachs' α: 0.97–0.98). Factor analysis on the pain questionnaire resulted in six factors: "pain limitations", "pain severity (not shoulder)", "distal pain", "shoulder pain", "proximal pain frequency (not shoulder)", and "elbow pain frequency". The internal consistency for all factors was high (Cronbachs' α: 0.89–0.98). Within the stiffness questionnaire, three factors were identified: "stiffness frequency", "stiffness limitations", and "stiffness severity", all having a high internal consistency (Cronbachs' α: 0.98–0.99). The total percentage of explained variance for the factors identified in each questionnaire ranged from 80–88%. All items had large rotated factor loadings and we were able to interpret the commonality in each of the factors. Detailed descriptions of the items and their rotated factor loadings are presented in Supplementary Table S5.

Table 2 represents the correlation coefficients between the different factors. Most of the correlations were below 0.80, which indicated that the factors had unique identities. As expected, some of the CUE and ABILHAND-plus factors showed high correlations (*R*: 0.71–0.91).

Construct validity

In Table 3, the means and 95% confidence intervals of the factor sum scores for patients in the early ambulatory stage and those in the late non-ambulatory stage are presented. We hypothesized that participants in the early ambulatory disease stage would perform significantly better than participants in the late non-ambulatory disease stage. All factors confirmed this hypothesis, reflecting a good construct validity.

Upper limb short questionnaire (ULSQ)

Table 4 gives a proposal for the Upper Limb Short Questionnaire, which can be used for stepwise assessment of upper limb function, pain, and stiffness in clinical practice. Based on the factors identified in this study (Table 3), 14 new initial questions were formulated. Depending on the intended use of the Upper Limb Short Questionnaire, a specific set of follow-up questions can be asked. These follow-up questions correspond with the items of the original questionnaires clustering under the same factor.

Discussion

We found 14 different underlying dimensions (factors) in a set of four questionnaires regarding upper extremity function, pain, and stiffness in boys and men with DMD. Each factor showed good internal consistency and good construct validity, with respect to discriminating patients in the early ambulatory from those in the late non-ambulatory disease stage. These results allowed us to propose a short questionnaire for stepwise assessment of upper extremity function, pain, and stiffness for clinical use based on the existing questionnaires: the Upper Limb Short Questionnaire.

Upper extremity function was originally measured using the CUE and ABILHAND-plus questionnaires. Although the CUE consists of 32 items, one item ("holding an object like a hammer with your hand (left and right)") was erroneously not included in the questionnaire, so that 30 items remained. From the CUE and ABILHAND-plus questionnaires, a total of five factors were extracted. Internal consistency of the items within these factors was high, but also the correlation between the factors related to CUE and ABILHAND-plus was high. As a result, it might be questioned if these factors can be considered to be independent factors. Additional research is needed to see whether all the newly formulated questions in the short questionnaire, which are based on the factors, independently contribute to the insight in arm function.

The factors on upper extremity function as identified in this study appear to be coherent with the clinical representation of DMD patients. It is well known that proximal muscles and extensor groups are affected earlier and more severely than distal muscles and flexor groups in DMD patients [10–12]. When looking at the CUE, we indeed saw that the factor "heavy lifting", which mainly depends on proximal muscle function, was most severely affected, in comparison to "light or no lifting" and "basic hand function", which are more dependent on distal muscle function. In addition, the ABILHAND-plus factors are roughly in line with the literature. Vandervelde et al. described the difficulty of the ABILHAND items in patients with neuromuscular disorders using a Rasch model, where positive difficulty scores indicate a higher level of item difficulty [6]. It becomes clear that the items that

Table 2. The Pearson correlation between each pair of two factors.

	CUE			ABILHAND-plus		PAIN						STIFFNESS		
	Basic hand function	Heavy lifting	Light or no lifting	Gross hand function	Fine hand function	Pain limitations	Pain severity (not shoulder)	Distal pain frequency	Shoulder pain	Proximal pain frequency (not shoulder)	Elbow pain frequency	Stiffness frequency	Stiffness limitations	Stiffness severity
CUE														
Basic hand function	1.00													
Heavy lifting	**0.71**	1.00												
Light or no lifting	**0.81**	**0.88**	1.00											
ABILHAND-plus														
Gross hand function	**0.85**	**0.81**	**0.91**	1.00										
Fine hand function	**0.75**	**0.86**	**0.88**	**0.91**	1.00									
PAIN														
Pain limitations	−0.27	−0.32	−0.34	−0.31	−0.29	1.00								
Pain severity (not shoulder)	−0.18	−0.30	−0.28	−0.25	−0.25	**0.46**	1.00							
Distal pain frequency	−0.21	−0.24	−0.23	−0.19	−0.19	**0.40**	**0.58**	1.00						
Shoulder pain	−0.33	−0.40	−0.38	−0.39	−0.35	**0.44**	**0.70**	**0.60**	1.00					
Proximal pain frequency (not shoulder)	−0.15	−0.24	−0.21	−0.16	−0.17	**0.40**	**0.56**	**0.74**	**0.64**	1.00				
Elbow pain frequency	−0.39	−0.40	**−0.42**	−0.37	−0.36	**0.41**	**0.55**	**0.61**	**0.61**	**0.69**	1.00			
STIFFNESS														
Stiffness frequency	**−0.41**	**−0.42**	**−0.47**	**−0.46**	**−0.40**	**0.47**	0.37	0.33	**0.44**	0.32	0.38	1.00		
Stiffness limitations	**−0.44**	**−0.43**	**−0.50**	**−0.50**	**−0.45**	**0.54**	0.26	0.28	0.34	0.25	0.30	**0.68**	1.00	
Stiffness severity	−0.34	−0.32	−0.38	−0.37	−0.31	**0.57**	0.23	0.23	0.28	0.22	0.24	**0.64**	**0.82**	1.00

Values with a Pearson correlation coefficient above 0.4 are printed in bold.

represented "gross hand function" in our study were generally less difficult than those representing "fine hand function". Only two items within the factor "fine hand function" had negative difficulty scores, and also two items within the factor "gross hand function" had a positive difficulty score. As Vandervelde et al. [6] examined patients with neuromuscular disorders, of whom more than 20% were patients with DMD, comparable outcomes are in the line of expectation.

The 42 items about pain resulted in six factors. Internal consistency of the items within each factor was high and the correlation between the factors was moderate, indicating that each factor described a unique aspect of pain. When looking at the different segments that are represented in each factor, it can be seen that for pain limitations and pain severity almost all segments grouped in one factor, whereas for pain frequency segments were divided over four different factors. This might imply that questions regarding pain frequency are more prone to discriminate between the effect of pain in different body segments than questions regarding pain severity or limitations due to pain. When looking at the factors that discriminated between segments, we found that the shoulder, elbow, more proximal aspects of the arm and more distal aspects of the arm loaded on separate factors. Pain was most frequently present in the shoulder, followed by the elbow, proximal aspects of the arm (upper and lower arm) and distal aspects of the arm (wrist, fingers, and thumb). This is generally in line with literature, as Engel et al. [13] and Tiffereau et al. [14] reported that pain was more frequent in the shoulder compared to other parts of the upper extremity, while Pangalila et al. [15] reported equal occurrence of pain in the shoulder and arm.

Factor analysis on the stiffness questionnaire resulted in three unique factors. In contrast with pain, stiffness complaints in the different body segments all grouped together in one factor. However, frequency, severity, and limitations due to stiffness represented different dimensions, which implies that it is important to ask for all these aspects of stiffness. Therefore, three questions regarding stiffness were formulated in the short questionnaire.

The factors found in this study explained more than 80% of the variance of the original questionnaires. In addition, the factors could be interpreted well, as the rotated factor loadings were moderate to high and showed clear commonalities between the items within the factors. Moreover, the construct validity of the factors was good, as all factors showed that participants in the early ambulatory stage scored significantly better than participants in the late non-ambulatory stage. For the above-mentioned reasons, we believe that the applied factor analysis is a valid tool to formulate a short questionnaire for the stepwise assessment of upper extremity function, pain, and stiffness in DMD patients.

We believe that the Upper Limb Short Questionnaire is suitable to be further developed into an outcome measure for research purposes as well as into a tool for the clinical assessment of upper extremity function, pain, and stiffness. Based on the intended use, the administration and scoring of the Upper Limb Short Questionnaire differs. When used as an outcome measure in research, we propose to assess each of the 14 Upper Limb Short Questionnaire items, without follow-up questions, scoring either 0 (no restrictions) or 1 (restrictions), yielding a minimal sum score of 0 (no upper extremity limitations, pain or stiffness) and a maximal score of 14 (severe upper extremity function limitations, pain and stiffness). However, before the Upper Limb Short Questionnaire can be used as an outcome measure, it should be further tested. Future studies should particularly investigate its discriminative capacity in patients with different disease stages as well as its internal consistency, item hierarchy and test–retest reliability. When the Upper Limb Short Questionnaire is used for clinical

Table 3. Mean with 95% confidence intervals (CI) of the factor sum scores for patient groups in different disease stages and of the differences between these two groups.

	Early ambulatory stage		Late non-ambulatory stage		Difference compared to late-non-ambulatory stage	
Factors (min–max)[a]	*N*	Mean (95% CI)	*N*	Mean (95% CI)	Mean (95% CI)	*p* values
CUE						
Basic hand function (8–56)	64	51.9 (50.6, 53.3)	94	31.0 (27.8, 34.2)	21.0 (16.9, 25.0)	<0.001
Heavy lifting (10–70)	64	55.1 (52.4, 57.8)	94	14.8 (13.1, 16.4)	40.3 (37.3, 43.4)	<0.001
Light or no lifting (12–84)	64	79.0 (77.3, 80.7)	94	27.4 (24.4, 30.3)	51.7 (47.8, 55.5)	<0.001
ABILHAND-plus						
Gross hand function (16–48)	53	45.2 (44.3, 46.0)	88	24.9 (22.9, 26.9)	20.3 (17.6, 23.0)	<0.001
Fine hand function (10–30)	52	25.8 (24.6, 26.9)	91	12.5 (11.7, 13.4)	13.2 (11.8, 14.6)	<0.001
PAIN						
Pain limitations (0–140)	66	1.1 (0.1, 2.1)	94	20.2 (13.7, 26.8)	−19.1 (−27.1, −11.2)	<0.001
Pain severity (not shoulder) (0–120)	66	2.7 (1.1, 4.3)	94	13.9 (9.4, 18.3)	−11.2 (−16.7, −5.7)	<0.001
Distal pain frequency (0–36)	66	0.6 (0.2, 1.0)	94	3.6 (2.4, 4.8)	−3.0 (−4.4, −1.5)	<0.001
Shoulder pain (0–32)	66	1.0 (0.3, 1.7)	94	5.7 (4.5, 7.0)	−4.7 (−6.3, −3.1)	<0.001
Proximal pain frequency (not shoulder) (0–24)	66	0.7 (0.3, 1.2)	94	2.7 (1.8, 3.6)	−1.9 (−3.1, −0.8)	<0.001
Elbow pain frequency (0–12)	66	0.2 (0.0, 0.3)	94	2.0 (1.5, 2.5)	−1.8 (−2.4, −1.2)	<0.001
STIFFNESS						
Stiffness frequency (0–84)	66	3.4 (1.9, 4.9)	94	23.2 (17.8, 28.7)	−19.8 (−26.5, −13.2)	<0.001
Stiffness limitations (0–140)	66	4.6 (-0.2, 9.4)	94	40.6 (31.3, 50.0)	−36.0 (−48.0, −24.1)	<0.001
Stiffness severity (0–140)	66	9.9 (2.5, 17.2)	94	34.9 (27.0, 42.7)	−25.0 (−36.3, −13.7)	<0.001

[a](min–max) = minimal and maximal possible score per factor.

Table 4. The proposed upper limb short questionnaire to assess upper limb function, pain, and stiffness in patients with DMD based on the factor commonalities that can be used as a stepwise approach in clinical practice.

Factor	Initial questions	Score options[a]
Heavy lifting	Do you experience problems in your arms when lifting heavy objects (>5 pounds)?	0: No 1: Yes
Light or no lifting	Do you experience problems in your arms when you reach for or lift light objects such as an empty can?	0: No 1: Yes
Basic hand function	Do you experience problems using your hands for basic functions like manipulating small objects or holding a key?	0: No 1: Yes
Gross hand function	Do you experience problems using your hands when performing daily activities that require gross hand function like washing your hands or eating with a spoon?	0: No 1: Yes
Fine hand function	Do you experience problems using your hands when performing daily activities that require fine hand function like buttoning up your shirt?	0: No 1: Yes
Pain limitations	Do you experience limitations performing daily activities due to pain in your upper limb?	0: No 1: Yes
Pain severity (not shoulder)	How severe is the pain you experience in your upper limb when performing daily activities?	0: No pain 1: Mild or severe pain
Distal pain frequency	How often do you have pain in your hands or fingers?	0: Not more than once a month 1: More than once a month
Shoulder pain	Do you experience pain in your shoulder(s)?	0: No 1: Yes
Proximal pain frequency (not shoulder)	How often do you experience pain in your upper or lower arm?	0: Not more than once a month 1: More than once a month
Elbow pain frequency	How often do you experience pain in your elbows?	0: Not more than once a month 1: More than once a month
Stiffness frequency	How often do you experience stiffness in your arms?	0: Not more than once a month 1: More than once a month
Stiffness limitations	Do you experience limitations performing daily activities due to stiffness in your upper limb?	0: No 1: Yes
Stiffness severity	How severe is the stiffness you experience in your upper limb when performing daily activities?	0: No stiffness 1: Mild or severe stiffness

[a]When the Upper Limb Short Questionnaire is used for the clinical assessment of upper extremity function, pain, and stiffness, additional follow-up questions related to the initial questions can be used to gain detailed insight into the specific problems experienced by an individual patient. Follow-up questions should consist of the items that group under the factor corresponding with the initial question (see also supplementary material).

assessment of upper extremity function, pain, and stiffness, a similar sum score (0–14) can be used, but the follow-up questions related to the initial questions can additionally be used to gain detailed insight into the specific problems experienced by an individual patient and to tailor clinical management.

A limitation of this study is that the results are based on subjective answers, as no clinical tests were performed to verify upper extremity function, pain, or stiffness levels of the respondents. In addition, the use of a questionnaire could lead to interpretation errors, as no researcher could be contacted in the case of uncertainties experienced by respondents. Therefore, the results of this study should be interpreted with care.

The results of this study only apply to patients with DMD and cannot be translated to other populations. The method used in

this study, however, is applicable in other populations, but could result in different factors. Marino et al. 1998 [5], for example, performed exploratory factor analysis on the CUE in patients with tetraplegia and found four subscales that were only partly in line with our research. Nevertheless, we think that the factors and short questionnaire as formulated in this study could be similar for patients with a similar clinical representation, such as patients with other neuromuscular disorders characterized by proximal muscular weakness. The Upper Limb Short Questionnaire can be used as an identifier of arm-hand limitations and the start of more thorough clinical investigation.

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References

[1] Hoffman EP, Brown RH, Jr, Kunkel LM. Dystrophin: the protein product of the Duchenne muscular dystrophy locus. Cell. 1987;51:919–928.

[2] Kohler M, Clarenbach CF, Bahler C, et al. Disability and survival in Duchenne muscular dystrophy. J Neurol Neurosurg Psychiatr. 2009;80:320–325.

[3] Janssen MM, Bergsma A, Geurts AC, et al. Patterns of decline in upper limb function of boys and men with DMD: an international survey. J Neurol. 2014;261:1269–1288.

[4] Eagle M, Baudouin SV, Chandler C, et al. Survival in Duchenne muscular dystrophy: improvements in life expectancy since 1967 and the impact of home nocturnal ventilation. Neuromuscul Disord. 2002;12:926–929.

[5] Marino RJ, Shea JA, Stineman MG. The capabilities of upper extremity instrument: reliability and validity of a measure of functional limitation in tetraplegia. Arch Phys Med Rehabil. 1998;79:1512–1521.

[6] Vandervelde L, Van den Bergh PY, Penta M, et al. Validation of the ABILHAND questionnaire to measure manual ability in children and adults with neuromuscular disorders. J Neurol Neurosurg Psychiatry. 2010;81:506–512.

[7] Brooke MH, Griggs RC, Mendell JR, et al. Clinical trial in Duchenne dystrophy. I. The design of the protocol. Muscle Nerve. 1981;4:186–197.

[8] Salerno DF, Franzblau A, Armstrong TJ, et al. Test-retest reliability of the Upper Extremity Questionnaire among keyboard operators. Am J Ind Med. 2001;40:655–666.

[9] Emery AE. Diagnostic criteria for neuromuscular disorders. London: Royal Society of Medicine Press; 1997.

[10] Bartels B, Pangalila RF, Bergen MP, et al. Upper limb function in adults with Duchenne muscular dystrophy. J Rehabil Med. 2011;43:770–775.

[11] James WV, Orr JF. Upper limb weakness in children with Duchenne muscular dystrophy-a neglected problem. Prosthet Orthot Int. 1984;8:111–113.

[12] Scott OM, Hyde SA, Goddard C, et al. Quantitation of muscle function in children: a prospective study in Duchenne muscular dystrophy. Muscle Nerve. 1982;5:291–301.

[13] Engel JM, Kartin D, Carter GT, et al. Pain in youths with neuromuscular disease. Am J Hosp Palliat Care. 2009;26:405–412.

[14] Tiffreau V, Viet G, Thevenon A. Pain and neuromuscular disease: the results of a survey. Am J Phys Med Rehabil. 2006;85:756–766.

[15] Pangalila RF, van den Bos GA, Bartels B, et al. Prevalence of fatigue, pain, and affective disorders in adults with Duchenne muscular dystrophy and their associations with quality of life. Arch Phys Med Rehabil. 2015;96:1242–1247.