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ORIGINAL ARTICLE

Validity of the Functional Classification of the Upper Extremities for Duchenne Muscular Dystrophy

Yuta Miyazaki, MD, PhD [a,b] Takatoshi Hara, MD, PhD [a,c] Kazuki Hagiwara, MD [a,b]
Takuya Nakamura, MD [a,b] Akiko Kamimura, OT [a] Eri Takeshita, MD, PhD [d] Hirofumi Komaki, MD, PhD [d,e]
Katsuhiro Mizuno, MD, PhD [f] Tetsuya Tsuji, MD, PhD [b] and Masahiro Abo, MD, PhD [c]

Objectives: Some upper-limb function assessments can evaluate treatments in the non-ambulatory stage of Duchenne muscular dystrophy (DMD). The Functional Classification of the Upper Extremities (FCUE) was developed for DMD in Japan. The FCUE is easier to use than the Performance of Upper Limb (PUL) and is more detailed than the Brooke Upper Extremity Scale. This study aimed to determine the concurrent validity of FCUE with other methods of assessment for DMD. **Methods:** This retrospective study reviewed the medical records of 39 boys with DMD from the National Center of Neurology and Psychiatry to evaluate the concurrent validity of the FCUE and PUL using non-parametric Spearman rank correlation (ρ). We also determined the concurrent validity of the Brooke Upper Extremity Scale and PUL for comparison. **Results:** The ρ value between the FCUE and PUL was −0.914 (P<0.001). The FCUE showed robust concurrent validity with the PUL. That correlation between the FCUE and Brooke Upper Extremity Scale gave a ρ value of −0.854 (P<0.001). **Conclusions:** The FCUE had a higher concurrent validity with the PUL than with the Brooke Upper Extremity Scale. The FCUE is considered a valid assessment tool of upper-limb function in boys with DMD. Selecting the best assessment method depends on the severity of the patient's condition and a balance between assessment accuracy and evaluation time.

Key Words: assessment; Brooke Upper Extremity Scale; Duchenne muscular dystrophy; Functional Classification of the Upper Extremities; Performance of Upper Limb

INTRODUCTION

Duchenne muscular dystrophy (DMD) is caused by pathogenic variants in the dystrophin gene, resulting in progressive muscle weakness, gait disturbance, and dysfunction of the upper limb.[1] In recent years, novel antisense oligonucleotide therapies[2] and gene therapies[3] have been developed. Therefore, functional measures that can detect the effectiveness of these therapies are becoming increasingly important.[4] Lower-limb function measures, like the Gross Motor Function Measure,[5] Motor Function Measure,[6] Vignos scale,[7] and 6-min walk test,[8] have been used in boys with DMD who are in the ambulatory stage. The North Star Ambulatory Assessment was previously developed as a disease-specific assessment for DMD.[9] Boys with DMD lose ambulatory functions early in their lives but can maintain upper-limb function for long periods of their lifetimes.[10] However, there are fewer assessments of the upper limb than for the lower limb.[11]

Some upper-limb function assessment methods have

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[a] Department of Rehabilitation Medicine, National Center Hospital, National Center of Neurology and Psychiatry, Tokyo, Japan
[b] Department of Rehabilitation Medicine, Keio University School of Medicine, Tokyo, Japan
[c] Department of Rehabilitation Medicine, The Jikei University School of Medicine, Tokyo, Japan
[d] Department of Child Neurology, National Center Hospital, National Center of Neurology and Psychiatry, Tokyo, Japan
[e] Translational Medical Center, National Center of Neurology and Psychiatry, Tokyo, Japan
[f] Department of Rehabilitation Medicine, Tokai University School of Medicine, Hiratsuka, Japan
Correspondence: Takatoshi Hara, MD, PhD, 4-1-1 Ogawahigashicho, Kodaira, Tokyo 187-8551, Japan, E-mail: t_hara1019@ncnp.go.jp

been reported for the evaluation of treatments in the non-ambulatory stage.[12] The Motor Function Measure is a scale designed to evaluate motor function, including upper-limb function, and the progression of weakness in neuromuscular diseases.[6] The Brooke Upper Extremity Scale[13] and Performance of Upper Limb (PUL)[12,14] are the most used upper-limb functional assessment methods for DMD.[15] The Brooke Upper Extremity Scale is a 6-point scale that can be evaluated in a short time because of the limited number of items.[15] However, the simplicity of the Brooke Upper Extremity Scale means that it cannot assess the progression of muscle weakness of distal muscular dystrophy[16] and that it is insensitive to minor functional changes.[11]

For these reasons, the PUL was designed to separately assess shoulder, forearm, and hand and finger levels.[12] A previous review reported that 35% of previous studies assessed functional outcomes with the PUL.[15] The PUL takes approximately 15 min to perform even though there are many items to be evaluated.[15] However, we believe that the 15-min evaluation time for the PUL is cumbersome in daily clinical practice and fatigues participants. Therefore, there is a need for an assessment method that is simpler than the PUL and more sensitive than the Brooke Upper Extremity Scale.

We focused on the Functional Classification of the Upper Extremities (FCUE) developed in 1983 in Japan. The original FCUE was created as a 9-point assessment method for upper-limb function in DMD.[17] This method uses shoulder and forearm assessment items and does not include hand or finger assessment items as in the Brooke Upper Extremity Scale. To rectify this disadvantage, the FCUE was revised in 2007 to a 13-point scale with the addition of a four-level hand assessment.[18] Inter-rater reliability of the FCUE was reported (kappa=0.91).[19] Because the FCUE is easier to administer than the PUL and more detailed than the Brooke Upper Extremity Scale, it is often used for clinical evaluation in Japan. However, to date, the FCUE has not been fully validated. It is also unclear how best to use the FCUE, Brooke Upper Extremity Scale, and PUL in different settings. Therefore, the aim of this study was to examine the validity and utility of the FCUE as a clinical assessment tool for determining upper-extremity function in boys with DMD. For this purpose, we compared it with the Brooke Upper Extremity Scale and PUL. In addition, because the FCUE requires only a few items and is simple to administer, the FCUE has the potential to be used for motion analysis through an artificial intelligence platform.

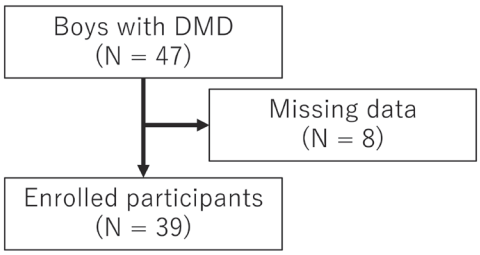

Fig. 1. Flowchart of patient enrollment. Forty-seven boys with DMD were included in this study. The records of 8 boys were missing data from one of the three assessments, leaving 39 participants enrolled in the study.

MATERIALS AND METHODS

Ethics

This study was conducted in accordance with the Declaration of Helsinki and was approved by the Ethics Committee of the National Center of Neurology and Psychiatry (A2021-067). Informed consent was obtained using the opt-out method. Information was provided on the National Center of Neurology and Psychiatry website, and potential participants were able to withdraw from the study.

Study Design and Participants

This was a single-center cross-sectional study, and we retrospectively reviewed electronic medical records of 47 boys with DMD who visited the rehabilitation of the National Center of Neurology and Psychiatry between September 2021 and October 2022. Occupational therapists evaluated the FCUE, Brooke Upper Extremity Scale score, and PUL score and recorded the results in electronic medical records. Eight patients were excluded because of missing data for one of the three assessments. Ultimately, 39 participants were enrolled in the study (**Fig. 1**).

Assessment Procedure

Data on age and FCUE, Brooke Upper Extremity Scale, and PUL scores were collected from electronic medical records. Eight occupational therapists evaluated the participants with three assessments. One occupational therapist was assigned to each participant.

The FCUE is a 13-level scale for evaluating upper-limb motor function and consists of the shoulder level (5 items), elbow level (4 items), and hand and finger level (4 items), as shown in **Table 1**. Administration of the FCUE used a 500-g weight, a 2.7-cm square cube as used in the Kohs Block De-

Table 1. Classification stages of the FCUE

Stage	Description
1	Raise a 500-g weight straight up in the forward direction using the dominant hand
2	Raise a 500-g weight in the dominant hand to 90° forward
3	Raise the dominant hand straight up without weight
4	Raise the dominant hand to 90° forward without weight
5	Flex the elbow greater than 90°
6	Move the hand horizontally forward on the desk by performing elbow extension on the desk
7	Move the hand horizontally forward on the desk by performing elbow extension using trunk movement
8	Move the hand horizontally forward on the desk by performing elbow extension using trunk movement and finger movements
9	Move the hand horizontally forward on the desk with only finger movements
10	Turn over a 10×14.8-cm piece of paper with the better hand
11	Grip the Kohs cube in the opposable thumb position
12	Impossible to grip the Kohs cube in the opposable thumb position, but finger movements are observed
13	No finger movement

Table 2. Participant background characteristics

Characteristic	Mean ± standard deviation (min–max)	Median
Age, years	16.9±7.3 (6–37)	17
FCUE	5.3±3.3 (1–12)	6
Brooke Upper Extremity Scale	3.4±1.9 (1–6)	4
PUL	23.7±2.7 (1–42)	22
Height, cm	140.5±19.2 (103.6–171)	145.5
Weight, kg	45.3±17.8 (19.2–80.2)	42.6

sign Test, and a 10×14.8-cm sheet of paper. First, we assessed whether the participants could raise their upper limb, and, if they could, we evaluated the shoulder level (1–4). When the participants could not raise their upper limb but could flex their elbows, we rated the classification as 5. If participants had difficulty flexing their elbow, they were instructed to extend the elbow on the desk, and we rated the classification between 6 and 10. If the participants had difficulty extending their elbow on the desk, we rated the classification between 10 and 13 based on hand function.

The PUL is an upper-limb functional assessment consisting of 22 test items.[12] We used version 2.0 in this study. Each item was divided into three major dimension levels (shoulder, elbow, and hand and finger items), and each test item was rated in terms of patient performance on a scale of 0–2. The Brooke Upper Extremity Scale is a six-level index of the proximal upper limb.[13]

Statistical Analysis

We calculated the respective non-parametric Spearman rank correlation coefficients (ρ) to evaluate the concurrent validity between the FCUE and PUL, between the Brooke Upper Extremity Scale and PUL, and between the FCUE and the Brooke Upper Extremity Scale. We also calculated the Spearman correlation coefficient between age and the FCUE, between age and the Brooke Upper Extremity Scale, and between age and the PUL. Statistical significance was set at $P<0.003$ using Bonferroni correction. All statistical analyses were performed using SPSS version 27 (IBM, Armonk, NY, USA).

RESULTS

Participants

We enrolled 39 participants after excluding those with missing data (mean age, 16.9 years; **Table 2**). The mean scores for the FCUE, Brooke Upper Extremity Scale, and PUL were 5.3, 3.4, and 23.7, respectively. The following Spearman correlation coefficients were observed: between age and FCUE, 0.670 ($P<0.001$); between age and the Brooke Upper Extremity Scale, 0.616 ($P<0.001$); between age and the PUL, −0.697 ($P<0.001$).

Table 3. Spearman rank correlation coefficients for comparison of evaluation methods

Correlation	Spearman ρ value	P value	95% confidence interval
FCUE and PUL	−0.914	<0.001	−0.955 to −0.838
Brooke Upper Extremity Scale and PUL	−0.854	<0.001	−0.923 to −0.73
FCUE and Brooke Upper Extremity Scale	0.882	<0.001	0.781 to 0.938

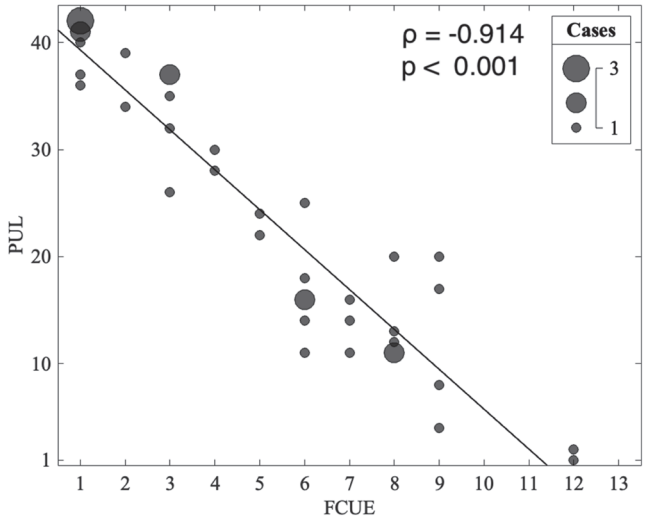

Fig. 2. Correlation between FCUE and PUL. The FCUE and PUL were significantly correlated (ρ=−0.914, P<0.001).

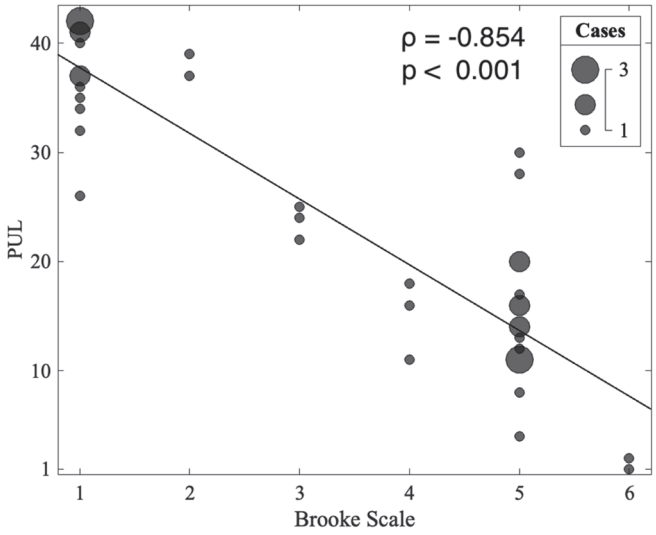

Fig. 3. Correlation between Brooke Upper Extremity Scale and PUL. The Brooke Upper Extremity Scale and PUL were significantly correlated (ρ=−0.854, P<0.001).

Concurrent Validity of FCUE and PUL

A significant negative correlation was observed between the FCUE and PUL. The ρ value was −0.914 (P<0.001, **Table 3**). **Figure 2** shows a validity scatterplot of the FCUE and PUL.

Concurrent Validity of Brooke Upper Extremity Scale and PUL

The ρ value between the Brooke Upper Extremity Scale and PUL was −0.854, which indicated a significant negative correlation (P<0.001, **Table 3**). The correlation between the FCUE and PUL was more substantial than that between the Brooke Upper Extremity Scale and PUL. **Figure 3** shows a validity scatterplot of the Brooke Upper Extremity Scale and PUL.

Concurrent Validity of FCUE and Brooke Upper Extremity Scale

The ρ value between the FCUE and the Brooke Upper Extremity Scale was 0.882, which indicated a significant positive correlation (P<0.001, **Table 3**). **Figure 4** shows a validity scatterplot of the FCUE and the Brooke Upper Extremity Scale.

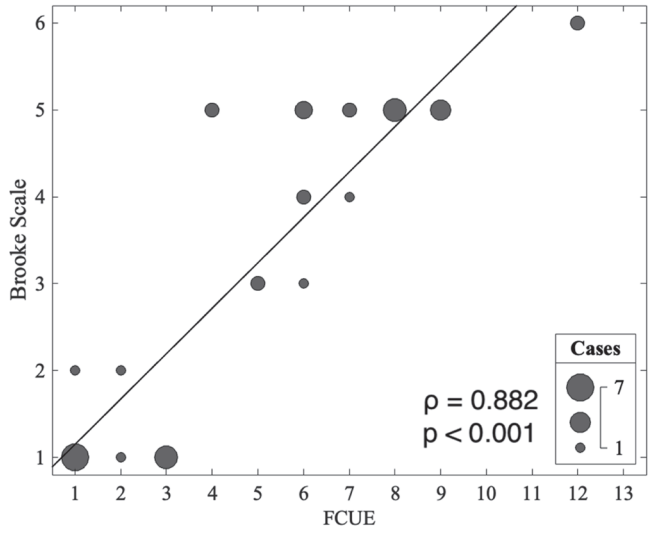

Fig. 4. Correlation between FCUE and Brooke Upper Extremity Scale. The FCUE and Brooke Upper Extremity Scale were significantly correlated (ρ=0.882, P<0.001).

Distribution of Participants

According to the FCUE, the participants were divided evenly across the range of possible scores, although no patients showed scores of 10, 11, or 13 (**Fig. 2**). According to the Brooke Upper Extremity Scale, 13 participants had a score of 1 and 16 participants had a score of 5 (**Fig. 3**).

DISCUSSION

This study focused on establishing the validity of the FCUE as a simple new index for the assessment of upper-limb function in boys with DMD. The FCUE is a simple scale that can be evaluated within 1–2 min and causes less fatigue in participants than the PUL. In addition to shoulder and elbow functions, it is also designed to determine the functions of the hands and fingers like the PUL. Therefore, this study aimed to clarify the concurrent validity of the FCUE and PUL, which are representative upper-limb function assessment indices for DMD. The results of this study suggest that the FCUE has sufficient concurrent validity with the PUL. In addition, the concurrent validity of the upper-limb impairment rating with the PUL was more robust than that of the Brooke Upper Extremity Scale with the PUL.

Correlation between FCUE and PUL

The ρ value between the FCUE and PUL was −0.914, indicating a strong correlation. At the beginning of this study, we were concerned that the smaller number of assessment items in the FCUE than in the PUL would reduce the concurrent validity. However, the results showed robust concurrent validity between the FCUE and PUL. Reportedly, the PUL can reduce the floor effect in assessments of boys with DMD older than 15 years because it can assess small movements of distal upper-limb function that remain until later stages of the disease.[14] The FCUE can assess shoulder, forearm, hand, and finger levels, which provide sufficient concurrent validity with the PUL. Therefore, the FCUE has limited floor and ceiling effects and has the potential to evaluate severe DMD.

Comparison between Brooke Upper Extremity Scale and PUL

In comparing the correlation of the FCUE and the PUL with that of the Brooke Upper Extremity Scale and the PUL, the higher ρ value of the former correlation (−0.914) indicates that the concurrent validity of FCUE with the PUL is greater than the concurrent validity of the Brooke Upper Extremity Scale with the PUL. There are two possible reasons for this result. First, like the PUL, the FCUE includes shoulder, forearm, and finger and hand evaluation items that are not included in the Brooke Upper Extremity Scale. Second, the 13-level FCUE provides a more detailed assessment than the six-level Brooke Upper Extremity Scale. The Brooke Upper Extremity Scale is easier to use in clinical and research settings owing to a quicker examination time, but a previous study reported that the Brooke Upper Extremity Scale is less sensitive to functional changes than the FCUE.[11] For these reasons, the FCUE was less subject to ceiling and floor effects than the Brooke Upper Extremity Scale and had greater concurrent validity with the PUL. **Figure 3** shows that 13 participants had a Brooke Upper Extremity Scale score of 1 and 16 participants had a Brooke Upper Extremity Scale score of 5, indicating ceiling and floor effects in the assessments. With the development of treatment for boys with DMD and rehabilitation medicine focusing on motor function, we reaffirmed the need for the assessment of distal upper-limb function.

Comparison between FCUE and Brooke Upper Extremity Scale

We also compared the concurrent validity of the FCUE and the Brooke Upper Extremity Scale. The ρ value was significant for the correlation between the FCUE and the Brooke Upper Extremity Scale. Although multiple participants scored 5 on the Brooke Upper Extremity Scale, the FCUE scores for the same participants ranged from 4 to 9, indicating that the FCUE resolved the ceiling effect. Similarly, multiple participants scored 1 on the Brooke Upper Extremity Scale, but the FCUE scores of these participants ranged from 1 to 3, suggesting that the FCUE improved the floor effect. Therefore, we consider that the FCUE provided a more detailed assessment of upper-extremity function with fewer ceiling and floor effects than the Brooke Upper Extremity Scale.

Correlation between Age and Each Assessment Method

The Spearman correlation coefficient between age and the Brooke Upper Extremity Scale was 0.616 ($P<0.001$). From this result, the coefficient of determination was calculated to be 0.379. A previous study[20] reported that the Spearman correlation coefficient between age and the Brooke Upper Extremity Scale was 0.435. Therefore, the correlation between age and the Brooke Upper Extremity Scale observed in this study is similar to that in previous research, as were our participants. The results of our study suggest that the FCUE and PUL also reflect some degree of declining function with age.

Utility of FCUE

The FCUE has the potential for easy use in daily clinical practice. Although the life expectancy of boys with DMD has increased to 28 years for those born after 1990,[21] those treated with steroids for more than 1 year also lose the ability to walk at about 13 years of age.[22] Therefore, in addition to the current mainstream gait-function assessment, the FCUE will become increasingly important for evaluating the effect of treatment after the loss of gait function.

The FCUE can be performed in 1–2 min and is more accessible than the PUL, which takes approximately 15 min.[15] The evaluation time of the FCUE is slightly longer than that of the Brooke Upper Extremity Scale. Given that time is limited in daily clinical practice, ease of evaluation is a critical factor. Furthermore, the functional outcome measures used in antisense oligonucleotide therapies are influenced by motivation and fatigue, which can be limiting factors.[23] Therefore, fast evaluation methods like the FCUE are less susceptible to motivation and fatigue than the time-consuming PUL.

One critical point is the distinction between the FCUE and PUL. We consider that the FCUE is suitable for functional stage classification of boys with DMD, routine clinical follow-up, and inclusion criteria in research studies because of its shorter assessment time than the PUL. However, the PUL is useful when a detailed evaluation of change is needed, such as in clinical trials or when checking for improvement in upper-limb function.

Limitations

The present study has several limitations. First, this was a single-center retrospective study, and some data were missing. Second, this study was conducted with outpatients; some participants had preserved hand function, and there were no participants with FCUE scores of 10, 11, or 13. Therefore, we should evaluate more patients in future studies. Third, Rasch analysis or other methods are considered necessary to examine the validity of the FCUE, but these analyses could not be performed in this study because of the number of participants. Fourth, this study could not determine the inter-rater reliability because only one examiner evaluated each patient. Lastly, because this study evaluated participants at a single timepoint, changes in clinical findings could not be assessed. Therefore, we will examine the same participants over time to determine whether clinical changes can be followed up in the future.

Conclusions

The results of this study showed that the concurrent validity between FCUE and PUL is higher than that between the Brooke Upper Extremity Scale and the PUL. Therefore, the FCUE can be considered a valid evaluation index of upper-limb function in boys with DMD. In evaluating upper-limb function, it is essential to understand the characteristics of each method and to select the best one based on the severity of the patient's condition and the compromise between accuracy and evaluation time.

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CONFLICTS OF INTEREST

KM has received annual payments from the General Insurance Association of Japan. HK has received research grants from Chugai Pharmaceutical and Taiho Pharmaceutical. ET has received research grants from Daiichi Sankyo, Nippon Shinyaku, Taiho Pharmaceutical, and Takeda Pharmaceutical. HK has received lecture fees from Chugai Pharmaceutical, Nippon Shinyaku, Biogen Japan, Novartis, and Pfizer. The remaining authors declare no conflict of interest.

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