

Development and validation of a muscular dystrophy-specific functional rating scale

Yi-Jing Lue Faculty of Physical Therapy, College of Health Science, Kaohsiung Medical University, **Chwen-Yng Su** Faculty of Occupational Therapy, College of Health Science, Kaohsiung Medical University, **Rei-Cheng Yang** Faculty of Sport Medicine, College of Medical Science, Kaohsiung Medical University, **Wei-Lieh Su** Faculty of Occupational Therapy, College of Health Science, Kaohsiung Medical University, **Yen-Mou Lu** Department of Orthopaedic Surgery, Kaohsiung Medical University Hospital, **Rong-Fong Lin** Faculty of Physical Therapy, College of Health Science, Kaohsiung Medical University, Kaohsiung and **Shun-Sheng Chen** Department of Neurology, Kaohsiung Chang Gung Memorial Hospital, Kaohsiung County, Taiwan

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Objective: To develop a reliable and valid new rating scale for measuring the functional impact of muscular dystrophy.

Design: Prospective and longitudinal investigation.

Setting: Three academic medical centres in Taiwan and the Muscular Dystrophy Association of Taiwan.

Measures: The Brooke Scale, the Vignos Scale, the Barthel Index, muscular strength, contracture severity, and predicted forced vital capacity (FVC%).

Methods: Scale development was in three stages. In stage I, a preliminary pool of 53 items was generated from patient interviews ($n = 25$), literature review, existing functional rating scales and expert opinion. In stage II, these items were administered to 85 patients with muscular dystrophy. The resulting data were analysed to construct a rating scale (the Muscular Dystrophy Functional Rating Scale, MDFRS) that encompassed four unidimensional constructs: mobility, basic activities of daily living, arm function and impairment. In stage III, the measurement properties of this rating scale were assessed in 121 muscular dystrophy patients different from those examined with the preliminary instrument.

Results: Internal consistency reliability was excellent for all domains of the final 33-item scale, with values of Cronbach's alpha ranging from 0.84 to 0.97. Intraclass correlation coefficients for test–retest and inter-rater reliability were 0.99 for all domains of the MDFRS. The MDFRS showed moderate to high correlations with a range of functional rating scales measuring similar aspects and impairment parameters (Spearman's $\rho = 0.65$ – 0.91 ; $P < 0.001$, each). Confirmatory factor analysis supported a unitary construct of the four-dimensional MDFRS. The MDFRS had small floor and ceiling effects in the study samples. Sensitivity to change was confirmed by large standardized response means for the MDFRS total score.

Conclusions: The MDFRS is a reliable and valid disease-specific measure of functional status for patients with muscular dystrophy.

Address for correspondence: Shun-Sheng Chen, Department of Neurology, Kaohsiung Chang Gung Memorial Hospital, No. 123, Ta-Pei Road, Niao-Sung Hsiang, Kaohsiung County, Taiwan. e-mail: yijilu@cc.kmu.edu.tw

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Introduction

Muscular dystrophy is an inherited disease marked by progressive weakness and degeneration of the skeletal or voluntary muscles. Regardless of the dystrophic types, functional deficits common to this population include joint contracture, limited mobility, poor respiratory function, as well as impaired activities of daily living.^{1,2}

In the clinical and research realms, the Brooke and Vignos functional scales are the most frequently used outcome measures for muscular dystrophy, although initially developed for use with Duchenne muscular dystrophic patients.^{3,4} They are simple to administer and score, and the reliability and validity have been well documented.⁵ However, the scope of both scales is restricted to assessing levels of function for the upper and lower extremities only. Taking into account that the goals of rehabilitation in dystrophic patients are to maximize functional capacities, improve or maintain capability for independence and self-care, reduce or prevent significant deformity, and promote quality of life, a comprehensive evaluation of the varied clinical problems associated with muscular dystrophy is of paramount importance to quantify accurately patient's functional status and to serve as basis for evidence-based disease management. In the absence of such a measure, a decision was taken to develop a disease-specific rating scale, which covered functional disturbances that have a profound impact on dystrophic patients, and to investigate its psychometric properties.

Methods

The development of the Muscular Dystrophy Functional Rating Scale (MDFRS) followed widely accepted development procedures for rating scales: item generation, item reduction and scale formation, and scale evaluation.⁶ The study was approved by the hospital's institutional review board, and written informed consent was obtained from all participants.

Stage I: item generation

Exploratory semi-structured interviews were first conducted with 25 patients by the principal

investigator. Patients were asked to indicate relevant aspects of life adversely affected by muscular dystrophy, as well as the extent to which they have experienced these specific functional limitations and symptoms. In addition, a computer search was carried out of MEDLINE, Ovid and EBSCO databases from 1966 to 2002 using the search terms *muscular dystrophy or neuromuscular diseases or activities of daily living or functional evaluation*. Approximately 20 publications were produced by this literature search. Based on these data, coupled with a review of existing functional rating scales,⁷⁻¹⁰ and discussions with the experts in the field of neurology and rehabilitation, we developed preliminary item pool grouped into clinically meaningful dimensions to produce a conceptual model of the functional impact of muscular dystrophy. Table 1 lists the items in the preliminary MDFRS drawn from diverse sources.^{2,3,8-16}

Stage II: item reduction and scale formation

The item pool was first administered to 85 dystrophic patients from three large academic medical centres in Taiwan and the Taiwan Muscular Dystrophy Association. The inclusion criteria were as follows: (1) age 6 years and above, and (2) a definite diagnosis of muscular dystrophy confirmed by two neurologists on the basis of clinical, EMG and muscle biopsy criteria in use at that time. Patients were excluded if they had other coexisting medical or psychiatric diseases. Over half of our pilot sample was diagnosed with Duchenne muscular dystrophy, followed by limb-girdle muscular dystrophy, Becker muscular dystrophy, and facioscapulohumeral muscular dystrophy (Table 2). To ensure that our patient sample presented with a wide spectrum of the severity of dysfunction in upper and lower limbs, the Brooke and Vignos Scales were used. The number of patients for the six grades of the Brooke Scale from 1 (highest) to 6 (lowest) was 22, 20, 8, 7, 26, and 2, respectively. On the Vignos Scale, the number of patients for the 10 grades from 1 (able to walk) to 10 (bed bound) was 3, 11, 10, 4, 3, 0, 1, 1, 46, and 6, respectively. The fact that none or few of the patients were in the sixth, seventh, and eighth grades of the Vignos Scale could be attributed to the cultural-specific attitudes, in which dystrophic patients were unwilling to be prescribed long-leg braces for various reasons

Table 1 Sources for developing the preliminary MDFRS items

Domain	Number of items	Item content	Sources
Mobility	12	Running Stair climbing Outdoor mobility Indoor mobility Transferring from bed to chair Transferring from wheelchair to toilet Transferring from wheelchair to bathtub Wheelchair manipulation Standing from sitting Sitting from lying Rolling Changing body position in bed	Barthel Index ¹⁰ FIM ⁸ ICF ¹¹ ALSFRS-R ⁹
Basic ADL	13	Feeding Swallowing Combing hair Brushing teeth Cutting fingernail Cutting toenail Put on /take off formal dresses Dressing upper part of body Dressing lower part of body Toileting Bathing Bladder control Bowel control	Barthel Index FIM ICF PSMS ¹² IADL ¹² ALSFRS-R
Arm function	11	Managing objects over head Carrying objects Heavy home maintenance tasks Washing clothes Cleaning table Food preparation Bilateral lifting Writing Turning books Picking up small objects Manipulating small objects	Brooke Scale ³ ICF IADL ALSFRS-R
Impairment	17	Severity of upper limb joint contractures Severity of lower limb joint contractures Number of contracted joints in the upper limbs Number of contracted joints in the lower limbs Severity of neck contracture Strength of neck Severity of trunk contracture Strength of trunk Scoliosis Orthopnoea Dyspnoea after activity Sputum clearance Ventilator assisted Number of pain areas Frequency of pain Intensity of pain Pain-inducing activities	ICF ALSFRS-R Fowler, 1995 ² McDonald, 1995 ¹³ McDonald, 1995 ¹⁴ McDonald, 1995 ¹⁵ Kilmer, 1995 ¹⁶

MDFRS, Muscular Dystrophy Functional Rating Scale; Basic ADL, basic activities of daily living. FIM, Functional Independence Measure; ICF, International Classification of Functioning, Disability and Health; ALSFR-R, Revised-Amyotrophic Lateral Sclerosis Functional Rating Scale; PSMS, Physical Self-Maintenance Scale; IADL, Instrumental Activities of Daily Living Scale.

Table 2 Patient characteristics

Characteristics	Stage I	Stage II	Stage III	
	Semi-structured interviews	Item reduction/scale formation	Reliability/validity	Factor analysis
Number	25	85	51	121
Age (years; mean $\pm$ SD/range)	25.3 $\pm$ 14.1/7–60	21.1 $\pm$ 12.8/6–60	21.6 $\pm$ 13.6/7–61	20.0 $\pm$ 12.4/6–61
Gender (male/female)	20/5	69/16	44/7	101/20
Disease duration (years; mean $\pm$ SD/range)	14.7 $\pm$ 6.5/2–34	13.2 $\pm$ 7.7/2–39	13.6 $\pm$ 8.0/2–39	12.4 $\pm$ 8.1/2–42
Type distribution (number/%)				
DMD	15/60%	54/63.5%	36/70.6%	79/65.3%
BMD	2/8%	9/10.6%	6/11.8%	13/10.7%
LGMD	5/20%	14/16.5%	4/7.8%	21/17.4%
FSHMD	3/12%	8/9.4%	5/9.8%	8/6.6%
Brooke Scale (median/range)	4/1–6	3/1–6	5/1–6	2/1–6
Vignos Scale (median/range)	9/2–10	9/1–10	9/1–10	9/1–10

SD, Standard deviation; DMD, Duchenne muscular dystrophy; BMD, Becker muscular dystrophy; LGMD, limb-girdle muscular dystrophies; FSHMD, facioscapulohumeral muscular dystrophy.

(e.g. inconvenience, poor brace design, and joint contracture of the lower limb).

The completed data were subjected to several item analysis techniques, including item reliability, principal components analysis, and item discrimination analysis. The decision rules leading to the retention or elimination of items include: (1) the corrected item-total correlation of each item with the dimension score had to be larger than 0.3; (2) the value of Cronbach's α should not decrease substantially when the item within each dimension was dropped; (3) the communality values of all items within each dimension had to be greater than 0.4; and (4) the index of discrimination of each item within each dimension had to be larger than 0.2. The discrimination index is a comparison of how overall high scorers on the whole test did on one particular item compared to overall low scorers. For inclusion, items had to reflect a problem that is relevant and important to dystrophic patients, irrespective of age or sex, and that affects tasks, that are performed by all subjects at some functional level.

After item reduction, the sets of remaining items within each dimension were submitted separately to a principal components analysis (PCA) to see if any of the dimensions was multidimensional. An exploratory principal components analysis was again performed to determine the dimensionality of the MDFRS.

Stage III: scale evaluation

The reliability, validity and responsiveness of the final scale thus developed were assessed in a second study of 121 dystrophic patients different from the patients assessed in stage II. The internal consistency of both total and dimension scores of the scale was quantified by Cronbach's α in the total sample of 121 dystrophic patients. Test-retest reliability over a one-week time span and inter-rater reliability with two pairs of four trained raters were evaluated by administering the test to a group of 51 patients randomly selected from the total sample. The raters were experienced physical and occupational therapists. They were required to participate in a one-day training session led by the principal investigator. During training, particular attention was drawn to the items' explicit nature and the grading criteria. Raters watched a video on how dystrophic patients performed the daily activities; and afterwards, follow-up discussion was held to illustrate assessment techniques and correct grading based on the patients' responses. To meet the competency requirement in MDFRS assessment, each rater completed a case and met individually with the lead trainer for review to ensure correctness and appropriateness in administering and scoring the MDFRS. Raters were randomly paired, and study patients were randomly allocated to each pair of raters, who were blinded to the results of each others' assessments in the inter-rater trials. Intraclass correlation coeffi-

cients (ICCs) were used to measure inter-rater and test–retest reliabilities.

The convergent validity of the final scale with the Barthel Index,¹⁰ the Brooke Scale,³ and the Vignos Scale⁴ was assessed in the same sample for reliability analyses by Spearman rank correlation coefficients. The construct validity was established in the above sample by investigating correlations among the final scale, muscle strength, contracture severity and pulmonary function. In order to verify the factor structure of the MDFRS obtained in the scale formation stage, a confirmatory factor analysis was performed in the total sample.

The responsiveness of the MDFRS, the Brooke Scale³ and the Vignos Scale⁴ was examined using the same 51 patients from the reliability and validity analyses, who were followed for at least six months. Responsiveness refers to the ability of a scale to detect clinically meaningful changes over time.¹⁷ Standardized response mean (SRM) provides a standardized measurement of the change in score of an instrument and was used in the present study to measure responsiveness of the above three instruments. SRM was computed as the mean of the change scores divided by the standard deviation (SD) of the change scores.¹⁸ SRM can be considered large (> 0.8), moderate (0.5–0.8) or small (0.2–0.5).¹⁹

Measures

*Barthel Index*¹⁰

The Barthel index is a 10-item ordinal scale that measures functional independence in the domains of personal care and mobility. For each item, patients receive a score of 0 if entirely unable to carry out the activity, a maximum score if able to perform the activity independently, and a partial score if able to perform the activity with assistance. Possible values are assigned in increments of 5. The totals can range from 0 to 100.

Brooke and Vignos Scales^{3,4}

The Brooke and Vignos Scales were used to grade upper and lower extremities function, respectively. In the Brooke Scale, the score range is from 1 to 6. The Vignos Scale is an ordinal scale, ranging from 1 to 10 points where 1 means that the patient is able to walk and climb stairs without

assistance, and 10 represents that the patient is bed-bound.

Muscle strength

Manual muscle testing of 30 muscle groups involving neck, and upper and lower extremities was carried out by physical therapists. A modified Medical Research Council scale was employed according to the method described by Kilmer *et al.*²⁰ On this scale, 5 represents normal strength and 0 no muscle movement. Mean scores attained for total muscle grade were used in the analysis.

Contracture severity

Range of motion for the following joint movements was evaluated using a goniometer: shoulder abduction, elbow extension, wrist extension, hip adduction, hip extension, knee extension and ankle dorsiflexion. Contracture was defined as a 5° or more loss of joint range of motion.²¹ Mean maximal loss of range in degrees for all seven joints was calculated.

Pulmonary function

Pulmonary function test was done with a spirometer (SpiroPro, SensorMedics Corporation, Yorba Linda, CA, USA). The testing procedure was carried out in accordance with the guidelines recommended by the American Thoracic Society.²² Percentage predicted forced vital capacity was computed.

Results

Stage I: item generation

A review of the literature, patient interviews and discussions with the experts in the field led to the identification of four broad clinically appropriate domains: mobility, basic activities of daily living, arm function and impairment. According to Wade, mobility was defined as change of location, such as transfers, walking, wheelchair and stairs.²³ Basic activities of daily living referred to routine activities that are oriented toward taking care of one's own body, such as bathing, dressing, feeding, etc. Measurement of arm function pertained to voluntary motor control, and specific as well as general arm abilities. Impairment refers to significant deviation in body structures, which often mani-

fested itself in the areas such as muscle strength, range of motion and pulmonary function.²³ Using these dimensions as content areas, items were framed to index the functional disability in dystrophic patients. As a consequence, a total of 53 items were generated. For each item, a four-point grading system was devised that conveyed a likely range of qualitatively different degrees of performance in the rating scale, starting from 1 (total dependence) to 4 (independence). The demographic and clinical profile of the patients is detailed in Table 2.

Stage II: item reduction and scale formation

In accordance with the above-stated item selection principles, 3 of the 12 items in the mobility domain, 7 of the 13 items in the basic activities of daily living domain, 4 of the 11 items in the arm function domain, and 6 of the 17 items in the impairment domain were deleted. The remaining items within each domain were then submitted to principal components analysis (PCA) to test the unidimensionality of each domain, the results of which are highlighted in Table 3. Because of the lack of a commonly acceptable definition of unidimensionality, the criteria for a single-factor solution was as follows: a first factor eigenvalue was notably larger than the second, a first factor that accounts for at least 50% of the variance, and no negative loadings on the first factor.^{24,25} For the mobility domain, a PCA generated one component with an eigenvalue of 7.02, accounting for 78% of the variance. The range of communality values was high for all items (0.63 to 0.90), suggesting that a large proportion of the variance in the items was accounted for by the factor. Factor loadings (component loadings in PCA) ranged from 0.80 to 0.95. From these analyses it could therefore be concluded that the nine-item mobility domain pertaining to activities such as rolling, sit-to-stand, climbing stairs and wheelchair manipulation was unidimensional. For the basic activities of daily living domain, the PCA yielded one factor with an eigenvalue of 4.62, explaining 77.1% of the variance. As apparent in Table 3, the factor loadings of the items were 0.82 or higher, and the communalities ranged from 0.66 to 0.83. As a result, it was concluded that the six-item basic activities of daily living domain related to

Table 3 Results of principal components analysis for the 33-item MDFRS after item deletion (stage II) ($n = 85$)

MDFRS	Factor loadings	Communalities
Mobility domain		
M1 (Stair climbing)	0.797	0.635
M2 (Outdoor mobility)	0.873	0.763
M3 (Indoor mobility)	0.916	0.839
M4 (Transferring from bed to chair)	0.951	0.904
M5 (Wheelchair manipulation)	0.796	0.633
M6 (Standing from sitting)	0.913	0.834
M7 (Sitting from lying)	0.910	0.827
M8 (Rolling)	0.884	0.782
M9 (Changing body position in bed)	0.895	0.802
BADL domain		
B1 (Feeding)	0.815	0.664
B2 (Combing hair)	0.878	0.770
B3 (Brushing teeth)	0.890	0.792
B4 (Dressing upper/lower parts of body)	0.908	0.825
B5 (Toileting)	0.864	0.747
B6 (Bathing)	0.909	0.826
Arm function domain		
A1 (Managing objects over head)	0.765	0.899
A2 (Carrying objects)	0.629	0.727
A3 (Cleaning table)	0.753	0.703
A4 (Writing)	0.710	0.525
A5 (Turning books)	0.842	0.869
A6 (Picking up small objects)	0.675	0.645
A7 (Manipulating small objects)	0.754	0.767
Impairment domain		
I1 (Severity of upper limb joint contractures)	0.804	0.668
I2 (Severity of lower limb joint contractures)	0.755	0.754
I3 (Number of contracted joints in the upper limbs)	0.809	0.681
I4 (Number of contracted joints in the lower limbs)	0.710	0.745
I5 (Severity of neck contracture)	0.803	0.666
I6 (Strength of neck)	0.725	0.529
I7 (Strength of the trunk)	0.820	0.676
I8 (Scoliosis)	0.806	0.666
I9 (Orthopnea)	0.530	0.749
I10 (Sputum clearance)	0.546	0.567
I11 (Ventilator assisted)	0.546	0.721
Total scale		
Mobility domain	0.924	0.854
BADL domain	0.964	0.929
Arm function domain	0.903	0.816
Impairment domain	0.880	0.775

MDFRS, Muscular Dystrophy Functional Rating Scale; BADL, basic activities of daily living.

activities such as grooming, feeding, dressing, toileting, along with bathing was unidimensional.

For the arm function domain, a PCA of all domain items identified two components with eigenvalues of 3.79 and 1.35, explaining 54.1% and 19.3% of the variance, respectively. However, evidence for a single factor consisted of a first factor eigenvalue 2–3 times greater than the second, a first factor that accounts for over 50% of the variance, all items with loadings above 0.63 on the first factor, and no negative loadings on the first factor. In addition, the communalities of seven items within arm function domain were all well above 0.50 (i.e. they ranged from 0.53 to 0.90). Loadings of the items on the first factor ranged from 0.63 to 0.84. Taken together, it was concluded that the seven-item arm function domain concerning functional tasks that measure movements of upper limbs was unidimensional. For the impairment domain, the PCA also extracted two components with eigenvalues of 5.74 and 1.68, accounting for 52.2% and 15.3% of the variance, respectively. Evidence for a single factor consists of a first factor eigenvalue 4–5 times greater than the second, a first factor that accounts for at least 50% of the variance, and no negative loadings on the first factor. Moreover, the communalities of the 11 items ranged from 0.53 to 0.75, and the factor loadings were 0.53 or higher. Accordingly, it was concluded that the 11-item impairment domain that taps range of motion and strength in the neck and trunk muscles, scoliosis, as well as respiratory function, was unidimensional. Cronbach's α coefficients were calculated in order to assess the internal consistency of the resulting domains. The results show that Cronbach's α for the mobility, basic activities of daily living, arm function, and impairment domains were 0.96, 0.94, 0.84, and 0.90, respectively.

To assess the underlying factor structure within the common core of four domains, a PCA was applied again. As shown in Table 3, a robust single factor was produced with an eigenvalue of 3.37, accounting for 84.4% of the variance. Factor loadings were all high (i.e. they ranged from 0.88 to 0.96), as were the communalities (i.e. ranging from 0.78 to 0.93). Overall, the results supported one unitary construct of the MDFRS.

Stage III: scale evaluation

Descriptive statistics

Descriptive statistics of the final MDFRS assessed at baseline are described in Table 4, both for the total sample and for Duchenne's, Becker's, facioscapulohumeral and limb-girdle muscular dystrophies separately. Patients participating in this stage fulfilled the inclusion criteria similar to those for stage II. The results showed that Duchenne's patients had the worst performance on the MDFRS in comparison with the other three groups. There were no relevant ceiling and floor effects for the total MDFRS score across different subtypes of muscular dystrophy. In terms of the individual domain scores, significant ceiling effects were found only for basic activities of daily living domain in patients with Becker's and limb-girdle muscular dystrophies.

Reliability properties

Internal consistency reliability was satisfactory for the final 33-item MDFRS (Appendix 1) as a whole and for each of its domains in 121 dystrophic patients, with values of Cronbach's α ranging from 0.84 to 0.97. Test–retest reliability was high; ICCs reached values of 0.99 for the total MDFRS and four domain scores. The ICCs for inter-rater reliability achieved 0.98 for all domain and the total scores, as well. The results of these analyses are displayed in Table 5.

Construct validities

Analysis of construct validity demonstrated moderate to high correlations of the MDFRS total score with Barthel Index (Spearman's $\rho = 0.91$), Brooke Scale (Spearman's $\rho = -0.75$), and Vignos Scale (Spearman's $\rho = -0.90$). Moderate to high correlations of the MDFRS total score with muscle strength (Spearman's $\rho = 0.89$), contracture severity (Spearman's $\rho = -0.82$), and pulmonary function test (Spearman's $\rho = 0.65$) were achieved. All the correlation coefficients reached great significant level ($P < 0.001$).

Dimensional structure of the MDFRS

A confirmatory factor analysis was performed to ascertain the latent structure of the MDFRS by means of version 4.0 of the Analysis of Moment

Table 4 Descriptive statistics and ceiling effects of the MDFRS in stage III of the study

MDFRS (no. of items)	Total sample (<i>n</i> = 121)	DMD (<i>n</i> = 79)	BMD (<i>n</i> = 13)	LGMD (<i>n</i> = 21)	FSHMD (<i>n</i> = 8)
Total score (33)					
Mean (SD)	88.5(25.0)	82.1(24.2)	100.7(22.9)	102.1(23.4)	92.3(19.1)
Floor effects	0%	0%	0%	0%	0%
Ceiling effects	0.8%	1.3%	0%	0%	0%
Mobility domain (9)					
Mean (SD)	18.9(9.0)	16.8(8.9)	21.9(8.6)	23.6(8.4)	22.1(6.3)
Floor effects	14%	20.3%	0%	4.8%	0%
Ceiling effects	0.8%	1.3%	0%	0%	0%
BADL domain (6)					
Mean (SD)	14.8(6.0)	12.9(5.5)	19.2(5.1)	18.7(5.4)	16.5(4.8)
Floor effects	5%	7.6%	0%	0%	0%
Ceiling effects	9.9%	2.5%	30.1%	28.6%	0%
Arm domain (7)					
Mean (SD)	20.9(4.9)	19.7(4.9)	23.8(4.0)	23.1(4.4)	22.6(3.9)
Floor effects	2.5%	3.8%	0%	0%	0%
Ceiling effects	6.6%	2.5%	15.4%	19.0%	0%
Impairment domain (11)					
Mean (SD)	33.8(7.6)	32.7(7.9)	35.8(7.4)	36.9(6.5)	34.0(6.1)
Floor effects	0%	0%	0%	0%	0%
Ceiling effects	3.3%	3.8%	0%	4.8%	0%

MDFRS, Muscular Dystrophy Functional Rating Scale; DMD, Duchenne muscular dystrophy; BMD, Becker muscular dystrophy; LGMD, limb-girdle muscular dystrophies; FSHMD, facioscapulohumeral muscular dystrophy; BADL, basic activities of daily living; SD, standard deviation.

Structures software.²⁶ Confirmatory factor analysis examines patterns of covariation among tests to determine if these patterns are congruent with the theoretically specified constructs.²⁷ The hypothesized model of the MDFRS was a hierarchical model with four latent variables (four domains). To create domain latent variables, item parcels were prepared. These item parcels consisted of items that were homogeneous with respect to intercorrelations. Two item parcels were constructed for each of the domains and were used to create the domain latent variable. Several indices were used to assess the goodness of fit of the model: the normed fit index (NFI), the Tucker–Lewis nonnormed fit index (TLI), the standardized root mean square residual (SRMR), and the comparative fit index (CFI). Conventional interpretation for these fit indices is that values of 0.90 or greater indicate excellent correspondence between the hypothetical model and the actual data and values between 0.85 and 0.90 indicate reasonable model fit.²⁸ As for SRMR, the value < 0.08 represents a good

model.²⁸ Our results revealed that the hypothesized model was found to have good fit to the data as all of the fit indices satisfied the above stated standards for goodness-of-fit (NFI = 0.948, TLI = 0.943, CFI = 0.961, SRMR = 0.029). In addition, the standardized regression path coefficients of two item parcels for each of the four latent domain factors were salient (0.99 and 0.98 for the mobility latent factor, 0.95 and 0.97 for the basic activities of daily living latent factor, 0.91 and 0.92 for the arm function latent factor, and 0.94 and 0.95 for the impairment latent factor). The standardized regression path coefficients of the first-order mobility, basic activities of daily living, arm function, and impairment factors for the second-order factor were 0.90 and 0.98, 0.91, and 0.83, respectively. Hence, the findings lent support to the results of the exploratory factor analysis described in stage II and indicated that the four domains comprising the MDFRS share a common characteristic in measuring functional disability.

Table 5 Reliability analyses of the 33-item MDFRS (stage III)

MDFRS	Internal consistency (<i>n</i> = 121)	Test-retest (<i>n</i> = 51)	Inter-rater (<i>n</i> = 51)
Total scale	0.97	0.99	0.99
Mobility domain	0.96	0.99	0.99
BADL domain	0.94	0.99	0.98
Arm function domain	0.85	0.99	0.98
Impairment domain	0.84	0.99	0.99

MDFRS, Muscular Dystrophy Functional Rating Scale; BADL, basic activities of daily living.

Responsiveness of the measures

Complete baseline and follow-up data were available for 43 of 51 (84%) patients participating in validation studies of stage III. Average length of follow-up was 7.48 months (*SD* = 0.96). Of these, two subgroups of patients were further divided on grounds of the rate of disease progression: fast disease progression (31 Duchenne's patients) versus slow disease progression (12 patients with Becker's, limb-girdle, and facioscapulohumeral muscular dystrophies). Means for age and disease duration at baseline were 14.58 years (*SD* = 4.5) and 10.42 years (*SD* = 4.4) in the Duchenne's patients. The mean age of the patients with slow disease progression was 39.25 years (*SD* = 11.2) and the mean disease duration was 21.58 years (*SD* = 9.7). Table 6 presents a summary of the mean change scores and SRMs for the selected instruments. As can be seen, the fact that change scores were negative for the MDFRS and positive for the Brooke and Vignos Scales implied more

disability on follow-up than baseline, which was coincident with the progressive nature of the disease. In regard to responsiveness, the total score SRMs of the MDFRS for all patients and two subgroups were more sensitive than those of the Brooke and Vignos Scales. In patients with fast disease progression, the SRMs were moderate to large (0.51–0.97) for the mobility, impairment and arm function domains, whereas a small SRM (0.37) was observed for the basic activities of daily living domain. For those whose disease progressed slowly, moderate to large SRMs (0.46–0.84) were found for the mobility, basic activities of daily living and arm function domains, but a small SRM was obtained for the impairment domain (0.39).

Discussion

The MDFRS was designed to fit the current gap for tools that incorporate both impairment and disability in assessing patients with muscular dystrophy. The final version contains 33 items organized into four discrete domains in reference to mobility, basic activities of daily living, arm function and impairment. These are the key elements of rehabilitation programme for patients with muscular dystrophy, whether it is provided in hospital, in a nursing home or in the patient's own home.

Our findings demonstrated that the MDFRS appears to be a reliable and valid disease-specific rating scale that is suitable for dystrophic patients

Table 6 Responsiveness of the MDFRS, Brooke Scale and Vignos Scale, using standardized response mean

Test	Total sample (<i>n</i> = 43)		DMD (<i>n</i> = 31)		BMD, FSHMD, LGMD (<i>n</i> = 12)	
	MCS	SRM	MCS	SRM	MCS	SRM
MDFRS total score	−6.95	−1.02	−7.23	−1.02	−6.25	−1.00
Mobility domain	−1.72	−0.50	−1.97	−0.51	−1.08	−0.46
BADL domain	−1.00	−0.48	−0.81	−0.37	−1.5	−0.84
Arm function domain	−2.32	−0.90	−2.06	−0.97	−2.67	−0.82
Impairment domain	−2.00	−0.59	−2.39	−0.66	−1.00	−0.39
Brooke Scale	0.17	0.15	0.16	0.14	0.18	0.24
Vignos Scale	0.88	0.47	0.77	0.42	1.18	0.58

MDFRS, Muscular Dystrophy Functional Rating Scale; DMD, Duchenne muscular dystrophy; BMD, Becker muscular dystrophy; FSHMD, facioscapulohumeral muscular dystrophy; LGMD, limb-girdle muscular dystrophies; MCS, mean change scores; SRM, standardized response means; BADL, basic activities of daily living.

Clinical messages

- The Muscular Dystrophy Functional Rating Scale (MDFRS) is a sufficiently reliable and valid measure for assessing functional limitations in patients with muscular dystrophy.
- The MDFRS combines both impairment and disability in a single measure that reflects the patient's status in the following areas: mobility, basic activities of daily living, arm function and impairment.

aged 6 years and above, and is simple to administer and score. Reliability analyses indicated high levels of internal consistency, and excellent test–retest and inter-rater reliabilities across all domains of the instrument. With regard to convergent validity, the MDFRS demonstrated moderate to high correlations with a range of established functional rating scales and impairment parameters in relation to muscular strength, contracture severity and pulmonary function. Aside from these, consistent evidence in favour of a four-domain hierarchical model of the MDFRS was afforded by both exploratory and confirmatory factor analyses.

Importantly, the distribution of baseline MDFRS sum scores demonstrates minimal floor and ceiling effects, denoting that the potential for the MDFRS to capture change in connection with natural history in this sample appears good. Moreover, the MDFRS was shown to be the most responsive to changes in functional status over time in dystrophic patients who deteriorate at different rates, as compared to the small effect sizes for the Brooke Scale and moderate effect sizes for the Vignos Scale. On inspecting effect sizes for the arm function domain of the MDFRS, it was found that effect sizes for the arm domain were much larger than for the corresponding Brooke Scale in the whole sample and each subgroup. On the other hand, effect sizes for the MDFRS mobility domain in the entire sample and Duchenne's subgroup were higher than for the corresponding Vignos Scale, whereas effect size for the mobility domain in patients with slow disease progression was lower than for the Vignos Scale. These findings were partly coincident with previous research in that several of the Brooke and Vignos categories did

not describe significant numbers of dystrophic patients. For example, studies found that patients with facioscapulohumeral muscular dystrophies fell mainly between grade 1 and 3 of the Brooke Scale.^{16,29} As for the Vignos Scale, Lue *et al.*³⁰ disclosed that Duchenne's patients were clustered between grades 1 and 4, and again in grade 9, but none of them fell between grades 5 and 8. In view of a relatively small number of patients with slow disease progression in the current report, further studies on a larger sample are needed to reexamine the effect size of the MDFRS in patients with slow disease progression. On the whole, our findings not only lent support for the use of the MDFRS for monitoring progressive functional decline associated with dystrophic patients, but demonstrated that the range of measurement (four response categories) of the MDFRS was highly matched to the range of disability in the dystrophic sample.

Several limitations of our study are worth noting. First, our sample was confined to patients diagnosed as Duchenne's, Becker's, limb-girdle and facioscapulohumeral muscular dystrophies. Although our patient sample covered a wide range of functional limitations for both upper and lower limbs, other types of muscular dystrophy, such as myotonic dystrophy, may present with different clinical profiles that were not taken into consideration during the development process of the MDFRS.³¹ Future work will include replicating the validity and reliability estimates with this patient population. Second, given the vulnerability of dystrophic patients, there is a restriction on obtaining a larger sample in any one subgroup in the present study. However, while the data may not be sufficient to generalize the results, they can be used as a starting point for future studies. Third, the responsiveness of the MDFRS to treatment-induced changes has not been established yet, and that awaits further investigation. Fourth, our initial psychometric analyses did not include an approach for evaluating discriminant validity of the MDFRS and thus future research regarding differentiation between healthy and dystrophic subjects, and among different types of muscular dystrophy could be explored. Furthermore, instead of use of a total score, a four-domain MDFRS profile could be established separately for patients with fast or slow disease progression so that interpretation of the patient's functional status

could be more clinically meaningful. Last, Rasch analysis was not employed to investigate some psychometric aspects of the MDFRS during stage II item reduction and scale formation on account of the small patient sample ($n=85$). Future research should be geared toward investigating whether MDFRS forms a hierarchy, whether the weights are appropriate, and whether the development of a short form of the MDFRS is psychometrically feasible.

In conclusion, the MDFRS is a clinically useful and scientifically sound tool that comprised four critical domains in relation to functional outcome in dystrophic patients, from which the patient's particular strengths and weaknesses could be clearly identified. The MDFRS appears to be sensitive to changes in functional status of dystrophic patients that are of considerable concern to both patients and health care professionals.

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Appendix 1 – Muscular Dystrophy Functional Rating Scale

Mobility domain

M1	Stair climbing	4	No problem with normal speed
M2	Outdoor mobility	3	Slowly without assistance (other person)
M3	Indoor mobility	2	With assistance (other person)
M4	Transfers from bed to chair	1	Unable (complete dependent)
M5	Wheelchair manipulation		
M6	Standing from sitting		
M7	Sitting from lying		
M8	Rolling		
M9	Changing body position in bed		

BADL domain

B1	Feeding	4	No problem with normal speed
B2	Combing hair	3	Slowly without assistance (other person)
B3	Brushing teeth	2	With assistance (other person)
B4	Dressing upper/lower parts of body	1	Unable (complete dependent)
B5	Toileting		
B6	Bathing		

Arm function domain

A1	Managing objects over head	4	No problem for over head activities
		3	Assisted with other hand for over head activities
		2	With assistance (other person)
		1	Unable elevate arm (complete dependent)
A2	Carrying objects	4	No problem with heavy objects

		3	Could be done with light objects but not heavy objects
		2	With assistance (other person)
		1	Unable (complete dependent)
A3	Cleaning table	4	No problem with normal speed
A4	Writing	3	Slowly without assistance (other person)
A5	Turning books	2	With assistance (other person)
A6	Picking up small objects	1	Unable (complete dependent)
A7	Manipulating small objects		
Impairment domain			
I1	Severity of upper limb joint contractures	4	No limitation
		3	Mild limitation (range of limitation less than 1/3 of normal range)
I2	Severity of lower limb joint contractures	2	Moderate limitation (range of limitation between 1/3 to 2/3 of normal range)
		1	Severe limitation (range of limitation greater than 2/3 of normal range)
I3	Number of contracted joints in the upper limbs	4	None
		3	1 to 2
		2	3 to 4
		1	5 or more
I4	Number of contracted joints in the lower limbs		
I5	Severity of neck contracture	4	No limitation
		3	Mild limitation (range of limitation less than 1/3 of normal range)
		2	Moderate limitation (range of limitation between 1/3 to 2/3 of normal range)
		1	Severe limitation (range of limitation greater than 2/3 of normal range)
I6	Strength of the neck	4	No problem to elevate head in lying
		3	Difficulty (slowly) to elevate head in lying
		2	Unable elevate head in lying but proper head control during sitting
		1	Poor head control during sitting
I7	Strength of the trunk	4	No problem to reach objects in sitting
		3	Difficulty (need one hand support) to reach objects in sitting
		2	Upright sitting by oneself with back support
		1	Unable upright sitting
I8	Scoliosis	4	None
		3	Mild (without rib hump)
		2	Moderate (with rib hump)
		1	Severe (with rib hump and centre of gravity not in midline position)

I9	Orthopnea	4	No respiration problem during sleep
		3	Sometime headache after sleep
		2	Poor sleep quality due to respiration
		1	Unable to sleep due to respiration
I10	Sputum clearance	4	No problem
		3	Sometime with difficulty
		2	Always with difficulty
		1	Need percussion (or even suction)
I11	Ventilator assisted	4	Not needed
		3	During the night
		2	During the night and day (< 24 hours)
		1	All day long

Test directions: The items in the mobility, basic activities of daily living, and arm function domains need to be scored on the basis of the patient's actual performance. Therefore, the examiner asks the patient to perform on each test item, and assigns a grade accordingly. As for the impairment domain, a standard goniometer is used to measure patient's range of motion as indicated for some items. Examiner interviews the patient or caregiver on the last three items regarding orthopnoea, sputum clearance and use of ventilator.