

Validity of the EK scale: a functional assessment of non-ambulatory individuals with Duchenne muscular dystrophy or spinal muscular atrophy

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ABSTRACT Background and Purpose. *The EK scale comprises ten categories (EK 1–10), each contributing to an overall picture of function in the non-ambulatory stage of Duchenne muscular dystrophy (DMD). The purpose of the present study was to investigate content and construct validity of the EK scale as a tool to discriminate between levels of functional ability in individuals with DMD or spinal muscular atrophy (SMA) who were non-ambulatory. Method.* Data from a sample of 56 subjects with DMD and 38 with SMA, who were non-ambulatory, were obtained from four separate studies. The relationship of functional ability by use of the EK scale and (1) muscle strength, (2) contractures, (3) forced vital capacity and (4) years of wheelchair dependency were assessed. All items of the EK scale were used except the one representing severe hypoventilation. **Results.** Regression analyses showed that the EK sum was the most significant explanatory variable ($p < 0.05$) of all variables measured to explain muscle strength in both DMD and SMA subjects. The individual categories of EK (1–10) all contributed as significant explanatory variables ($p < 0.05$) to the other variables measured. **Conclusions.** The categories and items of the EK scale were relevant and valid as means of discriminating between levels of functional performance in the population studied which was evidence of content and construct validity.

Key words: Duchenne muscular dystrophy, EK scale, functional assessment, spinal muscular atrophy, validity

INTRODUCTION

The overall aim of management programmes for severely disabled people is to

make it possible for individuals to reach and sustain an optimum level of independence and function (United Nations, 1994). The programme for disabled people with

neuromuscular diseases therefore usually includes strategies for monitoring and preserving functional performance throughout the whole course of the disease, and for the prevention of secondary impairments that could affect function.

To achieve an optimal management programme a co-ordinated, multidisciplinary approach is needed (Fowler, 1982; Vignos, 1983; Siegel, 1989). A prerequisite for successful co-operation between specialists is a common language for clinical decision-making (Jette, 1985; ICIDH-2, 1999). A functional assessment scale may serve as a common frame of reference for planning and evaluating intervention by the specialists involved (Grimby and Fugl-Meyer, 1988).

The EK scale, *Egen Klassifikation* (translation from Danish: 'our own classification'), was developed to meet this need in the later stage of Duchenne muscular dystrophy (DMD) when unassisted ambulation is no longer possible. DMD and spinal muscular atrophy (SMA) are well-documented, genetically determined neuromuscular diseases, both characterized by global skeletal muscle weakness primarily affecting the proximal and trunk muscles, and resulting in wheelchair use throughout a major part of the lifespan (Brooke, 1986; Brooke et al., 1989, McDonald et al., 1995). The present study investigated the validity of EK with respect to non-ambulatory individuals with either of these diseases.

In the present study, functional ability was defined as an individual's ability to interact with his environment in a way that permits him to achieve competence in tasks of daily living. As weakness increases so does the interdependence of physical components and the choice of ways to perform tasks become more limited. Small changes in any component may have a disproportionate effect on function. It is therefore

important to have a measure which provides an overall assessment of functional abilities in these diseases.

Existing functional scales or classifications specifically used for subjects with DMD or SMA are the Vignos lower extremity classification (Vignos et al., 1963), the Brooke upper extremity scale (Brooke et al., 1981; Hiller and Wade, 1992) and different kinds of timed tasks (Brooke et al., 1981; Hiller and Wade, 1992; Wagner et al., 1993). Function, in the sense of activities of daily living, is not measured by these tests. The existing generic measures of function which have been developed to assess daily activities in a wide range of other disorders do not focus on the specific losses of function which are characteristic for DMD or SMA in the later stage of disease. Since these generic measures do not fulfil this most important criteria for choosing a measure they were not considered to be appropriate in these conditions (Wade, 1992).

The EK is an ordinal scale where zero represents the highest level of independent function and 30 the lowest level. The assessment consists of 10 categories, each concerning a major domain, and each category has four items: zero to three. The sum of the categories (0–30 points) is called the 'EK sum' (appendices I and II).

The purpose of the present study was to investigate the content and construct validity of the EK scale as an instrument with which to discriminate between non-ambulatory individuals, with DMD or SMA, according to their functional abilities. A prerequisite for validity is reliability (Sim and Arnell, 1993). The EK scale has been shown to have high inter- and intra-rater reliability (Intra-class Correlation Coefficient (ICC) = 0.98) when used for scoring video-recorded individuals with DMD.

The construct of the EK scale is based on the assumption that functional ability in the groups studied is a result of the interaction of physical components:

- Muscle strength.
- Range of motion.
- Respiratory competence (Streiner and Norman, 1998).

Other factors of influence may be:

- Years of wheelchair dependence.
- Age, since there is a relationship between these factors and muscle strength in DMD (McDonald et al., 1995).

To assess the construct validity and the discriminative power of the scale, the following question was addressed:

- To what extent are the EK sum and the individual category scores able to explain the variation of the variables contributing to function?

Determination of content validity is essentially a subjective process, since there are no statistical indices that can assess content validity (Portney and Watkins, 1993). However, it should be shown that the scale covers the domain of interest, and that the categories and items of the EK reflect the natural history of these groups. Therefore, to assess content validity the present study addressed the following questions:

- Are the categories and items of the EK scale identifiable for the evaluator and applicable to subjects in all stages of the disease?
- Does the sequence in which the items are described, from zero to three, reflect the natural history in terms of loss of activities in the sequence they appear?

METHOD

Subjects

Ninety-four subjects were included in this study (Table 1):

- DMD ($n = 56$) (all male).
- SMA II ($n = 33$) (14 female and 19 male).
- SMA III ($n = 5$) (all male).

All the subjects had participated in one or more of four different studies with specified criteria for inclusion and exclusion (Appendix III). Thus all the measurements on all the variables required for this study were not available. For subjects participating in more than one study, the dataset from the study providing data on the most variables common to this study, that is EK, muscle strength, contractures, forced vital capacity or years of wheelchair dependence, was chosen for analysis, so that no subject was used twice for the present study.

General criteria for inclusion in the study were that:

- Subjects met the established diagnostic criteria for DMD, SMA II or SMA III with respect to family history, clinical course, muscle biopsy and serum creatinine kinase activity (Emery, 1997).
- Subjects had lost independent ambulation without orthoses at the date of examination and used a wheelchair for mobility.

All the study subjects were registered with the Institute for Neuromuscular Diseases of Muskelsvindfonden (the Danish Muscular Dystrophy Association) in Denmark and all the studies had been approved by the Ethical Committee of the county of Århus, Denmark.

TABLE 1: Presentation of the subjects in the study (median and range of variables)

<i>Diagnosis</i>	<i>Age at loss ambulation (years)</i>	<i>Age at examination (years)</i>	<i>In w/chair (years)</i>	<i>EK sum (points)</i>	<i>MRC% (%)</i>	<i>SOC (degrees)</i>	<i>FVC% (%)</i>
DMD							
median	10	14	4	16	40	215	37
range	6-12	9-29	0.2-56	1-28	10-80	20-880	10-103
n.obs.	56	56	56	56	39	33	36
SMA							
median	11	20	20	15	50	115	32
range	3-37	17-71	7-60	2-28	15-70	0-820	5-100
n.obs.	5*	38	38	38	33	17	28

*Five of the individuals with SMA had the diagnosis SMA III and have been walking.
DMD = Subjects with Duchenne muscular dystrophy; SMA = Subjects with spinal muscular atrophy; n.obs. = number of observations; EK sum = functional ability; MRC% = muscle strength in per cent of maximal obtainable; SOC = sum of contractures; FVC% = forced vital capacity in per cent of normal.

Evaluators

Four state-licensed physiotherapists examined the subjects. They had all had more than 10 years of experience in examining people with DMD and SMA. Inter-rater consistency between evaluators was not assessed.

The EK scale

The evaluator questioned and assessed the subjects (Appendix II). The item scored in each of the 10 categories of the scale was recorded and the EK sum was calculated. Subjects were asked about what they actually did in daily life. They were assessed in a standard hand- or electrically operated wheelchair without special equipment.

Muscle strength expressed as a percentage of the maximal possible score

Voluntary maximal muscle strength was graded by use of a manual muscle test (0–5) according to the Medical Research Council (MRC) scale (Medical Research Council, 1943). The total score of muscle strength of four muscle groups of the upper extremities, flexion and extension of the left and right elbows, were selected for measurement and expressed as the following (Scott et al., 1982):

$$\text{MRC\%} = \frac{\text{Sum of grade scores of muscles} \times 100}{5 \times \text{number of muscles tested}}$$

Sum of contractures

The range of motion (ROM) was measured by use of a goniometer and the method standardized by the American Academy of Orthopedic Surgeons (1965). The measurement of ROM was recorded to the nearest 5° according to the protocol of Brooke et al.

(1981). The degree of contracture was recorded as the difference between the measured range of motion and the normal range as defined by the American Academy of Orthopedic Surgeons (1965). Hypermobility was recorded as '0 contracture'. A sum of contractures (SOC) was calculated for each subject. Flexion of the shoulders, extension of the elbows, supination of the forearms, bilateral wrist extension, radial and ulnar deviation of the wrists were selected for measurement.

Forced vital capacity as a percentage of normal

Forced vital capacity (FVC), that is the volume of air which can be exhaled using maximal force in one breath after a maximal inhalation, was measured by use of a calibrated spirometer (COMPACT; Vitalograph Ltd, Buckingham, UK). The standardized position was with the subject sitting in his wheelchair wearing a nose clip. Special attention was given to keeping the connection between the lips and the mouthpiece tight. All subjects were familiar with the test. Three measurements were performed and the best of these was recorded. The FVC was compared to the individually calculated reference values and expressed as a percentage of normal (FVC%) (Quanjer, 1983).

Statistics

Descriptive statistics were used to determine the frequency of scorings of the items and categories of the EK scale in relation to age and diagnosis. In the absence of a criterion standard measure, which is a measure providing the underlying truth about the domain of interest (Guyatt and Juniper, 1998), the ability of the EK sum and the individual categories of EK 1–10 were

investigated to predict the response variables:

- Years of wheelchair dependency.
- MRC%.
- Natural logarithm to SOC (lnSOC).
- FVC%.

The natural logarithm to SOC was used to achieve linearity. Each measure was analysed using a backward, stepwise regression procedure (Altman, 1991) (Table 2). The initial model included all explanatory variables and the final model included only variables which were significant at the 10% level. If EK sum or EK 1–10, respectively, were among the remaining significant variables, this was taken as an indication that those variables had a clinically significant power to explain (predict) the measure in question.

The explanatory power (R^2), which is the proportion of variation explained by the explanatory variables, was calculated for the initial and final models. All analyses were performed using SAS software (Littel et al., 1996).

RESULTS

EK scores obtained in the 10 categories and four items in each diagnostic group are shown in Table 3. Subjects with DMD achieved scores in all items of EK except the item that reflects the most severe symptoms of hypoventilation: category 10, item 3. The range of scores of EK sum of all observations was 1–28.

Subjects with SMA achieved scores in all items except in categories 1 and 2, item 1 and category 10, items 2 and 3. The range of scores of EK sums obtained from all observations was 2–28.

The distribution of the sum of scores (the item number \times frequency of scores

summed for the four items) in percentage of maximal loss of function (item 3 \times n) within each category of the two diagnostic groups (Table 3) showed a similar pattern. However, four of the categories (3, 4, 5 and 6) differed more than 10% in loss of function. The SMA group was more limited in standing activity (category 3) and in sitting balance (category 4) but less limited in activities with the arms (categories 5 and 6) compared to the DMD group. The mean percentage of loss of function in all categories was 49.6% in subjects with DMD and 49.8% in subjects with SMA (Table 3).

Distribution of the sum of scores in different age groups (Figure 1) showed a relationship of greater functional loss with increasing age, that is, older subjects had the higher percent score, for subjects with DMD. Mean percentage of loss of function in the three age groups (<10, 11–15 and >15 years) was 28%, 42% and 72%, respectively. There was no such relationship in the subjects with SMA. Mean percentage of loss of function in these three age groups (<10, 11–20 and >20 years) was 53%, 45% and 52%, respectively.

The relationship between MRC%, lnSOC, FVC% and years of wheelchair use as a function of EK sum or EK 1–10 in both diagnostic groups was approximately linear. One subject with DMD was excluded from the analyses of regression, as he was an outlier with extreme contractures.

The first set of multiple linear regression analyses (Table 2) in subjects with DMD showed that EK sum, with age and lnSOC, was significant in explaining years in wheelchair. The explanatory power did not change from the initial model to the final model ($R^2 = 86\%$). EK sum was the only significant variable to explain muscle strength. When using EK sum as the only explanatory variable, the explanatory power (R^2) was 69%, whereas all the variables

Table 2: Results from two sets of backward stepwise regression analyses (I and II). The table shows *p*-values of significant variables (*p*≤0.1), and R² for the initial and final models

Analysis I (Study B) Explanatory variables	Response variables							
	Years in w/chair		MRC%		InSOC		FVC%	
	DMD (n = 19)	SMA too few	DMD (n = 19)	SMA (n = 7)	DMD (n = 19)	SMA (n = 7)	DMD (n = 19)	SMA (n = 7)
Years in w/chair								
MRC%					0.0005			
InSOC	0.048						0.004	
FVC%								
Age	0.035							
EK sum	<0.0001		<0.0001	0.012		0.08	57%	0.03
R ² initial model	86%		77%	89%		61%	52%	85%
R ² final model	86%		69%	75%		49%	39%	64%
NB: Grey areas indicate that the analysis was not performed. In SMA: Years in wheelchair were excluded from analyses since they were identical with age in most subjects.								
Analysis II (Studies A–D) Explanatory variables	Response variables							
	Years in w/chair		MRC%		InSOC		FVC%	
	DMD (n = 55)	SMA (n = 38)	DMD (n = 38)	SMA (n = 33)	DMD (n = 32)	SMA (n = 17)	DMD (n = 35)	SMA (n = 28)
EK1		0.0007						0.01
EK2		0.018						0.07
EK3		0.009						
EK4	0.018		0.1	0.02	0.007	0.03	0.0003	0.02
EK5	0.003		0.02	0.001	0.001			
EK6					0.052			
EK7		0.006	0.006			0.005	0.003	0.008
EK8								
EK9								0.04
EK10		0.03						
R ² initial model	75%	67%	83%	77%	74%	86%	72%	80%
R ² final model	69%	59%	81%	72%	72%	77%	65%	76%
The response variables are: years in wheelchair, muscle strength as percentage of three maximal possible score (MRC%), natural logarithm of the sum of contractures (In SOC) and forced vital capacity as a percentage of normal (FVC%). The first set of analyses are based on study B's data, where the explanatory variables are EK sum, age and the same variables mentioned above. The second set of analyses are based on data from all studies (A–D), where explanatory variables are the 10 categories of EK (EK1–10). The proportion of the variance in the response variable explained by the explanatory variables (R ²) is shown as percentages. R ² in the initial model includes all variables. R ² in the final model includes variables only. Number of individuals (n).								

TABLE 3: Distribution of EK scores obtained by 56 subjects with Duchenne muscular dystrophy and 38 subjects with spinal muscular atrophy over categories within items. The number within each table cell is the frequency scores.

<i>Duchenne muscular dystrophy</i>										
<i>Categories</i>										
<i>Item ↓</i>	<i>1</i>	<i>2</i>	<i>3</i>	<i>4</i>	<i>5</i>	<i>6</i>	<i>7</i>	<i>8</i>	<i>9</i>	<i>10</i>
0	4	1	4	18	16	11	8	24	36	45
1	8	2	7	11	8	15	4	18	15	9
2	19	27	16	16	14	18	23	11	4	2
3	25	26	29	11	18	12	21	3	1	0
Ssc	121	134	126	76	90	87	113	49	26	13
% max	72	79	75	45	54	52	67	29	15	8
<i>Spinal muscular atrophy</i>										
<i>Categories</i>										
<i>Item ↓</i>	<i>1</i>	<i>2</i>	<i>3</i>	<i>4</i>	<i>5</i>	<i>6</i>	<i>7</i>	<i>8</i>	<i>9</i>	<i>10</i>
0	4	2	1	7	7	9	5	12	28	32
1	0	0	3	10	18	15	2	20	7	6
2	20	13	7	12	8	10	13	4	2	0
3	14	23	27	9	5	4	18	2	1	0
Ssc	82	95	98	61	49	47	82	34	14	6
% max	72	83	86	54	43	41	72	30	12	5
Sum of score (Ssc) is the item number × frequency of scores, summed for the four items. Ssc as percentage of maximal loss of function ($3 \times n$) is presented for each category (% max).										

could explain 77% of the variation in MRC%. InSOC was better explained by years in wheelchair, and FVC% was better explained by muscle strength than by EK sum (NS).

In subjects with SMA, EK sum was the only significant variable in explaining MRC%, InSOC and FVC%. EK sum explained 75% and 64% of the variance of MRC% and FVC%. There were too few observations to analyse years in wheelchair.

In the second set of regression analyses (Table 2) each individual category of EK contributed as a significant explanatory variable ($p < 0.05$) to at least one of the response variables. Among DMD subjects, EK categories 4, 5, 6 and 8 were those that could best explain the variables ‘years in wheelchair’, ‘MRC%’, ‘InSOC’ and

‘FVC%’. Among SMA subjects all EK categories except category 5 could explain these same variables. Category 4 (balance in the wheelchair) was the category which most frequently (six times) became the significant explanatory variable in the analyses in both groups. The change of explanatory power from the initial model to the final model was less than 10 percentage points in all analyses. The explanatory power (R^2) in the final models ranged from 65% to 81% in the subjects with DMD and from 59% to 77% in the subjects with SMA.

DISCUSSION

To fulfil the purpose of the present study data were chosen from four different studies all using the EK scale, as well as variables

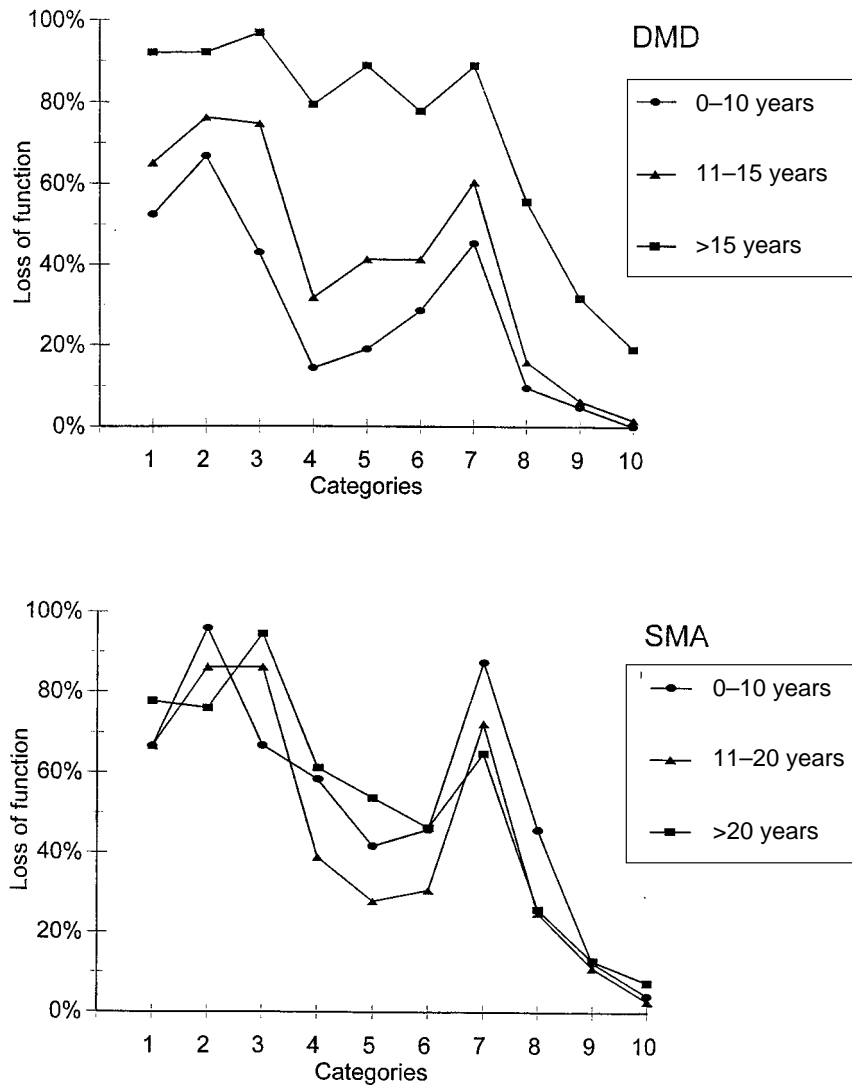


FIGURE 1: A profile of the sum of scores in percentage of maximal loss of function (y-axis) within each EK category (1–10) (x-axis) in age groups in subjects with DMD or SMA. The sum of scores is the item number \times frequency of scores summed for the four items. Maximal loss of function is item number 3 \times the maximal possible frequency of scores (3 \times n) in each age group. A line is joining the individual percentages calculated to visualize a profile. In DMD the number of subjects within each age group was 14 (0–10 years), 21 (11–15 years) and 21 (>15 years). In SMA the number of subjects within each age group was eight (0–10 years), 12 (11–20 years) and 18 (>20 years).

on muscle strength, contractures, forced vital capacity and years of wheelchair use.

The fact that nearly all items of the scale were used in scoring both diagnostic groups indicates that they were recognizable for the

clinical evaluators and representative for the populations studied. The items which were not scored were those indicative of severe hypoventilation with hypercapnia, as represented by category 10, items 2 and 3. Since

nearly all people in Denmark are offered mechanical ventilation before reaching this stage, these items may not be expected to be used very frequently. Items which were not scored in SMA subjects, the activity to transfer from a wheelchair with the use of an aid and to push a manual wheelchair, probably reflect the fact that most SMA subjects using a wheelchair are too weak to perform these activities due to profound weakness of the shoulder girdle muscles. However, to permit the stronger subjects with SMA III to be evaluated it is probably necessary to retain these items.

There were no observable 'floor' or 'ceiling' effects measured on EK sum since none of the wheelchair-dependent subjects scored 0 or 30. Distribution of scores was not clustered near the top or bottom of the scale (Table 3) which means that the actual distribution of functional ability of the subjects corresponded to the range of the EK scale.

The similarity of the results on scoring the categories in both diagnostic groups (Table 3) may be expected as a consequence of the dominance of weakness in the proximal and the trunk muscles because the individuals in both groups compensate for loss of muscle strength in similar ways when moving in a wheelchair. The differences in scoring categories 3, 4, 5 and 6 between DMD and SMA subjects is a reflection of the natural history of the diseases. In SMA, standing with support (category 3) was an activity which was never achieved or had been lost early, in contrast to subjects with DMD who might have walked until the age of 12 (Emery, 1997) and still preserved standing after loss of ambulation. Balance in the wheelchair (category 4) is often compromised from an early age in SMA due to weakness of the trunk muscles, early scoliosis and difficulty in balancing the head, whereas these problems are mostly present

in older subjects with DMD. Functional limitations of the upper extremities were less in SMA subjects (categories 5 and 6) compared to those in the DMD group who tend to lose the ability to move the arms against gravity within a few years of loss of ambulation (Figure 1), whereas subjects with SMA change very little over time.

Although the distribution of items scored over categories in the two diagnostic groups were similar in pattern, there were age-specific differences in DMD subjects. The successive loss of function with age from the lower to the higher percentage of loss of function (Figure 1) indicates that items 0–3 fit the typical progression of DMD and the EK scale is useful to discriminate among subjects with different levels of function. In SMA subjects the distribution of scores was independent of age (Figure 1) which fits with the natural history of a non-progressive disease (Brooke, 1986). Further studies are needed to show how well the EK scale reflects the progression of the disease in the single subject with DMD or SMA over time.

In the absence of a criterion standard variables were identified which were expected to influence and contribute to the overall functional ability as defined by the EK scale. Since the development of the EK scale was based on clinical observations on the sequence of loss of function in DMD a deductive method was chosen to test the effectiveness of the EK scale to explain the other variables.

The functional ability of subjects with DMD or SMA was expected primarily to be affected by their loss of muscle strength. The results of the multiple linear regression analyses in both groups showed that EK sum was the only highly significant explanatory variable of MRC%. In both groups the explanatory power (R^2) in the final model was high compared to the ini-

tial model (DMD 69% versus 77%; SMA 75% versus 89%). The explanatory power in the initial model and the final model were directly comparable because the regression analyses were based on the same set of data. Although the four muscle groups measured do not reflect the total muscle strength of the wheelchair users, they are the muscle groups that are essential for functional activity in the wheelchair.

In subjects with DMD or SMA, FVC% may be regarded as a measure which reflects respiratory and abdominal muscle strength since subjects have no primary lung diseases. The decreased FVC does not normally give rise to overt clinical problems, such as difficulty in clearing secretions, until the reduction is more than 50% of normal values (European Consortium on Chronic Respiratory Insufficiency, 1996) which means that it would not be registered as a clinical symptom in EK categories 8, 9 and 10 until late in the course of the disease in subjects with DMD (Figure 1) but, dependent on the extent of weakness, at any time in SMA. In the first set of multiple linear regression analyses in the DMD group, FVC% not surprisingly was better explained by muscle strength than by EK sum. In the SMA group, however, EK sum was significant in explaining FVC% and the explanatory power was fairly high (64%). The analysis showed that lnSOC was better explained by years in wheelchair in DMD subjects than by EK sum (NS) which fits with the understanding that length of constrained mobility is an important factor in development of contractures in DMD. In the SMA group, however, the individuals had usually been restricted to a wheelchair at a younger age than the people with DMD and hence had developed contractures earlier, and here EK sum was the only significant variable.

In the second set of multiple linear regression analyses with EK categories 1–10 as the only explanatory variables, all categories were significant at different levels in explaining the variation of MRC%, lnSOC, FVC% and years of wheelchair dependence in both DMD and SMA subjects. An explanatory power above 50% in both groups (Table 2) was found in the final model. All categories appear to contribute to an overall picture of function of the individual.

The fact that EK sum could not explain FVC% and lnSOC in DMD subjects might be explained by the dataset used. Study B was the only study which had all the measures needed for the first set of regression analyses. This study had the youngest population (aged 10–15 years) (Appendix III) which means that pulmonary symptoms and contractures in the upper extremities, might have been less influential in subjects with DMD, whereas subjects with SMA had never walked due to weakness and had had respiratory problems and contractures from an early age.

CONCLUSIONS

The study shows that EK sum can discriminate between subjects with different levels of functional ability and EK categories reflect the natural history of DMD and SMA in the non-ambulatory stage of disease thus fulfilling requirements of both construct and content validity. The results showed that:

- All categories of the EK scale contributed as significant explanatory variables to explain variations in MRC%, lnSOC, FVC% and years of wheelchair dependency.
- EK sum was the only highly significant variable in both diagnostic groups to

explain variation in muscle strength with an explanatory power of 69% in subjects with DMD and 75% in subjects with SMA in competition with the other variables, InSOC, FVC%, years of wheelchair dependence and age.

- All categories and items of the scale were representative for subjects with DMD or SMA since they obtained scores in all items but one.
- The scale reflected decreasing functional ability with age in subjects with DMD and a functional level that is independent of age in subjects with SMA, in concordance with the natural history of the two diseases.

Furthermore, in subjects with DMD, EK sum was found to explain variation in years of wheelchair dependence but was not found to explain variance in InSOC and FVC%. In subjects with SMA, EK sum was found to explain InSOC and FVC%, whereas years of wheelchair dependence were not analysed due to too few subjects.

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APPENDIX I

The EK scale

Each of ten categories consists of four items (0–3) and the EK sum is the sum of scores over categories

1 *Ability to use wheelchair*

- 0 Able to use a manual wheelchair on flat ground, 10 metres in less than one minute.
- 1 Able to use a manual wheelchair on flat ground, 10 metres in more than one minute.
- 2 Unable to use manual wheelchair, requires electric wheelchair.
- 3 Uses electric wheelchair, but occasionally has difficulty in steering.

2 *Ability to transfer from wheelchair*

- 0 Able to transfer from wheelchair without help
- 1 Able to transfer independently from wheelchair with use of aid.
- 2 Needs assistance to transfer with or without additional aids (lift, easy glide).
- 3 Needs to be lifted with support of head when transferring from wheelchair.

3 *Ability to stand*

- 0 Able to stand with knees supported, as when using braces.
- 1 Able to stand with knees and hips supported, as when using standing aids.
- 2 Able to stand with full body support.
- 3 Unable to be stood, marked contractures.

4 *Ability to balance in the wheelchair*

- 0 Able to push himself upright from complete forward flexion by pushing up with hands.
- 1 Able to move the upper part of the body more than 30° from the upright position in all directions, but cannot push himself upright from the total forward flexed position.
- 2 Able to move the upper part of the body less than 30° from one side to the other.

3 Unable to change position of the upper part of the body, cannot sit without total support of trunk and head.

5 *Ability to move the arms*

0 Able to push himself upright from complete forward flexion by pushing up with hands.

1 Unable to lift the arms above the head, but able to raise the forearms against gravity, that is, hand to mouth with or without elbow support.

2 Unable to lift the forearms against gravity, but able to use the hands against gravity when the forearm is supported.

3 Unable to move the hands against gravity but able to use the fingers.

6 *Ability to use the hands and arms for eating*

0 Able to cut meat into pieces and eat with spoon and fork. Can lift a filled cup (approximately 250 ml) to the mouth without support at elbow.

1 Eats and drinks with support at elbow.

2 Eats and drinks with elbow support and with reinforcement of the opposite hand ± feeding aids.

3 Has to be fed.

7 *Ability to turn in bed*

0 Able to turn himself in bed with bed-clothes.

1 Able to turn himself on a couch, but not in bed.

2 Unable to turn himself in bed. Has to be turned three times or less during the night.

3 Unable to turn himself in bed. Has to be turned four times or more during the night.

8 *Ability to cough*

0 Able to cough effectively.

1 Has difficulty to cough and sometimes needs manual reinforcement. Able to clear the throat.

2 Always needs help for coughing. Only possible to cough in certain positions.

3 Unable to cough. Needs suction or positive pressure breathing techniques in order to keep the airways clear.

9 *Ability to speak*

0 Powerful speech. Able to sing and speak loudly.

1 Speaks normally, but cannot raise his voice.

2 Speaks with quiet voice and needs a breath after three to five words.

3 Speech is difficult to understand except to close relatives.

10 *Physical well-being*

0 No complaints, feels good.

1 Easily tires. Has difficulty resting in a chair or in bed.

2 Has loss of weight, loss of appetite. Scared of falling asleep at night, sleeps badly.

3 Experience additional symptoms such as: change of mood, stomach ache, palpitations, perspiring.

APPENDIX II

Administration of the EK scale

The administration of the EK scale consists of a question to the individual and his helper on how the task is performed in daily life. The items are scored according the explanation and observation of the performance.

- 1 How do you drive your wheelchair (items 0–3)? Please, show me how you do it (items 0–2).
- 2 How do you transfer from the wheelchair (items 0–3)? Please, show me how you do it (items 0–1).
- 3 Do you stand up (items 0–3)? How do you stand? Please, show or explain to me how you do it (items 0–2).
- 4 Do you change position in the wheelchair (items 0–3)? Please show me how much you can lean forwards and to the sides and get back to the upright position (items 0–2).
- 5 Can you move your fingers, hands and arms against gravity (items 0–3)? Please show me how you do it (items 0–3).
- 6 How do you feed yourself (0–3)? Please, show me or explain to me how you do it (items 0–2).
- 7 How do you turn in bed during the night (items 0–3)? Please, explain to me how you do it (items 0, 1) and how often (items 2, 3).
- 8 What do you do to produce the most effective cough (items 0–3)? Please, show and explain to me how you do it (items 0–3).
- 9 Do you speak loudly and clearly enough to make people understand you at the other end of the classroom (items 0–3)?
- 10 How is your physical well-being (items 0–3).

Category 10 focuses on symptoms of respiratory insufficiency and the descriptions of the items from 0 to 3 are used for questioning and scoring for instance: do you need to rest during the day? Do you sleep well during night? How is your appetite?

APPENDIX III

Additional criteria for inclusion and exclusion of subjects in the original studies

Studies A and D, concerning respiratory function

Inclusion criteria:

- All registered subjects with DMD or SMA above 10 years of age, living in the western part of Denmark

Exclusion criteria:

- Subjects using any form of mechanical ventilation.
- Subjects who had a tracheostomy.

Study A: 43 subjects were invited to participate. Four refused, 14 did not reply and 25 accepted. Of those accepting 14 were people with DMD and 11 were people with SMA II. Two of those with DMD were excluded since they did not fulfil the diagnostic criteria (Emery, 1997). Twelve subjects with DMD and 11 subjects with SMA were included.

Study D: The study was planned as a long-term follow-up study. Subjects were examined once a year. All subjects fulfilling the above criteria until 15 April 1996 were invited and accepted. Twelve subjects with DMD and 17 subjects with SMA were included.

Study B, concerning contractures

Inclusion criteria:

- All registered subjects with DMD and SMA between the age of 10 and 15 years from all over Denmark.

Forty-two children were invited to participate in the study. Fourteen refused. Of the remaining 28 children, 20 had diagnoses of DMD and eight had SMA. One of the children with SMA was excluded since he did not fulfil the diagnostic criteria (Emery, 1997). Twenty subjects with DMD and seven subjects with SMA were included.

Study C, concerning function of the upper extremities

Inclusion criteria:

- All registered subjects with DMD and SMA between the age of five and 30 years living in the eastern part of Denmark.

Sixty-one subjects were registered as fulfilling the criteria. Forty-five subjects agreed to participate, 29 subjects had DMD and 16 had SMA II. Eight of the subjects with DMD did not fulfil the diagnostic criteria (Emery, 1997), since they had lost independent ambulation after the age of 13 and were excluded from the study. Twenty-one subjects with DMD and 16 with SMA were included.

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