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The quality of life in boys with Duchenne muscular dystrophy

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Abstract

We conducted a study to evaluate the quality of life in boys with Duchenne muscular dystrophy aged 8–18 years, compared with that in matched healthy controls. A total of 85 boys with Duchenne muscular dystrophy aged 8–18 years and 136 age, sex and living place matched healthy controls were included in this study. Patients and one of their parents separately completed the 27-item Persian version of KIDSCREEN questionnaire (child and adolescent version and parent version). From the children's perspective, the quality of life in patients was found to be lower in two subclasses: "physical activities and health" ($p < 0.001$) and "friends" ($p = 0.005$). Parental estimation of their sick child's quality of life was significantly lower than children's own assessment in two subclasses: "physical activities and health" ($p < 0.001$) and "general mood and feelings" ($p < 0.001$). Our results indicate that boys with Duchenne muscular dystrophy have quite a satisfactory quality of life. A happier and more hopeful life can be promoted through increasing social support and improving the parental knowledge regarding their child's more positive life perspective.

Keywords: Quality of life; Duchenne muscular dystrophy; KIDSCREEN questionnaire

1. Introduction

Duchenne muscular dystrophy (DMD) is the most common hereditary progressive muscular disease in children, with a prevalence of 1 in every 4000 boys [1]. Despite advances in diagnosis and treatment measures, psychological and environmental aspects of patients have received less attention in daily practice [2].

Health-related quality of life has a multidimensional definition and explains the effect of the health status of individuals including physical, mental, and social domains on the quality of life [3]. Quality of life assessment has become an inseparable part of the evaluation of treatment trials and the US Food and Drug Administration recommends using quality of life assessment tools [4].

Health-related quality of life is used for the assessment of the health requirements of the society, evaluation of the social impact of diseases, identification of individuals at risk, execution of appropriate health policies proportionate to these issues, and allocation of resources in the health sector [5].

Health-related quality of life assessment is well-recognized in adults, but its application in children does not have a long history. Previous studies have shown that children can report their well-being with a high degree of certainty using age appropriate tools. However, if the child is not cooperative due to the lack of lingual and cognitive skills or the presence of a severe disease, the parent's participation in the assessment of their child's health-related quality of life is essential and vital [6,7]. In such cases, however, assessment may be inaccurate because parents answer the questions from their own perspective and viewpoint [8].

Previous studies on the effect of DMD on the quality of life in boys have reported different results. In one study, the quality of life in these patients has showed a decrease only in the physical subclass when compared to controls, whereas other

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studies have shown a decrease in all quality of life related subclasses when compared with age matched controls [9–13]. Very few studies have investigated the level of agreement between children's self-reports and parents' reports of the health-related quality of life in children with DMD. In two separate studies, Bray et al. and Davis et al. have shown that parental estimation of their child's quality of life in the subclass "general mood and feelings" was lower than children's own self-reports [2,13].

To our knowledge, no study has assessed the health-related quality of life in children and adolescents with DMD in Iran. Therefore, we aimed to evaluate the health-related quality of life in boys with DMD aged 8–18 years compared with matched healthy controls by using the standard 27-item KIDSCREEN questionnaires for child self-assessment and parent assessment.

2. Patients and methods

2.1. Patients

This study was performed on 85 boys with DMD aged 8–18 years who lived in Tehran and were monitored in the Pediatric Neurology Department of Children's Medical Center and Iran Muscular Dystrophy Association. The reason for considering this age group was that both patients and their parents were adapted to the disease.

Inclusion criteria were patient's age (8–18 years), residence in Tehran, clinical picture of disease, high levels of muscle enzymes, and DMD confirmed via biopsy or genetic testing. Exclusion criteria were age under 8 or above 18 years, residence outside Tehran, presence of mental disorders, and a lack of confirmatory biopsy or genetic testing. Control group comprises 136 age, sex, and living place matched healthy children. For convenience and reliability, both patients and their controls were selected from the same school. All children in the control group were volunteers. After receiving an explanation on the study objective and providing their informed consent, participants were enrolled into the study voluntarily and willingly.

2.2. Assessment tools

In the first questionnaire, we collected personal information, demographic data, data related to medical and family history, clinical picture, treatments and supportive procedures, and the results of diagnostic tests like muscle enzymes, biopsy, and genetic testing. Moreover, we used the 27-item KIDSCREEN questionnaire to assess the quality of life. The child and adolescent version (self-assessment) and the parent version of this questionnaire were standardized for the Iranian population aged 8–18 years old [14]. These questionnaires were completed by children and parents, respectively. In both groups, all the questionnaires were completed at home. In our country, mothers spend most of their time at home; thus, the parent questionnaire was completed by the mother unless she was unavailable during the study. The KIDSCREEN-27 is a valid and reliable questionnaire for assessing the quality of life in sick and healthy children and adolescents aged 8–18 years old [15]. This questionnaire has 5 major subclasses:

1 Physical activities and health (5 items)
2 General mood and feelings (7 items)
3 Family and free time (7 items)
4 Friends (4 items)
5 School and learning (4 items)

Each item assesses the frequency or intensity of a behavior or feeling through a 5-point Likert scale (1 = never, 2 = seldom, 3 = quite often, 4 = very often, 5 = always). The recall period was the week before answering the questionnaire. Scores were calculated separately for each subclass and were changed to the values of T with a mean (M) of 50 and standard deviation (SD) of 10. Higher scores indicated a better health-related quality of life [16].

2.3. Data collection

One part of the patients and their parents were visited in the outpatient neurology clinic and the rest were visited at their houses after an appointment was set by the Iranian Muscular Dystrophy Association via telephone calls. After obtaining an explanation of the study, subjects agreed to participate willingly and voluntarily by signing an informed consent. Afterward, they completed the parent and the child and adolescent versions of the KIDSCREEN-27 at home. In addition, parents were assured that if they decide not to participate, this decision would not affect the treatment and care process of their children. Demographic data, medical and family history, clinical status, diagnostic tests and supportive treatments of the patient were collected by using another questionnaire. The control group was composed of age and geographical location matched healthy boys selected from the same schools that were attended by the patients. Children in the control group and their parents (preferably mother) also completed the questionnaires.

2.4. Statistical analysis

All data gathered from participants were registered in a computerized database. Normality was checked by using the Kolmogorov–Smirnov test; if data were distributed normally, then analysis was performed using parametric tests. All continuous variables are expressed as mean ± (SD) and categorical variables as number (percentage). The independent sample *t*-test was used to compare HRQOL scores between groups (case and control). Linear regression was used to assess the effect of demographic variables on different subclasses of QOL. The paired t-test was used to compare the parent and child attitudes regarding different subclasses of QOL. Correlation between parent and child attitude regarding different subclasses of QOL was assessed using the Pearson correlation test. P-values less than 0.05 were considered statistically significant.

3. Results

3.1. Sample description

The patient group comprised 85 boys with DMD aged 8–18 years (mean age 12.6 ± 3.34 years) and the control group included 136 age-matched healthy children aged 8–18 years (mean age 12.1 ± 2.51 years).

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3.2. Comparative analysis of the health-related quality of life in healthy and affected children

The quality of life in healthy children and boys with DMD from the perspective of the children and adolescents and the perspective of their parents was assessed by using the KIDSCREEN-27. From the children's and adolescents' perspective, significantly lower scores were observed in the patient group in two following subclasses: "physical activity and health" ($p < 0.001$) and "friends" ($p = 0.005$), whereas the scores in other subclasses of the quality of life were similar between the two groups (Table 1).

From the parents' perspective, the quality of life in the patient group was significantly lower in the subclasses "physical activity and health" ($p < 0.001$), "general mood and feelings" ($p < 0.001$), and "friends" ($p < 0.001$) compared with the control group, whereas in the subclasses "family and free time" ($p = 0.94$) and "school and learning" ($p = 0.07$), the quality of life was similar in both groups (Table 1).

3.3. The effect of age and ability to walk and sit on different subclasses of the quality of life

In the patient group, the effect of patient's age, ability to walk, and ability to sit on different subclasses of the quality of life was analyzed from the parents' perspective. The results of the analysis are shown in Table 2. With increasing age, the quality of life showed significant differences only in the subclasses "physical activity and health" [B: −1.15 ($p < 0.001$)] and "friends" [B: −1.33 ($p = 0.01$)]. On the other hand, the quality of life showed significant differences in "physical activity and health" [B: −3.78 ($p = 0.01$)] and "general mood and feelings" [B: −6.03 ($p = 0.03$)] between the patients who were not able to sit unsupported and those who were able.

Table 1
Scores for five subclasses of KIDSCREEN-27 (child and adolescent version and parent version) in the case group (boys with DMD) and control group.

	Case group	Control group	P-value
	Mean (SD)	Mean (SD)	
Child and adolescent			
Physical activities and health	38.62 (7.54)	51.77 (11.37)	<0.001
General mood and feelings	45.51 (11.50)	46.37 (9.31)	0.54
Family and free time	50.05 (9.31)	50.06 (8.78)	0.99
Friends	38.85 (12.76)	43.29 (8.63)	0.005
School and learning	51.05 (10.66)	52.60 (9.15)	0.25
Parent			
Physical activities and health	36.44 (7.66)	48.18 (9.48)	<0.001
General mood and feelings	39.21 (9.46)	44.96 (10.32)	<0.001
Family and free time	49.55 (9.22)	49.45 (10.41)	0.94
Friends	39.37 (12.20)	47.25 (10.11)	<0.001
School and learning	49.47 (11.96)	46.64 (10.04)	0.07

Table 3
Scores for five subclasses of KIDSCREEN-27 (child and adolescent version and parent version) in the case group (boys with DMD).

Variable, mean (SD)	Child and adolescent	Parent	P-value
Physical activity and health	38.62 (7.54)	36.44 (7.66)	<0.001[a]
General mood and feelings	45.51 (11.50)	39.21 (9.46)	<0.001[b]
Family and free time	50.05 (9.31)	49.55 (9.22)	0.64
Friends	38.85 (12.76)	39.37 (12.20)	0.62
School and learning	51.05 (10.66)	49.47 (11.96)	0.12

[a] $t = 3.9$.
[b] $t = 5.8$.

3.4. Comparison between patient and parent assessment of the HRQOL subclasses

Table 3 compares the results of children's and parents' assessments in different subclasses of KIDSCREEN-27 in the patient group. Significant differences between patient and parent assessments were found in the subclasses "physical activity and health" ($t = 3.9$, $p < 0.001$) and "general mood and feelings" ($t = 5.8$, $p < 0.001$). In other words, the parental estimation of the HRQOL was significantly lower than patients' own assessment in these two subclasses ($p < 0.001$). The correlation coefficients between parents' and children's attitude in the subclasses "physical health and activity", "general mood and feeling", "family and free time", "friends", and "school and learning" were 0.77, 0.55, 0.50, 0.66, and 0.66, respectively, and all these values were statistically significant ($p < 0.001$).

4. Discussion

The present study showed that from the perspective of children and adolescents, the quality of life was similar to boys with DMD when compared to that in healthy controls, except for the subclasses "physical activity and health" and "friends". The scores in the subclasses "general mood and feeling", "family and free time", and "school and learning" were similar in patients and controls, indicating that these subclasses were not affected by DMD. In a study by Opstal et al. on 40 patients, the QOL was lower only in the subclass "physical activity and health", whereas the scores in other subclasses were similar between patients and controls [9]. However, in all other previous studies, all the subclasses of QOL were lower in DMD patients compared with controls [10–13]. We believe that the larger sample size of our study, the size of the control group that

Table 2
Effect of variables on the five subclasses of KIDSCREEN-27 (child and adolescent version) in boys with DMD.

Variable	Physical activity[a]	Mood and feeling[a]	Family and free time[a]	Friends[a]	School and learning[a]
Age (years)	−1.15 (0.27)[b]	−0.19 (0.48)	0.02 (0.39)	−1.33 (0.50)[b]	−0.028 (0.45)
Walking (No/Yes)	2.56 (1.96)	1.88 (3.53)	3.14 (2.28)	−0.83 (3.68)	0.47 (3.27)
Sitting (unsupported) (No/Yes)	−3.78 (1.57)[b]	−6.03 (2.83)[b]	−4.47 (2.30)	−4.87 (2.94)	−4.35 (2.62)

[a] Beta coefficient (standard error).
[b] Statistically significant ($p < 0.05$).

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was about twice the size of the case group, and the type of the questionnaire can explain these differences. In our study, with increasing age, the QOL decreased significantly only in the subclasses "physical activity and health" and "friends".

The findings mentioned previously in this section suggest that boys with DMD have a positive attitude toward life despite the progressive nature of the disease and limitations in physical activities and relationship with friends. Therefore, in order to maintain this positive attitude, the health system should create and implement a plan for improving the social support of these patients and facilitating their participation in social activities. This may include rehabilitation facilities at home, supportive consultations for the families that are provided by social workers who are experts in this field, and educational and recreational programs for parents and patients.

In previous studies, the parents' perception and estimation of their child's feelings and emotions were lower than the children's self-reports [2,13]. In our study, the attitude of the boys with DMD in the subclass "general mood and feelings" was better than their parents' perception and the value of the correlation coefficient between the parent's and their affected child's attitude was significant. One possible explanation for this difference is that parents use their own bad feeling as a basis for estimating their child's limitations. Therefore, it would be recommended to develop social networks for patients' families support. Furthermore, parents should be aware of the differences in opinion between them and their children in order to feel more relaxed and confident and to continue their child's supportive and therapeutic measures with more hope. The results of this study regarding the attitudes of the children and their parents emphasize the importance of self-assessment questionnaires in the evaluation of the QOL in DMD patients.

One of the limitations of our study was that we did not use a quantitative questionnaire for motor function assessment. Opstal et al. used the Vignos and Brooke Scales to assess the patient's functional status and concluded that boys received more love and understanding from their parents when they lost their ability to ambulate or sit or when their ability to use hands decreased, which made them feel happier at home [9].

In our study, patients who lost their ability to walk did not experience a significant psychological and mental decline, whereas the patients who were not able to sit unsupported had severe emotional and mood decline. In other words, lack of psychological support as the disease progresses may be a reason for emotional and mood decline; however, the overwhelming loss of physical strength and function itself may also be a cause for mood decline even with an adequate support. In another study by Baiardini et al., it was seen that children received more attention and care from their families when they are concerned about the child's clinical status and had more knowledge of the progressive nature of the disease [11].

5. Conclusions

In conclusion, our results indicate that boys with DMD have quite a satisfactory quality of life. In addition, boys with DMD have a positive attitude toward life despite the progressive nature of the disease and limitations in physical activities and relationship with friends. Therefore, in order to maintain this positive attitude, the health system should create and implement a plan to improve the social support for boys with DMD and their families and to facilitate their participation in social activities.

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