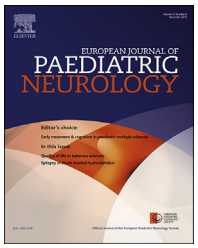
Official Journal of the European Paediatric Neurology Society



Original article

Psychosocial adjustment and parental stress in Duchenne Muscular Dystrophy

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ARTICLE INFO

Article history:
Received 12 June 2019
Received in revised form
12 August 2019
Accepted 16 September 2019

Keywords:
Duchenne muscular dystrophy
Psychosocial adjustment
Parental stress
CBCL
PARS-III
PSI-SF

ABSTRACT

Objective: This cross-sectional study aimed to assess psychosocial adjustment of children with Duchenne Muscular Dystrophy (DMD) and to explore its possible association to parental stress.

Methods: 34 children with DMD, 9–14.1 years of age, and their parents were included in the study. Caregivers completed the Child Behaviour Checklist (CBCL), the Psychosocial Adjustment and Role Skills Scale III (PARS-III) and the Parenting Stress Index–Short Form (PSI-SF). Patients older than 11 years completed the Youth Self Report (YSR). Regression analyses including parental stress, socio-demographic and disorder-related factors were performed to determine how these aspects influence the psychosocial adjustment in children with DMD.

Results: Depending on the measure, 15%–47% of children with DMD were found to be psychosocially "at risk" for emotional and behavioural problems. Age showed no association with psychosocial adjustment. Half of the caregivers experienced very high parenting stress. Moreover, the two aspects parent-child dysfunctional interaction and difficult child scores were associated to psychosocial adjustment. Regression analyses showed that both parental stress and participation in a DMD support group are related to the psychosocial adjustment.

Conclusions: The PARS-III represents a more suitable instrument assessing psychosocial adjustment in DMD, since compared to the CBCL it excludes physiological symptoms regarding chronic diseases. Decreased parents' stress levels and participation in a DMD support group positively contributed to good psychosocial adjustment. A family-centered

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https://doi.org/10.1016/j.ejpn.2019.09.008

approach is crucial for interventions in order to improve the psychosocial adjustment of these children and their families even while living with the significant burdens associated with DMD.

1. Introduction

A major issue for children with chronic medical conditions and physical disability is to cope with the challenges of their chronic disease.[1] Chronically ill or physically disabled individuals are at a much greater risk of significant psychosocial maladjustment, internalizing problems and somatic complaints compared to healthy peers.[2,3] Research has shown a high risk of maladjustment in neurological disorders and those involving motor functioning.[4] For those who must cope with a progressively disabling, terminal illness like Duchenne Muscular Dystrophy (DMD), the psychosocial adjustment process is even more complicated.

DMD is the most common, inherited childhood neuromuscular disorder affecting mainly boys with an estimated incidence of 1:3600 to 6000 among new-born males.[5] It is characterised by a progressive muscle loss, which results in muscle weakness. The impact of the disease can begin as early as age 3, with an impact upon practises of daily life.[6] Gait loss and functional dependence typically occur in the second decade of life.[5] Up to date, the disease has no cure and life expectancy is limited. Death generally occurs by the third decade of life, usually caused by extreme muscle weakness that leads to respiratory or cardiac failure.[5] In view of the devastating outcome of DMD, most attention has been directed toward improving muscle function and structure. However, this perspective neglects the social, psychological, and emotional needs of patients with DMD since they not only face inevitable deterioration of physical functioning, but also become susceptible to emotional and behavioural problems.[7]

At present, there are relatively few studies examining the psychosocial adjustment in DMD and findings concerning psychosocial adjustment are equivocal. Early research indicates that between 30% and 50% of children with DMD reported psychosocial maladjustment and behavioural problems.[7–9] More precisely, symptoms of depression and anxiety, social isolation, and social problems have been reported.[10–12] In contrast, newer research found no indication of decreased psychosocial adjustment or behavioural problems among DMD boys compared to normative data and other chronic medical conditions.[13,14] While some research reports that the psychosocial adjustment in children with DMD is positively associated with increases in age,[13] others found no association between age and child's behaviour.[15] Understanding what may contribute to the psychosocial adjustment among children with DMD is a valuable and necessary information to ensure each child has the best possible quality of life and adjustment to the disease.

DMD may have implications on the psychosocial well-being of the children and their families. Caregivers of children with DMD must not only deal with the stressors most families with chronically ill children encounter, but also the additional stressors associated with one family member's progressively disabling and terminal disorder. Research indicates that the majority of caregivers of children with DMD report higher levels of psychological stress than parents of healthy children or of children affected by other chronic diseases.[16–20] Providing care to a child with DMD is a heavy physical and emotional burden,[21] with factors such as difficulty in accessing adequate and timely health services and managing everyday difficulties were found to contribute to the burden.[22] Moreover, parents of children with DMD were reported to have a higher probability of having a major depressive episode than general population.[16] Landfeldt et al. revealed that half of the 700 investigated DMD caregivers of children with DMD report being moderately or extremely anxious or depressed.[23] Another study found that parents of boys with DMD exhibited great psychological stress and decreased enjoyment of life.[24] Moreover, parents reported increased difficulty in discussing death issues with their children, which only contributed further to the children's feelings of isolation.[7] Most of the parents expressed significant feelings of guilt, and thus were unable to cope appropriately with their grief or help their children cope with theirs. These previous results highlight the association of parental factors to psychosocial functioning of children with DMD and suggest that parental stress may contribute to the psychological adjustment of children with DMD, whereas good parental functioning predicts better psychosocial adjustment.

Accordingly, the current study had the following objectives: (1) to examine the psychosocial adjustment/functioning in children with DMD; (2) to measure parental stress in caregivers of children with DMD and (3) to assess the association between the psychosocial adjustment of children with DMD and parental stress as well as the influence of other sociodemographic and disorder-related factors.

2. Materials and methods

2.1. *Participants*

This observational cross-sectional study was performed with children with DMD who have participated in the past in two investigator-initiated clinical trials[25,26] at the Division of Neuropediatrics, University Children's Hospital Basel. Former participants from both studies were invited to participate for

the observational single visit 2.5 up to 5 years after completion of the clinical trial. Inclusion criteria were child age between 8.5 and 16 years, and being able to provide informed consent and comply with the study procedures. Of the 48 former participants, 34 (71%) subjects, 33 boys and 1 girl, with molecular diagnosis of DMD and their respective caregivers agreed to participate and were included in this study.

2.2. Procedures

Patients were contacted by telephone and email and asked if they are willing to participate in the study. After obtaining an explanation of the study, subjects agreed to participate willingly and voluntarily by signing the informed consent during a study visit at the hospital. All assessments were done by a trained medical doctor and appointments were scheduled at the hospital. All caregivers completed the following questionnaires: the Child Behaviour Checklist 6–18, the Psychosocial Adjustment and Role Skills Scale III and the Parenting Stress Index – Short Form. Further, caregivers answered questions regarding their child's current state of health (disease-specific questions) and other sociodemographic questions. Patients older than 11 years completed the Youth Self Report 11–18.

When families had difficulties to get to the hospital (mainly due to loss of ambulation of the child), they were sent a cover letter, consent form, and return envelope. Families who agreed to participate returned their signed consent forms to the investigators. After signing the consent forms, families received the questionnaires with a return envelope. Additionally, a trained medical doctor called each family to arrange a phone interview in order to clarify questions regarding the questionnaires, assess patient's current state of health and answer sociodemographic and disease-specific questions.

2.3. Measures

2.3.1. Psychosocial adjustment

The level of psychosocial adjustment was assessed by the Child Behaviour Checklist 6–18 (CBCL), the Youth Self Report 11–18 (YSR), and the Psychosocial Adjustment and Role Skills Scale (PARS-III).

The CBCL[27] is a widely used 118-item questionnaire assessing behavioural, emotional, and social problems. Parents rate, on a 0 (never) to 2 (very much) scale, how often their child engages in each behaviour. The CBCL includes 8 syndrome scales (Anxious/Depressed, Withdrawn/Depressed, Somatic Complaints, Social Problems, Thought Problems, Attention Problems, Rule-Breaking Behaviour, and Aggressive Behaviour) and two broadband scales (Internalizing Problems and Externalizing Problems), and a Total Problem scale. Internalizing Problems are problems that are primarily within the individual and include Anxious/Depressed, Withdrawn/Depressed and Somatic Complaints, while Externalizing Problems are problems that mainly involve conflict with other people and their expectations for the child and include Rule-Breaking and Aggressive Behaviour subscales. Higher scores on the CBCL indicate more adjustment problems. The CBCL yields T scores (M = 50, SD = 10), which are derived from a comparison of the individual's score with the appropriate normative group, based upon gender and age. The YSR[27] is a self-report version of the CBCL questionnaire that is addressed to children and adolescents aged 11–18 years.

The PARS-III[28] is a brief parent-completed measure of psychosocial adjustment. All 28 items use a 4-point interval rating scale, ranging from 1 = "never or rarely" to 3 = "always". The PARS-III includes 6 psychosocial subscales (Peer Relations, Dependency, Hostility, Productivity, Anxiety/Depression, and Withdrawal) and a Total Score. Higher scores indicate better adjustment.[28] In the original study by Walker and colleagues conducted with 450 children with a variety of chronic medical conditions, the reliability (coefficient α) of the total summary score was 0.88 overall, with subscales ranging from 0.70 to 0.80.[28] Construct validity of the six subscales was supported by principal component factor analysis and concurrent validity was adequate, as supported by significant correlations in the expected directions with the CBCL.[27]

2.3.2. Parental stress

Parental stress was assessed by the Parenting Stress Index – Short Form (PSI-SF). The PSI-SF[29] is a 36-items self-reported questionnaire developed from the perspective that the stress which a parent experiences is a function of characteristics of both the child and the parent, as well as their unique style of interaction. It includes a Total score and 3 subscales: Parental Distress (emotional distress in the parenting role), Parent-Child Dysfunctional Interaction (problematic parent-child interactions), and Difficult Child (problematic child behaviour or demands). The items of the scale range from 1 (strongly disagree) to 5 (strongly agree). Higher scores indicate greater levels of parenting stress.[29] Raw scores above 33 on the Parental Distress and Difficult Child subscales and above 27 on the Parent-Child Dysfunctional Interaction subscale are considered as clinically elevated. Raw Total score above 90 indicates clinically significant high level of stress scores.[29] The PSI-SF includes a "defensive responding" scale, indicated by low scores on seven items from the parental distress scale, and indicates the degree to which parents may deny or minimize problems. A score lower than 11 on the defensive responding scale is considered "defensive" and the PSI-SF protocol's validity is therefore questionable. Test-retest reliability of the PSI-SF total score and the subscales ranges from 0.68 to 0.85. Internal consistency (alpha) for the short form total score and subscales ranges from 0.80 to 0.91.[29]

2.3.3. Sociodemographic and disorder-related measures

Sociodemographic and disorder-related informations were collected through a questionnaire developed by the investigators. Questions addressed items pertaining to the child's diagnosis, physical function (age, ambulation status, and corticosteroids use), school type of the child, participation in a DMD support group, and marital status of caregivers. The highest level of parental education was used as an estimate of their social-economic status.

2.4. Statistical analysis

Descriptive statistics were generated for demographic and clinical variables and were reported as mean and SD values for continuous variables and frequencies for categorical

variables. Psychosocial adjustment level in the DMD population was calculated by frequencies of clinical syndrome and broadband scales. According to ASEBA Multicultural Manual, as measured by the CBCL and YSR, we considered clinical range scores corresponding to T > 69 for the syndrome scales, and T > 63 for the Internalizing, Externalizing, and Total Problems Scales. Pearson correlation examined the relationship between the CBCL Total Problem Scales and the PARS-III Total score. Moreover, Pearson correlation examined the relationship between age and psychosocial adjustment (measured by CBCL, YSR and PARS-III total scores). One-sample t-tests were performed to compare parental stress level, measured by the PSI-SF, in parents of children with DMD to norm mean subscale scores reported by the scale's authors ($n = 800$ parents of children at well-child clinic visits).[30] For these analyses all participants were included as potential defensive responders were included in the normative sample as well. Six per cent of the parents had a range of scores indicating defensive responding. Pearson correlations examined the relationship between psychosocial adjustment of the children and parental stress. Linear regression analyses were conducted on data from DMD participants to determine the relative contributions of other variables to psychosocial adjustment. For these analyses each of the three psychosocial adjustment (measured by CBCL, YSR and PARS-III) total scores was included as the dependent variable. Parental stress (PSI-SF total score) and child age were used as independent variables. In a second step, the models were refit each time adding one of the following variables: ambulation status, corticosteroid use, school type of the child, support group participation, and marital status and highest level of parental education. These analyses were explorative analyses, results have thus to be interpreted as hypothesis generating and not confirmatory. P values should be interpreted as a continuous measure of evidence against the corresponding null-hypothesis and not as confirmatory. No correction for multiple testing was performed. Data were analysed using R. Significance was set at $p < 0.05$ for all statistical analyses.

3. Results

3.1. Patients' and caregivers' characteristics

Table 1 presents the main sociodemographic, parental and patients' illness data. The mean age of the DMD participants was 11.6 years (SD: 1.34, range 9–14.1 y). Among all participants, 25 of 34 (73.53%) were taking corticosteroids (prednisone or deflazacort). 14 patients (41.20%) were ambulant, 20 were not able to walk 10 m without assistance (58.80%). Many patients visited a school for physically handicapped children (41.18%). No patient needed assisted ventilation. Caregivers' mean age was 44.44 years (SD: 6.28, range 36–57.58 y). Caregivers were primarily mothers (73.53%). The majority of the parents were married (73.53%). The sample was roughly evenly distributed among parental educational levels: 41% had university degree or higher, 35% had vocational education or training, and 21% were high school educated. Ten families participated in a DMD support group (29.41%).

3.2. Psychosocial adjustment

The CBCL syndrome and broadband scales T values (mean and SD) are reported in Figs. 1 and 2. In addition, to ensure that the behavioural outcome data were "clinically relevant", T values above the clinical cut-off (T value up to 69 for the syndrome scales and 63 for the broadband scales) were reported. According to the "clinically significant" range scores for the CBCL Total score presented by the ASEBA Multicultural Manual, 46.88% of the children with DMD had significantly elevated scores as rated by the caregivers. Moreover, 56.25% had elevated Internalizing Problems and 25% had elevated Externalizing Problems. Examination of the YSR self-reports revealed that 21.74% rated to have significantly elevated Total score. Moreover, 30.43% reported Internalizing Problems and 4.35% Externalizing Problems. Fig. 1 shows that parents T values were higher than self-reports for all syndrome scales except for the Thought Problems.

Data from the PARS-III Total score ranged from 50 to 103 (mean: 83.09; SD: 11.65). According to the clinical cut-off for the Total score,[13] 5 patients out of 34 (14.71%) were identified as being at risk for having adjustment problems. Pearson correlation showed a significant high correlation between the PARS-III Total score and the CBCL Total score ($r = -0.82$), indicating that both measures correlate well.

Pearson correlation showed negligible associations between psychosocial adjustment and age (CBCL: $r = 0.12$, YSR: $r = 0.27$, PARS-III: $r = -0.17$).

Table 1 – Sociodemographic, parental and children's illness data.

	N (%) or Mean (SD)
Patients (N = 34)	
Age	11.6 years (SD 1.34)
Males/Females	33 (97.10%)/1 (2.90%)
Non-ambulant	20 (58.80%)
Corticosteroids (current therapy)	25 (73.53%)
Education patients (type of school)	
Regular school	19 (55.88%)
School for physically handicapped	14 (41.18%)
Unknown	1 (2.94%)
Caregiver (N = 34)	
Age	44.44 years (SD 6.28)
Mother	27 (79.41%)
Father	6 (17.65%)
Other	1 (2.94%)
Marital Status	
Married	25 (73.53%)
Divorced	4 (11.77%)
Living with partner	2 (5.88%)
Single	1 (2.94%)
Separated	1 (2.94%)
Unknown	1 (2.94%)
Highest level of parental education	
High school or less	7 (20.59%)
Vocational education or training	12 (35.29%)
University degree or higher	14 (41.18%)
Unknown	1 (2.94%)
Participation in DMD support group	10 (29.41%)

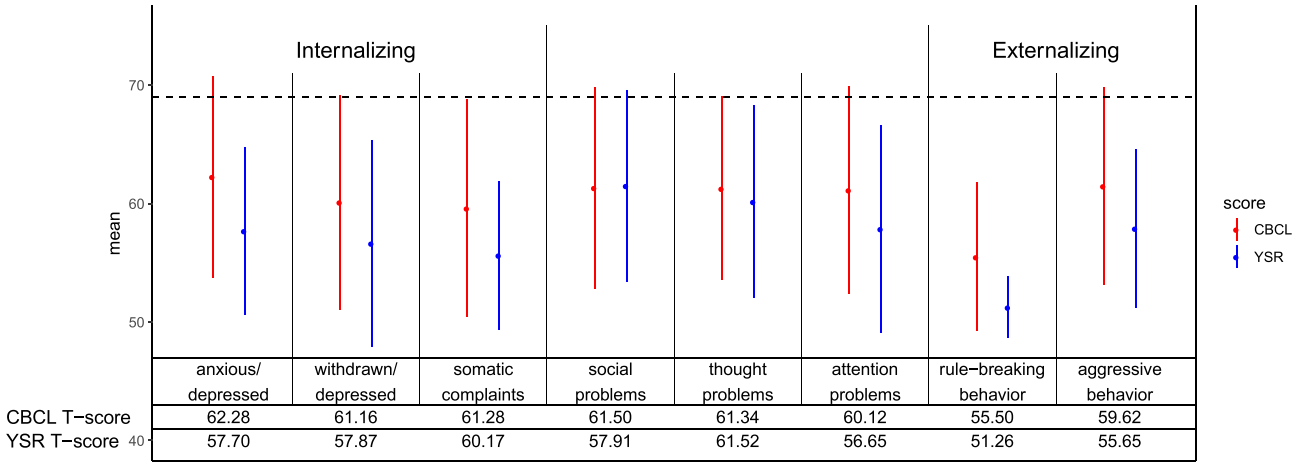

Fig. 1 – Mean and standard deviations of CBCL and YSR T scores of syndrome scales.

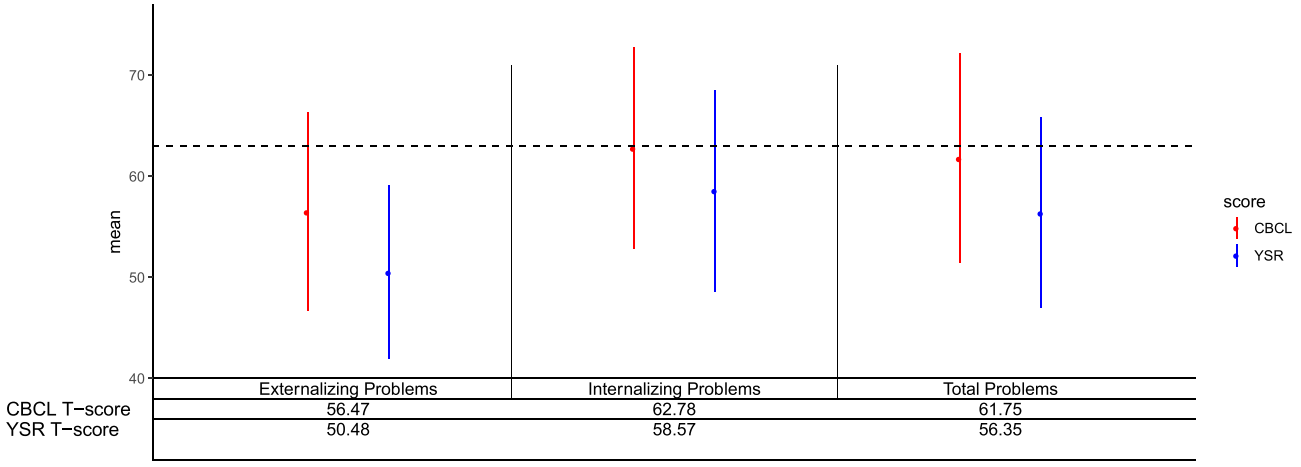

Fig. 2 – Mean and standard deviations of CBCL and YSR T scores of broadband scales.

3.3. *Parental stress*

Parental stress, as measured by the PSI-SF, revealed a mean Total score of 91.67 (SD 20.41), with scores ranging from 53 to 148. Table 2 demonstrates that caregivers of children with DMD reported significantly greater PSI-SF Total score compared to the normative sample's mean ($p < 0.01$). Moreover, mean scores of parental distress ($p = 0.01$), parent-child dysfunctional interaction score ($p < 0.01$), and difficult child score ($p < 0.01$) in caregivers of children with DMD were significantly greater compared to the normative sample. Further, 50.0% of the caregivers had PSI-SF Total scores greater than or equal to the 90th percentile. This rate differs substantially from the comparison norms of parents of healthy children (10%) reported by the PSI-SF's author.[31]

Table 2 – Significant association between parental stress in DMD sample compared to norms of parents of healthy children.

	DMD sample (N = 33)	General population[a] (N = 800)	*p*
	Mean (SD)	Mean (SD)	
PSI-SF			
Total Score	91.67 (20.41)	71.00 (15.40)	<0.01*
Parental Distress	30.76 (9.44)	26.40 (7.20)	0.01*
Parent-Child Dysfunctional Interaction	28.09 (6.77)	18.70 (4.80)	<0.01*
Difficult Child	32.82 (7.38)	26.00 (6.70)	<0.01*

Note: Significant at the acceptable level of *$p < 0.01$.
[a] Parkes, Caravale, Marcelli, Franco, Colver.[30]

3.4. Association between psychosocial adjustment in children with DMD and parental stress

Table 3 illustrates the association between parental stress and patients' psychosocial adjustment assessed by Pearson correlations. There was a strong correlation between PSI-SF Total score and the CBCL Internalizing Problems ($r = 0.72$), CBCL Total score ($r = 0.72$) and moderate correlations with the CBCL Externalizing Problems ($r = 0.64$), the PARS Total score ($r = -0.59$), and the subscores Withdrawal ($r = -0.58$), Anxiety/Depression ($r = -0.57$), and Hostility ($r = -0.52$). PSI-SF Parent-Child Dysfunctional Interaction scale revealed significant high correlations with the CBCL Externalizing Problems ($r = 0.72$) and Total score ($r = 0.71$), and moderate correlations with CBCL Internalizing Problems ($r = 0.68$), PARS Total Score ($r = -0.62$), Anxiety/Depression ($r = -0.62$), Hostility ($r = -0.60$), and Withdrawal ($r = -0.59$). PSI-SF Difficult Child scale showed significant high correlations with the PARS Total score ($r = -0.71$), Anxiety/Depression scale ($r = -0.71$), and the CBCL Total score ($r = 0.82$), and Internalizing ($r = 0.82$) and Externalizing Problems ($r = 0.82$). Moderate correlations were found between PSI-SF Difficult Child scale and PARS Hostility ($r = -0.64$), Withdrawal ($r = -0.53$), YSR Total score ($r = 0.53$) and Externalizing Problems ($r = 0.54$).

Regression model analyses were performed to examine the effects of the following variables on the psychosocial adjustment in children with DMD: parental stress, child age, ambulation status, school type of the child, corticosteroid use, marital status and highest level of parental education and DMD support group participation (see Table 4). The estimates of the PSI-SF Total score were adjusted for age. All other estimates were adjusted for age and the PSI-SF Total score. Significant contributors to the psychosocial adjustment measured by the PARS-III and CBCL were next to the total parental stress also the participation in a DMD support group ($B = -0.23$ and 9.57; $p < 0.01$ and <0.01, respectively). For the self-reports, no significant contributors to the psychosocial adjustment were found.

4. Discussion

The present study investigated psychosocial adjustment in children with DMD and its possible association to parental stress as well to other sociodemographic and disorder-related factors. Our investigation of sociodemographic variables showed that most caregivers are mothers of DMD children and the majority are married. Very few caregivers participated in a DMD support group.

Based on the CBCL, 47% of the included children were found to be psychosocially "at risk" for emotional and behavioural problems. In our cohort, caregivers reported a high prevalence of internalizing and externalizing problems, 56% and 25% respectively. This finding indicates that rates of psychosocial adjustment problems are increased in DMD, which is in accordance with previous publications.[32–34] Additionally, it is noteworthy that self-perception among many children is more positive than what their caregivers indicated. Based on the YSR self-reports, 21.74% of the children rated to have overall high psychosocial adjustment problems while their parents reported higher rates. An explanation of these discrepancies may be that factors such as mental state and level of stress may also influence parents' accounts of their children's problems.[35] Distressed or depressed parents may have a lower tolerance of frustration and regard their children as more of a burden and subsequently report more behavioural problems.[36] In contrast to the CBCL, based on the PARS-III only 15% of children with DMD were identified as being at risk for having adjustment problems. Hendriksen et al. examined psychosocial functioning in a large cohort of boys with DMD using the PARS-III and reported comparable rates of psychosocial adjustment and indicated that patients with DMD are not at a significantly greater risk of psychosocial difficulties than those with other paediatric chronic medical conditions such as seizure disorders, cystic fibrosis and cerebral palsy.[13]

Table 3 – Pearson correlation between psychosocial adjustment and parental stress.

	PSI Total Score		Parental distress		Parent-child dysfunctional interaction		Difficult child	
	r	*p*	*r*	*p*	*r*	*p*	*r*	*p*
PARS								
Total Score	−0.59	<0.01**	−0.20	0.27	−0.62	<0.01**	−0.71	<0.01**
Peers Relations	−0.20	0.26	−0.10	0.60	−0.15	0.42	−0.20	0.27
Dependency	−0.23	0.20	−0.04	0.81	−0.33	0.06	−0.31	0.08
Hostility	−0.52	<0.01**	−0.11	0.55	−0.60	<0.01**	−0.64	<0.01**
Productivity	−0.39	0.03*	−0.17	0.35	−0.31	0.08	−0.47	<0.01**
Anxiety/Depression	−0.57	<0.01**	−0.26	0.14	−0.62	<0.01**	−0.71	<0.01**
Withdrawal	−0.58	<0.01**	−0.37	0.04*	−0.59	<0.01**	−0.53	<0.01**
CBCL								
Total Score	0.72	<0.01**	0.32	0.08	0.71	<0.01**	0.82	<0.01**
Internalizing Problems	0.74	<0.01**	0.43	0.01**	0.68	<0.01**	0.82	<0.01**
Externalizing Problems	0.64	<0.01**	0.18	0.32	0.72	<0.01**	0.77	<0.01**
YSR								
Total Score	0.46	0.03*	0.35	0.10	0.40	0.06	0.53	<0.01**
Internalizing Problems	0.28	0.20	0.29	0.17	0.17	0.43	0.33	0.13
Externalizing Problems	0.44	0.04	0.22	0.31	0.41	0.05*	0.54	<0.01**

Note: Significant at the acceptable level of *$p < 0.05$, **$p < 0.01$.

Table 4 – Multiple regression models of factors influencing psychosocial adjustment to DMD. Each line indicates the estimate from a separate model. The estimate for "parental stress" is adjusted for the patients' age. All other estimates are adjusted for the patients' age and parental stress.

	PARS-III		CBCL		YSR	
	B (95% CI)	p	*B* (95% CI)	p	*B* (95% CI)	p
Parental stress (PSI-SF total score)	−0.23 (−0.40;−0.06)	<0.01	0.74 (0.38; 1.10)	<0.01	0.41 (−0.02; 0.84)	0.06
Marital status (living with partner vs. living alone)	5.60 (−5.09; 16.29)	0.29	−7.57 (−30.45; 15.30)	0.50	−4.89 (−28.97; 19.19)	0.68
School type (special school vs. regular school)	1.59 (−5.73; 8.91)	0.66	−0.72 (−16.58; 15.13)	0.93	3.88 (−16.77; 24.53)	0.70
Ambulation status (no vs. yes)	1.80 (−5.76; 9.36)	0.63	−2.28 (−18.36; 13.80)	0.77	−2.19 (−21.90; 17.52)	0.82
Corticosteroid use (no vs. yes)	2.03 (−6.14; 10.20)	0.62	−1.14 (−19.27; 17.00)	0.90	5.83 (−14.89; 26.55)	0.56
Support group participation (no vs. yes)	9.57 (2.61; 16.53)	<0.01	−18.97 (−33.91; −4.04)	0.02	−7.78 (−29.11; 13.56)	0.46
Parental education* (vocational education vs. high school or less)	−2.66 (−12.85; 7.53)	0.60	11.48 (−11.00; 33.97)	0.30	−3.70 (−30.42; 23.02)	0.77
Parental education* (university degree vs. high school or less)	−1.26 (−11.48; 8.97)	0.80	6.41 (−16.49; 29.31)	0.57	11.80 (−13.94; 37.55)	0.35

Note: *B* (regression coefficient unstandardized), 95% CI (confidence interval). Each line indicates the estimate from a separate model except the last two lines (*), investigating parental education.

Most of former studies investigating psychosocial adjustment in children with DMD so far have used the CBCL, which represents the gold standard of screening and detecting psychopathology in children and adolescents; however, it may not be the most suitable instrument measuring psychosocial functioning in children with a chronic physical illness. Since the CBCL includes a range of items that may be overly sensitive to illness-related variables (items related to somatic complaints), the reliance on the CBCL when assessing psychosocial adjustment in DMD may over-represent psychosocial maladjustment in DMD or mislabel normal behaviour as pathological resulting in false positives.[13] Therefore, in this study we included also the PARS-III. The strength of the questionnaire is that it excludes items based on physiological symptoms (e.g. aches and pains and fatigue), which are part of the chronic disease, and therefore represents a more suitable instrument assessing psychosocial adjustment in children with chronic illnesses.[28]

In our study, the psychosocial adjustment did not vary across different ages. However, it is important to note that our results are to be interpreted with caution as the age range of the included patients is rather small (9–14 y).

As compared to the normative sample, caregivers of children with DMD reported greater parental stress. In our study, half of the caregivers had very high parenting stress – defined here as Total score of 90 or more in a general population sample, where 10% reported very high stress. This means that very high parenting stress among families with a child with DMD is five times more common than in a general population sample. These findings indicate that, indeed, caregivers of children with DMD experience greater stress than a healthy normative group, which is consistent with results of previous studies.[16–20] In particular, the parental stress is related to their children, in that the children's behaviour and interactions with them are more stressful for caregivers of children with DMD than for caregivers of healthy children.

Moderate to high correlations were found between parental stress and psychosocial adjustment levels measured by both the CBCL and PARS-III. A closer look at the associations demonstrated that mainly the parent-child dysfunctional interaction and difficult child subscores correlate with aspects of the psychosocial adjustment (CBCL: Externalizing Problems, Internalizing Problems; PARS-III: Anxiety/Depression, Hostility and Withdrawal). However, the correlations between parental stress and psychosocial adjustment were all negligible. This result indicates that parental stress largely depends on the child's behavioural functioning as well as practical aspects of caring for the child, rather than stress independent from the parent-child interaction. It may be that the experience of having a chronically ill child has more global effects. Therefore, the additional stress leads to an overall lower stress tolerance in these parents, which can lead to poorer parenting skills and coping mechanisms.[19]

Finally, and most importantly, the regression analyses indicated that psychosocial adjustment level in children with DMD is strongly associated with the intensity of parental stress and the participation in a DMD support group. Those findings suggest that parental stress related to parent-child interaction and the participation in a support group is more salient to psychosocial outcomes than the influence of disease progression.

This study has clinical implications for health-care professionals and families with children affected by DMD. Clinicians who care for patients with DMD should assess psychosocial adjustment/functioning regularly through the use of screening measures such as the PARS-III.[37] If concerns of psychosocial maladjustment are identified, a structured or semi-structured interview based on clinical evaluation is needed to accurately assess psychopathology so that a more intensive psychiatric service may be warranted. Further, family variables have been shown to protect against maladjustment in cases of chronic illness and in adverse environments.[15] Parents, who are supportive, involved, and have positive attitudes increase a stress-resilient outcome in their children.[38] Among families living with DMD, family functioning has been shown to be positively associated with child's outcome.[18] Therefore, a family-centered approach that recognizes the family as central to the child's health may be helpful including comprehensive support not only for the affected child but also for the family for example with the participation in a parent-to-parent support group.[39]

If there is reason to believe that a lack of psychosocial adjustment to life with a muscle disorder is a significant contributor to distress, psychological interventions directed at improving acceptance may be an optimal first-line treatment. For example, Acceptance and Commitment Therapy (ACT), a cognitive-behavioural model of disease self-management with acceptance as the central component, aims specifically to improve an individual's ability to persist with or to adopt behaviour patterns in line with deeply held values.[40] In the context of a muscle disorder, a key process in improving adjustment might be helping someone find new ways of expressing personal values despite functional limitations, while accepting both of those limitations and the negative thoughts and feelings they are likely to have. ACT might be applied to address the issues of distress, nonadherence to treatments, pain, and fatigue in people with muscle disorders.[41]

These results should be regarded as preliminary and with some limitations in mind. Firstly, the lack of a control group is a weakness, but the questionnaires have all been used extensively in non-clinical samples allowing comparisons with general population norms. Since our focus was mainly on the psychosocial adjustment of children with DMD and the association to parental factors, we did not include further control groups. Considering that DMD may have implications on the well-being of all family members, future studies should include also data of unaffected siblings. A study investigating the psychosocial adjustment in unaffected siblings of young people with DMD reported that although siblings mean psychological functioning and well-being scores were comparable to normative data, there was a trend for more siblings scoring at high-risk for psychological (mainly emotional) problems.[42] Also, future studies could investigate the psychosocial adjustment in children with DMD, their unaffected siblings and parents in comparison to families affected by other neuromuscular diseases. Secondly, even though the sample size is appropriate for a monocentric study, it is relatively low for the range of age and clinical phenomenology. Using self-reported questionnaires in general may lead to an under/overestimation of the true rate of psychosocial maladjustment in this special clinical population. Therefore, patients that are above the clinical cut-off should be evaluated by clinically structured interviews to detect the rate of emotional and behavioural problems. Since caregivers were predominantly female, the generalisability of results is restricted. Future research should include other factors which may be associated to the psychosocial adjustment such as the cognitive functioning or behaviour diagnoses of the child and socio-economic status. In addition, future studies should use longitudinal designs to investigate how key variables change over critical time periods.

5. Conclusions

In the present study, psychosocial adjustment in 34 children with DMD and stress among their caregivers were assessed. Our data indicate that depending on different measures between 15% and 47% of children with DMD exhibited psychosocial adjustment problems. Additionally, it is noteworthy that self-perception among children with DMD was more positive than what their caregivers imagine. Further, half of the caregivers of children with DMD experienced very high parenting stress. Low parental stress along with the participation in a DMD support group were contributory factors to better psychosocial adjustment of children with DMD. A family-centered approach for interventions is needed in order to improve the psychosocial adjustment of these children and their families.

Ethics approval and consent to participate

Eligible subjects were included in the study only after providing written informed consent. Ethics approval has been obtained from the local Ethics Committee (EKNZ 2017-01028).

Consent for publication

We confirm that (1) the authors of this manuscript had access to all study data, are responsible for all contents of the manuscript, and had authority over the preparation of the manuscript and the decision to submit the manuscript for publication and (2) all authors have read and approved the submission of this manuscript to the journal.

Availability of data and material

Data used in the analysis is available upon request from the corresponding author. Patient-level data remains confidential under patient data privacy regulations.

Authors' contributions

VG participated in the design of the study, acquired data and drafted the manuscript. PH, AO and SiS participated in the design of the study and acquired data. VG and NR participated in patient recruitment. NR and VG participated in the organization and the conduct of the study. SaS performed the statistical analysis. PW revised the manuscript critically for important intellectual content. DF designed the study, analysed data and drafted the manuscript. All authors read and approved the final manuscript.

Conflict of interest

DF is principle investigator for studies on spinal muscular atrophy sponsored by Hofmann-La Roche Ltd. There are no other activities related to commercial companies. All authors declare that they have no conflict of interest.

Authors' information

Not applicable.

Acknowledgements

We would like to acknowledge our patients who participated in the trial and made a major contribution to advance knowledge in Duchenne muscular dystrophy. This study was funded by the Neuromuscular Research Association Basel (NeRAB), Switzerland, we'd like to thank for this financial support. We also would like to acknowledge Sabine Hobi for linguistic revision.

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