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Psychosocial adjustment in adults with Duchenne muscular dystrophy: A pilot study on a shortened parent-report questionnaire

Pien M.M. Weerkamp[a,*], Philippe Collin[a,b], Rosa J. Maas[a], R. Jeroen Vermeulen[c,d], Sylvia Klinkenberg[a,c,d], Jos G.M. Hendriksen[a,d,e]

[a] *Kempenhaeghe Centre for Neurological Learning Disabilities, Heeze, Netherlands*
[b] *Koraalgroep Gastenhof, Maasbracht, Netherlands*
[c] *Maastricht University Medical Centre, Department of Neurology, Maastricht, Netherlands*
[d] *Maastricht University, School for Mental Health and Neuroscience, Maastricht, Netherlands*
[e] *Duchenne Center Netherlands*

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Abstract

The primary aim of this study was to describe the psychometric properties of an adult revision of the 28 item Personal Adjustment and Role Skills Scale (PARS-III). This scale was originally developed to assess psychosocial adjustment in children 4–18 years of age and has been applied in boys with Duchenne muscular dystrophy (DMD) and was found to be reliable and valid. Within the context of a longer lifespan in dystrophinopathies there is a growing need to assess psychosocial adjustment in an adult population. The original 28 items questionnaire was administered to parents of 90 adult men with DMD. The items of the PARS-III were rated by three experts, one parent, and one adult with DMD to indicate appropriateness of the items. For 22 items, there was consensus among the raters. Results of the Confirmatory Factor Analysis show an acceptable fit and closely resembles the original factor structure of the PARS-III, thereby justifying the use of the previously identified six subscales of psychosocial adjustment. In conclusion, the current 22 item PARS-Adult is a valuable, reliable, and valid screening of psychosocial adjustment in adult DMD patients. With this tool, continuity of assessment and follow up can be guaranteed in this clinical population.

Keywords: Adult; Duchenne muscular dystrophy; Psychosocial adjustment; Screening.

1. Introduction

Duchenne Muscular Dystrophy (DMD) is the most common inherited childhood neuromuscular disorder. DMD is an X-linked progressive disorder. The worldwide estimated prevalence is 4.78 per 100,000 males [1]. The absence of dystrophin as in DMD leads to muscle weakness and subsequent physical symptoms such as fatigue, respiratory difficulties, cardiac complications. Boys with DMD usually get diagnosed between 2-7 years [2], and become wheelchair dependent by age of 12. Life expectancy has significantly increased in the last two decades: patients with DMD are now expected to live into their 30s and beyond [3].

As dystrophin is known to play a role in brain functioning there is growing interest in brain involvement. Brain related comorbidities such as problems with learning and IQ as well as behavioral problems such as autism spectrum disorders and attention deficit disorders, are now well described and need special care in diagnostics, treatment, and rehabilitation [4,5]. Many men living with DMD are psychosocially well adjusted [6]. More limiting factors e.g., physical restrictions and medical complications may hinder a healthy transition from adolescence to adult life, as (young) men with DMD rely more on others during daily activities. Loss of ambulation reduces independence and increases the risk of becoming isolated from peers [7]. After high school, personal contact

* Corresponding author.
E-mail address: weerkampp@kempenhaeghe.nl (P.M.M. Weerkamp).

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and social engagement become more challenging in young adulthood for patients with DMD [8]. As the disease progresses, there is an increased risk of depression and anxiety [6,9,10]. Moreover, interviews with adults with DMD indicate that they experienced anxiety when confronted with the progressiveness of the disease [11]. They felt anxious or worried when they transitioned to using a wheelchair and started using a respirator. However, the literature on anxiety and depression in DMD is diverse. As mentioned before, many men with DMD are psychosocially well adjusted [6], which is in line with results of [12] Hendriksen et al. (2009) and [13] Elsenbruch et al. (2013) reporting improving ability to adjust with age. Presumably, patients with DMD learn to adjust to the more advanced stages of the disease in general, but when confronted with their worsening health they experience anxiety and depression. These findings emphasize the importance and the challenge of assessing coping with and adjustment to a chronic and progressive disorder from childhood into adulthood.

The standards of care for DMD strongly advice regular mental health screening for males with DMD [4]. Four instruments are recommended: the Patient Health Questionnaire (PHQ-9; [14]) depression scale, the Generalized Anxiety Disorder 7-item scale (GAD-7; [15]), the Strength and Difficulties Questionnaire (SDQ; [16]) for mental health problems, and the Personal Adjustment and Role Skills Scale-III (PARS-III). The PARS-III parent-reported questionnaire assesses six domains of psychosocial adjustment i.e. dependency, peer relations, anxiety, productivity, withdrawal, and hostility, where psychosocial adjustment is defined as "the adaptive task of managing frustrations and upsetting feelings provoked by a disease, and preserving an emotional balance" [17]. The PARS-III has been specifically developed to measure psychosocial adjustment in children with chronic physical illnesses [18]. Hendriksen et al. (2009) [12] reported the PARS-III to be a promising and useful clinical and research measure for boys and adolescents with DMD. One important shortcoming of the PARS-III is the age range of 5 to 17 years. An instrument to measure psychosocial adjustment in adult men with DMD has not been developed yet. This raises the question whether the PARS-III might be a reliable and valid instrument to measure psychosocial adjustment in an adult (DMD-) population.

As the items are originally formulated to address maladjustment behavior in children (e.g., does not respond to discipline) we hypothesized that some of the original PARS-III items need to be adjusted or removed from a new adult version of the PARS-III (PARS-Adult, [PARS-A]). The primary aim of this study was to establish the psychometrics of the new shortened PARS-A for use in the adult DMD population. We expect that after the removal of some of the items the structure of the PARS-A will be identical to the one documented in the original study by [19] Walker et al. (1990) (hypothesis 1). We also hypothesized that the PARS-A will reach acceptable levels of reliability and validity (Hypothesis 2). Our secondary aim was to assess psychosocial adjustment in adult males with DMD. As described earlier, overall psychosocial adjustment increases with age [20,21] which was also seen within the DMD population [12,13]. Therefore, we expect adults with DMD to have higher scores on PARS items, reflecting better adjustment as compared to boys with DMD (hypothesis 3). The third aim was to describe a cutoff score for the shortened adult version of the PARS-A.

2. Methods

2.1. Participants

Parents of patients with DMD were invited to participate by letter /email/information via several platforms: 1) parents of patients registered in the database of Leiden University Medical Center (LUMC), 2) websites of University Medical Centers (UMC); patient associations (Spierziekten Nederland and Duchenne Parent Project) and a Dutch center specialized in Duchenne (Duchenne Centrum Nederland) and 3) practitioners of a Dutch workgroup called All Against Duchenne in the Netherlands (ALADIN). Informed consent was obtained from all participants and/or their legal guardians.

In 2009 Hendriksen et al. [10] published a study on the psychometric properties and clinical utility of the PARS-III in boys and males with DMD, focusing on children. Data on the PARS-III of adults (>18 years) were excluded.

Combining data of the study of Hendriksen et al. (2009) [12] and our study, 110 Dutch parents and 275 American parents participated (see Fig. 1). The mean age of the males was 13.39 (SD = 6.81) with a minimum age of 3 years and maximum of 41 years old. As this study focusses on the DMD adult population, 292 males of 17 years or younger were excluded from analysis. Participants with incomplete data on the PARS-III were also excluded. The final sample included data from 90 parents of patients with DMD (N=90) in total. The mean age of the males was 23.54 (SD= 5.84), with a minimum age of 18 years and a maximum age of 41 years. To estimate parents' socioeconomic status (SES), they were asked to rate their educational status on a 5-point scale ranging from "some high school or less" to "professional or graduate degree", as previously done in the Child Health Questionnaire [22]. The Shapiro Wilk test was performed to test whether educational status was normally distributed in our study population (D [76]= 0.86, $p < .001$). The distribution was negatively skewed toward higher levels of educational attainment, suggesting a response bias toward a higher SES than expected in the general population. Next to that, we estimated how many of the participants are living with their parents/caregivers. Of the 35 responses in our sample, 91.4% of the men with DMD were living with their parents. So, we believe that a by proxy version is feasible for adult males with DMD, especially for those who are living at their parents' home.

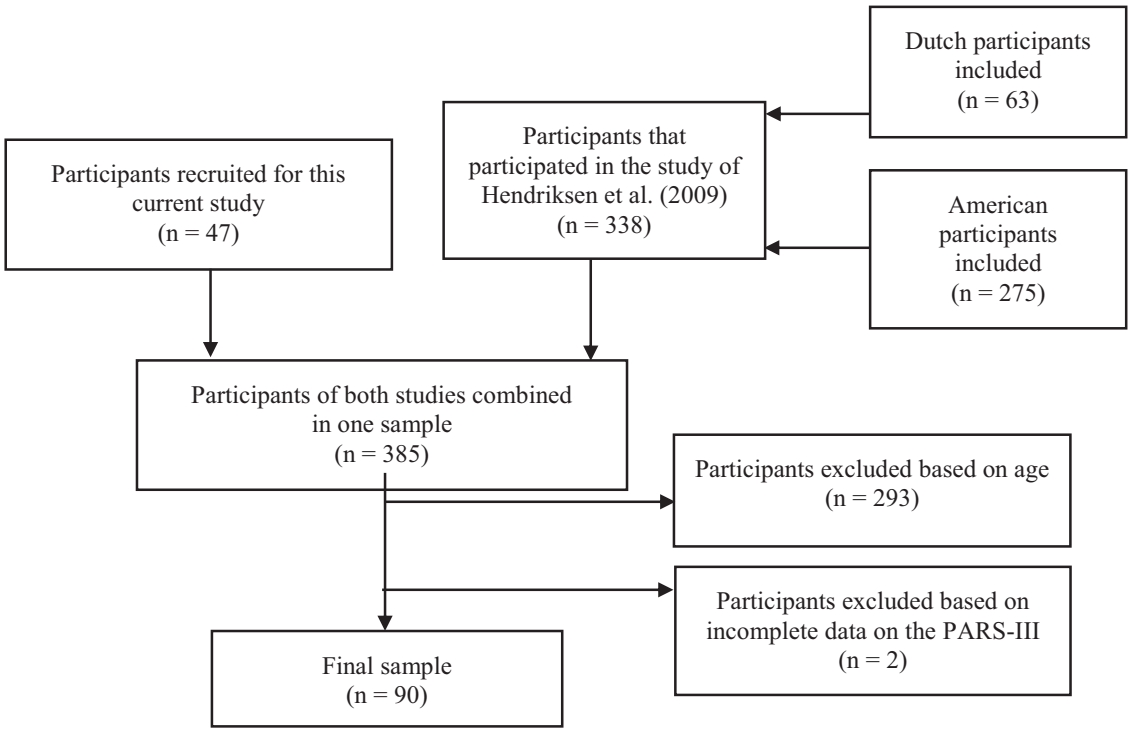

Fig. 1. Flowchart of the final study sample.

2.2. Measures

Parents were asked to complete the PARS-III and other various items assessing demographics (age of patient with DMD, parental educational status, living situation), and disease parameters (e.g., use of steroids) were included.

The Psychosocial Adjustment and Role Skills Scale III [18] is a standardized questionnaire of 28 items originally designed to measure youth psychosocial adjustment. Parents were asked to rate how often their son showed certain behaviors on a 4-point Likert scale: “never or rarely”, “sometimes”, ‘often’, “always”. Twenty items are based on reversed scoring. The total score is calculated by summing up scores on the items, resulting in a possible minimum score of 28 points and a maximum score of 112 points, where higher scores indicate better adjustment. The items can be divided into six psychosocial subscales: peer relations, dependency, hostility, productivity, anxiety/depression, and withdrawal. Previous research [12] showed that the PARS-III scale is a valid and reliable index to assess psychosocial adjustment in boys with DMD (<18 years old). Furthermore, it was found that a total score below 71 indicates a higher risk for adjustment problems.

In order to attain the best possible PARS-III version for adults (PARS-A), items of the original PARS-III were separately and independently presented to three experts in clinical and psychological care, one adult patient, and one parent. They were asked to indicate whether the items were appropriate for a parent to answer about their adult son with DMD. Fleiss multi-rater Kappa was used to measure the inter-rater reliability between the five independent reviewers. A kappa value of $\kappa \geq .60$ indicates substantial agreement [23]. If no consensus was reached between the reviewers, an item was deleted. The six removed questions were not spread evenly across the original domains; the subscales peer relations, anxiety/depression, and withdrawal have the same number of items on the PARS-A as on the PARS-III; one item got excluded from both the dependency, and productivity subscale; and four questions from the hostility subscale. Consequently, there were 22 items appended in further analysis.

2.3. Statistical analysis

Data analysis was performed using SPSS (IBM, USA, version 26). Initially, demographic variables were calculated (see Table 1). To examine whether the 22 items of the PARS-A loaded on the six domains as predicted, a structural equation-based confirmatory factor analysis (CFA) was conducted as suggested in literature [24,25] using AMOS (version 26) . CFA is a method to verify a factor structure that has already been defined. The Comparative Fit Index (CFI), Root Mean Square Error of Approximation (RMSEA), and standardized root mean square residual (SRMR) were calculated to evaluate the goodness-of-fit for the factor solution. A CFI-value close to .90, and the SRMR of $\leq .09$ suggest a good fit according to the combination rules recommended by Hu and Bentler (1999) [26]. A RMSEA value between .08 and .10 is a mediocre fit and a value of $\leq .08$ a good fit ([27]. To interpret the internal consistency, reliability coefficients (Cronbach’s alpha) was calculated for total scores and scores on the six subscales of PARS-A. For

Table 1
Patient characteristics of the adult DMD sample ($N = 90$).

Variable	*N*	%	Mean	*SD*	Range
Clinical diagnosis					
Duchenne muscular dystrophy	90	100			
Country					
The Netherlands	50	55.6			
United States of America	40	44.4			
Age in years			23.54	5.84	18 - 41
Highest parental education[1]			4.00	1.16	(1 - 5)
1 (some high school or less)	5	5.6			
2 (high school degree or GED)	9	10.0			
3 (vocational school or some college)	17	18.9			
4 (college degree)	29	32.2			
5 (professional or graduate degree)	18	20.0			
Steroid use[2]					
Yes	23	25.6			
No	46	51.1			
Not anymore	8	8.9			
Lives with parents[3]					
Yes	32	91.4			
No	3	8.6			

Note. [1]12 missing values; [2]13 missing values; [3]55 missing values.
Abbreviations used: GED=General Educational Development; *N*=number; SD = standard deviation.

total scores, a coefficient of $\alpha \geq .80$ was set as a minimum limit of acceptance and for the six subscales a coefficient of $\alpha \geq .70$ [28].

3. Results

3.1. Primary Analysis

Fleiss Multirater Kappa was calculated to determine the level of agreement between the five independent reviewers on the items of the original PARS-III. A substantial strength of agreement was reached, $\kappa = .69$.

The results of the Confirmatory Factor Analysis are presented in Table 2. Our data provided an acceptable fit with a CFI of .88, RMSEA of .082 and SRMR of .085. With this acceptable fit a supplementary EFA is not necessarily recommended [29]. Factor loadings ≥.50 and estimated item-factor correlations are shown in Table 2. Factor loadings on the 22 items of the PARS-A closely correspond with the factor loadings on the 28 items of the PARS-III. The factors included in our model resemble the original factor structure of the PARS-III closely, thereby justifying use of the six subscales of psychosocial adjustment that were previously identified.

The Pearson correlations corrected for attenuation are presented in Table 3. Subscale inter-correlations provide information on construct validity. Correlations among subscales ranged from .21 ($p > .05$) to .66 ($p < .001$) indicating that some subscales were significantly related (see Table 3).

Mean scores, *SD*, *SE* and α-coefficients for the total PARS-A score and each subscale are provided in Table 4. The descriptive data suggest that 59.1 (mean – 1*SD,* as proposed by Pless et al., (1994) [30] and Witt et al., (2003) [31] would be an appropriate cutoff score for the PARS-A.

Cronbach's α of the total PARS-A scale was .87, indicating good internal consistency. The subscales showed acceptable internal consistency with α-values ranging from .70 for productivity to .89 for peer relations.

We compared our data of adult males with DMD to a group of boys <18 years with DMD [12] (Table 5). Our preliminary data show that on average, the total

PARS-A score was higher within the adult group ($\mu = 68.51$, $SD = 9.43$) than within the children group ($\mu = 65.63$, $SD = 9.26$). However, our data on the different (sub)scales were not normally distributed, as assessed by the Shapiro-Wilk test ($p < .001$). Nonetheless, non-parametric comparisons of our data also indicate better psychosocial adjustment in adults with DMD than in boys with DMD. Subscale comparisons showed that the adult males also scored significantly higher on hostility and dependency (see Table 5), indicating lower perceived hostility and dependency in adults than in boys with DMD. Scores on productivity and anxiety/depression subscales were also higher in adults, but these group differences were not significant. Regarding the peer relations and withdrawal subscales, adults with DMD scored lower than boys with DMD, but these differences were also not statistically significant.

4. Discussion

To our knowledge this is the first study to describe assessment of psychosocial adjustment in an adult sample of DMD patients. Until now only the childhood version of the PARS-III (age 4-18 years) has been used as according to the recent standard of care [4]. Based on the childhood version of the PARS-III [18] we constructed an adjusted adult version (PARS-A) which was validated by a group of DMD experts. Our results show that the 22-item PARS-A by proxy questionnaire is a reliable and valid instrument for screening psychosocial adjustment in a DMD adult population. The six previously identified domains of psychosocial adjustment in boys with DMD could be replicated in an adult population (hypothesis 1) and the internal consistency of the total PARS-A was good ($\alpha = .87$), and acceptable for the subscales with α-values ranging from .70 to .89 (hypothesis 2). The data in our sample suggest a preliminary clinical relevant cutoff point of 59.1 for the PARS-A. Due to the reduced number of items, this cutoff score is lower than the cut-off of 72.3 estimated by [12] Hendriksen et al. (2009) for the PARS-III.

In keeping with earlier findings [6,12,13,32], psychosocial functioning improves with advancing age. Using the PARS-A we found that adult males with DMD score higher on the PARS items than children and adolescents, suggesting better psychosocial adjustment with older age (hypothesis 3) and thus showing better coping mechanisms despite the progressive nature of DMD. Interpretation of comparisons between adults and children on the six domains should be done with caution. However, as the structure of the PARS-III and PARS-A is similar, it is possible to monitor patients psychosocial functioning into adulthood. Our suggestion is to assess children and adolescents with DMD with the PARS-

Table 2
Short item descriptions and results of confirmatory factor analysis: Factor loadings and estimated correlations (in italic).

Item description	**Factor loadings and estimated correlations**											
	F1		**F2**		**F3**		**F4**		**F5**		**F6**	
1. Spent time with friends	.75	*.13*		*.00*		*.00*		.00		*.00*		*.01*
2. Made friends without difficulty	.83	*.16*		*.00*		*.00*		.00		*.00*		*.01*
3. Joined others of own accord	.86	*.23*		*.00*		*.00*		.01		*.00*		*.01*
4. Had many friends	.85	*.21*		*.00*		*.00*		.01		*.00*		*.01*
5. Wanted help in things		*.00*	.66	*.00*		*.00*		.02		*.00*		*.01*
6. Unable to decide for himself		*.00*	.50	*.10*		*.00*		.01		*.00*		*.00*
7. Asked for help		*-.01*	.91	*.05*		*.01*		.10		*.00*		*.03*
8. Flared up if could not have own way		*-.01*		*.50*	.90	*.44*		-.01		*.06*		*-.01*
9. Became upset if others did not agree		*-.01*		*.01*	.88	*.35*		-.01		*.05*		*-.01*
10. Stayed with task until finished		*.00*		*.01*		*.00*	.50	-.01		*.00*		*.01*
11. Made full use of abilities		*.00*		*.02*		*.00*	.70	.08		*.00*		*.01*
12. Kept on task even when difficult		*.01*		*.04*		*.00*	.79	.18		*.00*		*.02*
13. Complained about problems		*.00*		*.00*		*.02*		.26	.66	*.07*		*.02*
14. Seemed restless		*.01*		*.00*		*.04*		.00	.82	*.14*		*.04*
15. Said people didn't care about him		*.00*		*.00*		*.03*		.00	.69	*.11*		*.03*
16. Seemed sad		*.00*		*.00*		*.03*		.00	.76	*.12*		*.03*
17. Said he couldn't do things right		*.00*		*.00*		*.03*		.00	.76	*.11*		*.03*
18. Acted afraid		*.01*		*.00*		*.04*		.00	.81	*.14*		*.04*
19. Stared without doing anything		*.02*		*.02*		*-.01*		.02		*.04*	.76	*.22*
20. Appeared listless		*.02*		*.02*		*-.01*		.02		*.04*	.75	*.21*
21. Seemed unaware of things		*.01*		*.01*		*.00*		.00		*.02*	.56	*.08*
22. Showed little interest in things		*.01*		*.01*		*-.01*		.01		*.02*	.67	*.12*

Note. F1 = peer relations; F2 = dependency; F3 = hostility; F4 = productivity; F5 = anxiety/depression; F6 = withdrawal.

Table 3
Correlation Matrix (Pearson Coefficients) for the six subscales.

	Peer relations	Dependency	Hostility	Productivity	Anxiety/depr	Withdrawal
Peer relations	1.00					
Dependency	.08	1.00				
Hostility	.03	.21*	1.00			
Productivity	.12	.42***	.10	1.00		
Anxiety/depr	.22*	.31***	.66***	.20	1.00	
Withdrawal	.34***	.42***	.35***	.31***	.56***	1.00

Note. $^{***}p < .001$; $^{*}p < .05$.
Abbreviations used: depr=depression.

Table 4
Mean Scores, *SD,* SE, and α-Coefficient for the PARS-A total scale and six subscales.

	Mean	***SD***	**SE**	***N***	**α-coefficient**	**Number of items**
PARS total	68.51	9.43	.994	90	.873	22
Peer relations	8.40	3.43	.362	90	.893	4
Dependency	9.42	2.01	.211	90	.720	3
Hostility	6.93	1.53	.161	90	.882	2
Productivity	8.48	2.23	.235	90	.700	3
Anxiety/depression	20.84	3.48	.366	90	.883	6
Withdrawal	14.43	2.02	.213	90	.776	4

Abbreviations used: *N*=number; PARS-*A*=Personal Adjustment and Role Skills Scale Adult; SD=standard deviation; SE=standard error.

III (as recommended by the Standards of Care [4], and adult males with DMD with the 22 items of the PARS-A.

A limitation of our study is the non-longitudinal follow-up design of our cohort study. For the current study, we used a convenience sample of 90 patients. It consists however of the largest adult DMD population assessed until now with this questionnaire. Secondly, data came from parents of adult patients instead of the adult patients themselves. This might have led to distorted data. For example, the so called 'disability paradox' should be considered within our population. This term was used by Albrecht & Devlieger (1990) [33] describing the fact that most external observers find it counter intuitive that patients who have serious and lasting disabilities have a good quality of life (QOL). Landfeldt et al. (2016) [6] encountered the same tendency in a large cohort of boys with DMD reporting lower QOL

Table 5
Psychosocial adjustment scores of adult males with DMD compared to boys with DMD in the study of Hendriksen et al. (2009).

	Number of items	Adults with DMD ($N=90$), μ	Boys with DMD ($N=262$), μ	Adults with DMD ($N=90$), M	Boys with DMD ($N=262$), M	Wilcoxon Signed-Rank Test
PARS total	22	68.51 (9.43)	65.63 (9.26)	70 (9.43)	67 (9.21)	2.58*
Peer relations	4	8.40 (3.43)	8.91 (3.07)	8 (3.43)	9 (3.10)	−1.71 NS
Dependency	3	9.42 (2.01)	8.92 (1.76)	10 (2.01)	9 (1.75)	2.21*
Hostility	2	6.93 (1.53)	5.68 (1.70)	8 (1.53)	6 (1.68)	4.91***
Productivity	3	8.48 (2.23)	7.77 (2.19)	9 (2.23)	8 (2.20)	1.93 NS
Anxiety/depression	6	20.84 (3.48)	20.02 (2.92)	22 (3.47)	21 (2.96)	0.55 NS
Withdrawal	4	14.43 (2.02)	14.32 (1.98)	15 (2.02)	15 (1.97)	−1.86 NS

Note. ***$p < .001$; *$p < .05$.
Abbreviations used; DMD=Duchenne muscular dystrophy; μ=mean; M=median; N=number; NS=not significant.

as reported by caregivers. It would have been useful to have a self-report measure next to the by proxy reports. However, a self-report version of the PARS-III/PARS-A does not yet exist. Another concern to our study is the relatively high level of parental educational attainment, which may suggest that there is a response bias toward higher SES. As parental SES may influence somatic and psychological health in adolescence and early adulthood [34], a higher SES may have been a bias in our study. Another limitation is that parents and caregivers completed the original (child-oriented items) of the PARS-III which contained six items that were rated inappropriate by adult men with DMD. This may have had a demotivating impact on the recipient, and it may have a mild negative skew to our results. In the current study, we used the by proxy version as is used in children to test the validity of the psychosocial adjustment concept in adult DMD patients. As validity has been proved, future research can be done with the 22-item PARS-A questionnaire as a by proxy and a self-report version in a greater sample of subjects. Along those lines, convergent evidence of the

PARS-A scores should be evaluated to determine sensitivity and specificity for a cut-off score. Suggesting to measure the PARS-A against other questionnaires recommended by the standards of care [4] e.g., the PHQ-9 or GAD-7. Furthermore, future research should consider that the living situation of an adult men with DMD (e.g. with parents, in a special sanatorium or living independently) may be an important predictive factor for psychosocial adjustment. The data of our current study do not allow such an analysis as the living situation of 58 patients was unknown.

In conclusion, our preliminary data show that the current 22 item PARS-A is a valuable, reliable, and valid screening of psychosocial adjustment in adult DMD patients older than 18 years. As of their increased life expectancy, closely following up transition from childhood into adulthood is highly essential in the care for Duchenne patients. In using an adjusted –adult-version of the previously reported childhood version [12], continuity of assessment and follow up can be guaranteed in this clinical population. In the light of the recently published revision of the standards of care [4], the PARS-A is a new, essential, and easy to administer screening instrument to be included in clinical practice. The preliminary cutoff score of 59.1 should be used with caution, but if a patient obtains a score below this cut-off point, further clinical driven assessment is advised.

Compliance with Ethical Standards

Conflict of Interest

The authors declared no potential conflicts of interest with respect to the research, authorship and/or publication of this article.

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