Instruments for the Assessment of Behavioral and Psychosocial Functioning in Duchenne and Becker Muscular Dystrophy; a Systematic Review of the Literature

Citation for published version (APA):

Hellebrekers, D. M. J., Lionarons, J. M., Faber, C. G., Klinkenberg, S., Vles, J. S. H., & Hendriksen, J. G. M. (2019). Instruments for the Assessment of Behavioral and Psychosocial Functioning in Duchenne and Becker Muscular Dystrophy; a Systematic Review of the Literature. *Journal of Pediatric Psychology*, 44(10), 1205-1223. <https://doi.org/10.1093/jpepsy/jsz062>

Document status and date:

Published: 01/01/2019

DOI:

[10.1093/jpepsy/jsz062](https://doi.org/10.1093/jpepsy/jsz062)

Document Version:

Publisher's PDF, also known as Version of record

Document license:

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Instruments for the Assessment of Behavioral and Psychosocial Functioning in Duchenne and Becker Muscular Dystrophy; a Systematic Review of the Literature

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Received February 13, 2019; revisions received July 3, 2019; accepted July 5, 2019

Abstract

Objective This systematic review aims to provide an overview of instruments used to assess behavioral and psychosocial functioning of patients with Duchenne and Becker muscular dystrophy, as well as to review the psychometric properties and applicability of these instruments.

Methods Five databases (Embase, Psyc.info, ERIC, Pubmed/Medline, and Cochrane) were searched from inception to June, 2018. Potential articles were rated by two independent reviewers. A predefined PROSPERO form (CRD42017074518) was used to extract data from included articles.

Results Sixty-one instruments were used in 54 studies. The Child Behavior Checklist is commonly used, but it lacks disease specific psychometric information. Sixteen instruments that contained disease specific psychometric information were included for final evaluation. The results displayed three instruments that are potentially valid for screening of psychosocial problems: The Psychosocial Adjustment and Role Skills Scale 3rd edition, the Pediatric Quality of Life Inventory Generic module, and the Life Satisfaction Index for Adolescents with Duchenne muscular dystrophy. Appropriate instruments for screening of behavioral problems may be: the Strengths and Difficulties Questionnaire, the Generalized Anxiety Disorder-7 item questionnaire, and the Patient Health Questionnaire-9 item questionnaire. **Conclusions** Further research on psychometric properties of screening instruments is crucial to ascertain a gold standard for clinical and research purposes. Meanwhile, for definite diagnostics purposes we recommend a multimethod, multi-source, multisetting assessment in this high-risk population.

Key words: systematic review; muscular dystrophy; Duchenne-Becker; instruments; screening; diagnostics; behavior; psychosocial; psychometrics.

Introduction

Duchenne muscular dystrophy (DMD) and Becker muscular dystrophy (BMD) are allelic disorders covering the spectrum of X-linked dystrophinopathies, and

are further mentioned as the dystrophinopathy population. Both disorders are caused by mutations in the *DMD* gene, that encodes multiple dystrophin isoforms in various tissues (i.e., muscles and the brain)

(Doorenweerd et al., 2017; Hendriksen et al., 2015; Muntoni, Torelli, & Ferlini, 2003). Depending on mutation location one or more dystrophin protein isoforms are absent in DMD, resulting in progressive muscle wasting (Hendriksen et al., 2015; Muntoni et al., 2003). By contrast, in BMD, gene mutations cause an open reading frame with a milder and more variable phenotype (Mori-Yoshimura et al., 2018). In addition to the myopathy, behavioral disorders are common in the dystrophinopathy population (Hendriksen & Vles, 2008; Snow, Anderson, & Jakobson, 2013; Young et al., 2008). High prevalence rates of autism spectrum disorders (ASD; up to 21%), attention-deficit hyperactivity disorders (ADHD; up to 32%), anxiety and depression (up to 27%), oppositional defiant disorders (15%), and obsessive compulsive disorders (up to 5%) have been described in DMD compared to the general population (Banihani et al., 2015; Hendriksen & Vles, 2008; Hinton et al., 2009; Pane et al., 2012; Ricotti et al., 2016; Steele et al., 2008). Additionally, impaired psychosocial functioning has been reported in DMD (Thompson, Zeman, Fanurik, & Sirotkin-Roses, 1992; Uzark et al., 2012). Three previous BMD studies also investigated the presence of behavioral disorders, (Melo et al., 1995; Mori-Yoshimura et al., 2018; Young et al., 2008) but only one study found increased prevalence rates of attention problems (33%), ASD (8.3%), social problems (33%), and withdrawn behavior (33%) in BMD compared to the general population (Young et al., 2008). Current evidence suggests that these behavioral disorders are partially caused by neurobiological processes and relates them to impaired or altered expression of brain dystrophin isoforms. A relation between the absence of brain dystrophin isoforms and behavioral comorbidities such as ADHD and ASD has been described in patients with DMD (Banihani et al., 2015; Doorenweerd et al., 2017; Pane et al., 2012; Ricotti et al., 2016). There is no research available yet on possible relations between partially functional dystrophin production in the brain and behavioral comorbidities in BMD. Research on dystrophin-associated behavioral comorbidities remains limited for both disorders and no clear explanation exists for the different rates in comorbidities between DMD and BMD. It is suggested that the discrepancy might be explained by the difference in expression of brain dystrophin isoforms in DMD versus BMD, but this needs further investigation.

With the increased life expectancy nowadays due to changes in medical care (i.e., improved ventilation assistance), the dystrophinopathy population is confronted with long-lasting or new mental health issues that negatively influence their quality of life (Birnkrant et al., 2018). Recent standards of care for DMD emphasize that adequate and routine behavioral

and psychosocial assessment is crucial (Birnkrant et al., 2018), though current literature lacks an overview of available and adequate instruments. Nonetheless, the updated standards of care for DMD recommend four instruments: The Strengths and Difficulties Questionnaire (SDQ), the Patient Health Questionnaire (PHQ-9), the Generalized Anxiety disorder 7-item scale (GAD-7), and the Personal Adjustment and Role Skills Scale-III (PARS-III) (Birnkrant et al., 2018). However, disease-specific psychometric information is unknown for the SDQ, the PHQ-9, the GAD-7, and the suitability of the PARS-III for DMD care has been proven by only one previous study (Hendriksen et al., 2009).

Recently, improvements in screening and diagnostics of behavioral and psychosocial problems have received more attention in this high-risk aging population. Several studies have tried to improve the assessment of behavioral and psychosocial problems by using (1) the Diagnostic and Statistical Manual of Mental Disorders (DSM: Banihani et al., 2015), (2) more than one instrument, (e.g., informant-ratings in addition to structured clinical interviews; Banihani et al., 2015; Colombo et al., 2017; Ricotti et al., 2016), or (3) instruments with items relevant to dystrophinopathy (e.g., Muscular Dystrophy Child Health Index of Life with Disabilities questionnaire [MDCHILD] for children with DMD; Propp et al., 2018). However, most studies have used instruments with poor or unevaluated psychometric properties or instruments that contain items unsuited for patients with motor impairments. Therefore, the present systematic review aims to provide an overview of instruments used to assess behavioral and psychosocial functioning, and evaluates their psychometric properties and applicability for the dystrophinopathy population.

Methods

Search Strategy and Selection Criteria

This systematic review was conducted according to the Preferred Reporting Items for Systematic Reviews and Meta-Analyses statement (Liberati et al., 2009) and predefined PROSPERO protocol (CRD42017074518). Databases (EMBASE, Psyc.info, ERIC, PUBMED/Medline, and Cochrane) were searched from inception to July 2017 and updated to June 19, 2018. The search strategy is presented in [Supplementary Table 1](#). To be eligible for inclusion, studies had to (a) include males with dystrophinopathy (i.e., DMD or BMD), (b) examine the psychometric properties of behavioral and psychosocial instruments, (c) be available in English, Dutch, or German, and (4) be longitudinal or cross-sectional design studies. Case report studies ($n < 10$), nonpeer reviewed articles, abstracts from scientific

meetings, intervention studies or studies using an instrument that consisted of one subscale or one item of a questionnaire were excluded.

Two reviewers (D. H.: psychologist and J.L.: Doctor of Medicine, both having clinical experience with patients with DMD and BMD) independently screened titles and abstracts manually for potential inclusion, using the search terms of [Supplementary Table 1](#). Full-texts of articles were retrieved when inclusion could not be based on title and abstract. Cohen's kappa was used to measure the inter-rater reliability between the two independent reviewers (D.H. and J.L.). If no consensus was reached between the two reviewers, a third independent reviewer (J.H. psychologist with clinical experience in DMD and BMD) examined whether the article fulfilled the inclusion criteria. After selection based on title and abstract, one reviewer (D.H.) screened the full-text of the selected articles to assess whether the article could be included for final evaluation (i.e., data extraction). Doubtful articles were examined by a second independent reviewer (J.H.). Furthermore, reference lists of the selected full-text articles were screened to identify whether additional articles had to be included for review.

Data Collection and Extraction

One reviewer (D.H.) extracted data from included articles for final evaluation using a predefined form (PROSPERO: CRD42017074518). The following data were extracted: (1) authors and year of publication, (2) demographic characteristics (e.g., age), (3) in- and exclusion criteria if specified, (4) study design, (5) instruments used to assess behavioral or psychosocial functioning, (6) results of psychometric properties and applicability of instruments, and (7) study limitations and main conclusion.

Classification of Method of Assessment

Selected articles were classified according to methods of assessment ([Table 2](#)): clinical observation ratings, (semi) structured clinical interviews, informant-ratings, and self-ratings. During clinical observations, a clinician rates the extent of behavioral or psychosocial problems based on clinical judgement. Structured clinical interview instruments are designed to acquire both quantitative as well as qualitative information about the degree of behavioral or psychosocial functioning and are also completed by clinicians. Informant-rating instruments are questionnaires completed by a person who knows the patient well, for instance parents or caregivers. Self-rating instruments are completed by patients themselves.

Evaluation of Psychometric Properties and Applicability

The psychometric properties of the instruments were evaluated by D.H. using seven criteria (see [Table I](#))

from previous (systematic review) studies ([Hu & Bentler, 1999](#); [Smeets, Ponds, Verhey, & van Heugten, 2012](#); [Terwee et al., 2007](#); [Visser-Meily, Post, Riphagen, & Lindeman, 2004](#)). The applicability of the instruments were evaluated based on versions used in previous studies. The following aspects were assessed: availability (an easily obtained instrument), duration of assessment, and availability in different languages.

Results

The literature search identified 1,009 articles based on the inclusion criteria. All articles were accessible. After removing duplicates and excluding articles based on title or abstract, a total of 114 articles remained for full-text screening (see [Figure 1](#)). Cohen's kappa was calculated to determine the level of agreement between the two independent reviewers (D.H. and J.L.) on title and abstract screening. A moderate agreement was found between the two reviewers, $k = 0.72$. After full-text evaluation of the remaining 114 articles, a total of 53 articles were included for final evaluation. Reasons for exclusion were (1) conference abstracts, (2) no assessment of behavioral or psychosocial instruments, (3) small sample size <10 , (4) study population other than DMD and BMD, or no separate results available for DMD and BMD subpopulation, (5) no cross-sectional or longitudinal study design, and (6) language other than English, Dutch, or German. One additional article was extracted from the reference list of the 114 selected articles. This additional article fulfilled the eligibility criteria and was added to the 53 previous included articles, which resulted in a total of 54 included articles (see [Figure 1](#)).

Overview of Included Studies and Instruments

Among the 54 included articles ($N = 51$ DMD and $N = 3$ BMD), 61 different instruments ($n = 1$ clinical observation, $n = 12$ semistructured interviews, $n = 22$ informant-ratings, and $n = 26$ self-ratings) were used to assess behavioral and psychosocial functioning (see [Table II](#)).

The psychometric properties of these instruments were not evaluated in 33 ($N = 30$ DMD and $N = 3$ BMD) of the 54 articles and were excluded for final evaluation of psychometric properties (see [Table III](#) for excluded instruments). Due to the final selection, a total of 21 articles (all DMD) with 16 instruments ($n = 5$ informant-ratings, $n = 6$ self-ratings, and $n = 5$ instruments with informant-ratings and self-ratings) were reviewed. Patients ($N = 2,580$) included in these $N = 21$ studies were aged between 0.7 months and 44 years.

Table I. Criteria used to Evaluate Psychometric Properties

	Insufficient	Moderate	Sufficient
1. Internal consistency ^{a,b,d} Cronbach's alpha	< 0.70	0.70–0.80	> 0.80
2. Test-retest reliability ^{a,b} Spearman/Pearson correlation	< 0.70	0.70–0.80	> 0.80
3. Interrater reliability ^{a,b,d} Intra-class correlations (ICC), Pearson correlation or Kappa between raters	< 0.70	0.70–0.80	> 0.80
4. Convergent validity ^{a,b} Spearman/Pearson correlation	< 0.30	0.30–0.60	> 0.60
5. Construct validity ^{a,b} Principal component analysis Comparative fit index if available ^c Standardized root mean square residual ^c	Not confirms structure		Confirms structure > 0.95 < 0.9
6. Responsiveness ^{a,b,d}	Nonsignificant changes: $p > .05$, or effect size < 0.40		Significant changes: $p < .05$, or effect size > 0.40
7. Content validity ^d Aim of instrument is clarified Focus on dystrophinopathy Clarification of concepts of subscales Important items for population Interpretability of items (simple, no jargon or two questions at one time)	No No No No No		Yes Yes Yes Yes Yes

^aVisser-Meily et al. (2004).^bSmeets et al. (2012).^cHu and Bentler (1999).^dTerwee et al. (2007). **Figure 1.** Flowchart of the selected articles.

Table II. Overview of used Instruments in *Duchenne and Becker Muscular Dystrophy*

Method	Instruments	Recommended age for assessment ^b	Times used	Key feature	Examples of items	N	Key references (authors & year)
Clinical observations (Semi) structured clinical interviews	1. ADOS	1–23	2	Diagnosing ASD	“Does X ask for help?”	59	Banihani et al. (2015)
	2. RG	7–12	1	Psychiatric disorders in children	“Is your son anxious?”	47	Colombo et al. (2017)
	3. BSQ	2.5–3.5	1	Behavioural problems in young children	“Does X have temper tantrums-shouting?”	23	Fitzpatrick and Barry (1986)
	4. DAS	18+	1	Social adjustment	“Having difficulties in maintaining a friendship?”	33	Smith, Sibert, and Harper (1990)
	5. SADS-L	18+	1	Affective disorders and schizophrenia in adults	“Have you felt very good or too cheerful or high - not just your normal self?”	22	Eggers and Zatz (1998) ^a
	6. SIDP-R	All ages	1	Psychiatric assessment	NA	22	Melo et al. (1995) ^a
	7. Three wishes	6–12	1	Material goods, pets/animals, activities, interpersonal/family, future/goal, personal attribute, situational, situation/health-related, altruism	“If you would make three wishes, any three wishes in the world, what would they be?”	74	Nereo and Hinton (2003)
	8. Open - and structured questions	18–42	1	Bodily functions, social participation, quality of life	“Having sexual problems”	65	Rahbek et al. (2005)
	9. VABS	0–90	1	Adaptive behaviour, communication, socialization, daily living, motor skills	“How does the individual interact with others?”	20	Cyrulnik et al. (2008)
	10. KSADS	6–18	1	Affective disorders and schizophrenia in children and adolescents	“Have you ever felt sad, blue, down, or empty?”	10	Steele et al. (2008)
	11. Open- and structured	15+	1	Important topics in DMD	“What is it like to live with DMD?”	40	Abbott and Carpenter (2015)
	12. DAWBA	5–17	1	Generating psychiatric diagnosis based on DSM-IV and ICD-10	“In the last for weeks have there been times that X has been very sad, miserable, unhappy or tearful?”	47	Colombo et al. (2017)
	13. 3Di-sv	0–18	1	Communication and repetitive/stereotyped behaviour	“Avoids eye-contact”	130	Ricotti et al. (2016)
	14. RBQ (A)	7–13	1	Antisocial, neurotic, somatic behaviour	“Does she/he have eating difficulties?”	57	Leibowitz and Dubowitz (1981)
	15. RBQ (B)	7–13	2	Antisocial, neurotic, somatic behaviour	“Frequently fights with other children?”	57	Leibowitz and Dubowitz (1981)
	16. MCBC	9–15	1	Internalizing and externalizing behaviour	“Does not try new situations”	23	Fitzpatrick and Barry (1986)
	17. RBPC	5–18	1	Conduct disorders, socialized aggression, attention problems, anxiety-withdrawal, psychotic behaviour, motor tension	“Seeks attention, shows-off”	34	Thompson et al. (1992)
						44	Solden et al. (1999)

(continued)

Table II. (continued)

Method	Instruments	Recommended age for assessment ^b	Times used	Key feature	Examples of items	N	Key references (authors & year)
18. CBCL		1, 5–5 6–18	11	Anxious/depressed, somatic complaints, attention deficit/hyperactivity, autism spectrum, oppositional defiant, stress problems	-“Does he argues a lot?” -“Is he inattentive or easily distracted?”	74 26 50 50 10 22 16 159 130 47 24 22	Nereo and Hinton (2003) Hinton, De Vivo, Fee, Goldstein, and Stern (2004) Hinton, Nereo, Fee, and Cyrulnik (2006) Hinton, Fee, De Vivo, and Goldstein (2007) Steele et al. (2008) Donders and Taneja (2009) Donald, Mathema, Thomas, and Wilmschurst (2011) Fee and Hinton (2011) Ricotti et al. (2016) Colombo et al. (2017) Young et al. (2008) ^a Donders and Taneja (2009)
19. BRIEF		5–18	1	Executive behaviour index	“Troubles in remembering things even for a few minutes?”	287	Hendriksen et al. (2009)
20. PARS-III		5–18	1	Psychosocial adjustment	“Is he unable to decide for himself?”	85	Hinton et al. (2009)
21. SCQ		4–23	1	Screens for ASD	“Has he/she ever used odd phrases or said the same thing over and over in almost exactly the same way?”		
22. PedsQL GM		2–4 5–7 8–12 13–18 18–25 26+	9	Quality of life in children and adolescents	“Problems with feeling afraid or scared?”	35 52 44 203 56 63 99 107 98	Bray et al. (2010b) McDonald et al. (2010) Davis et al. (2010) Uzark et al. (2012) Hu et al. (2013) Lim et al. (2014) Wei, Speechley, Zou, and Campbell (2015) Messina et al. (2016) Wei, Speechley, Zou, and Campbell (2017)
23. PedsQL NMD		2–4 5–7 8–12 13–18	5	Quality of life in children & adolescents with NMD	“Has difficulties in telling the doctors and nurses how he feels?”	44 56 99 770 107 52	Davis et al. (2010) Hu et al. (2013) Wei et al. (2015) Landfeldt et al. (2016) Messina et al. (2016) McDonald et al. (2010)
24. PODCI		4–18	1	Quality of life in children and adolescents	“How happy has your child been with how he looks?”		

(continued)

Table II. (continued)

Method	Instruments	Recommended age for assessment ^b	Times used	Key feature	Examples of items	N	Key references (authors & year)
Self-ratings	25. CHQ-PF50	5–18	2	Quality of life in children	“How much of the time do you think your child felt like crying?”	27	Baiardini et al. (2011)
	26. CTRS-R: L	3–17	2	Oppositional, cognitive, social, psychosomatic problems, hyperactivity, anxious-shy, perfectionism	“Does not follow through on instructions and fails to finish schoolwork”	34	Bray, Bundy, Ryan, North, and Burns (2011)
	27. CPRS-R: L	3–17	2	Oppositional, cognitive, social, psychosomatic problems, hyperactivity, anxious-shy, perfectionism	“Cannot remain still”	103	Pane et al. (2012)
	28. PedsQL DMD	5–7 8–12 13–18	2	Quality of life in DMD	“Worries about his muscle problem”	59	Banihani et al. (2015)
	29. ABS	0–42 months	1	Social-emotional and adaptive behaviour	NA	24	Connolly et al. (2014)
	30. KIDSCREEN-52	8–18	2	Quality of life in children and adolescents	“Has your child felt lonely?”	40	Houwen-van Opstal et al. (2014)
	31. HUI	5+	1	Quality of life	“Happy and interested in life”	85	Zamani et al. (2016)
	32. CPRS: S	3–17	1	Attention, hyperactivity/impulsivity, learning problems, executive functioning, peer relations, aggression	“Restless or overactive”	770	Landfeldt et al. (2016)
	33. SCDC	3–19	1	Social communication disorders	“Not aware of other people's feelings”	130	Ricotti et al. (2016)
	34. QoML	4–18	1	Global quality of life	“Overall his life is . . .”	98	Wei et al. (2017)
	35. SDQ	2–18	1	Emotional, conduct problems, ADHD, peer relationship, prosocial behaviour	“Does he considers other people's feelings?”	47	Colombo et al. (2017)
	36. MMPI	18–80	1	Personality, behavioural difficulties and psychopathology	“I am easily awaked by noises”	44	Harper (1983)
	37. OSIQ	13–19	2	Psychosocial adjustment in adolescents	“I feel so very lonely”	15	Reid and Renwick (1994)
	38. LSIA	12–19	2	Life satisfaction in adolescents with DMD	“I have friends to talk to”	32	Reid and Renwick (2001)
	39. SWLS	NS	2	Life satisfaction in all ages	“The conditions of my life are excellent”	15	Reid and Renwick (1994)
	40. RCMAS	6–19.5	1		“I worry a lot of the time”	95	Simon, Resende, Simon, Zanoteli, and Reed (2011)
						15	Reid and Renwick (1994)
						16	Graham and Rose (2017)
						10	Steele et al. (2008)

(continued)

Table II. (continued)

Method	Instruments	Recommended age for assessment ^b	Times used	Key feature	Examples of items	N	Key references (authors & year)
41. CDI		7–18	1	Depression in children	“I hate myself”	10	Steele et al. (2008)
42. PEDSQL NMD		5–7 8–12 13–18	7	Quality of life in children & adolescents with NMD Anxiety problems in children and adolescents	“It is hard for me to tell the doctors and nurses how I feel”	44 99 770 107 321 56 278 44 203 50	Davis et al. (2010) Wei et al. (2015) Landfeldt et al. (2016) Messina et al. (2016) Otto et al. (2017) Hu et al. (2013) Landfeldt et al. (2018) Davis et al. (2010) Uzark et al. (2012) Bendixen, Senesac, Lort, and Vandenborne (2012)
43. PEDSQL GM		5–7 8–12 13–18 18–25 26+	9	Quality of life in children & adolescents	“I feel afraid or scared”	56 63 99 107 321 85 203 99 50	Hu et al. (2013) Lim et al. (2014) Wei et al. (2015) Messina et al. (2016) Otto et al. (2017) Wei et al. (2017) Uzark et al. (2012) Wei et al. (2015) Elsenbruch, Schmid, Lutz, Geers, and Schara (2013)
44. PEDSQL DMD		8–12 13–18 4–16	2	Quality of life in children & adolescents with DMD	“I worry about my muscle problem”	50	Elsenbruch et al. (2013)
45. DISABKIDS		NS	1	Quality of life in children with chronic diseases	NA	79 41 50	Pangalila et al. (2015b) Lue, Chen, and Lu (2017) Elsenbruch et al. (2013)
46. SF-36		NS	3	Quality of life in adults	“Have you felt calm and peaceful?”	50	Elsenbruch et al. (2013)
47. DIKJ		8–17	1	Depression in children and adolescents	NA	50	Elsenbruch et al. (2013)
48. BDI		13+	1	Depression in adults	“I don’t feel like a failure”	50	Elsenbruch et al. (2013)
49. KIDSCREEN-52		8–18	2	Quality of life in children and adolescents	“Have you felt under pressure?”	40 85	Houwen-van Opstal et al. (2014) Zamani et al. (2016)
50. WHOQOL-BREF		18+	3	Quality of life	“How well are you able to concentrate?”	80 79 41 80 321	Pangalila et al. (2015a) Pangalila et al. (2015b) Lue et al. (2017) Pangalila et al. (2015a) Otto et al. (2017)
51. HADS		16–65	1	Depression and anxiety	“I feel cheerful”	47	Colombo et al. (2017)
52. KIDSCREEN-10		8–18	1	Quality of life in children and adolescents	“Have you felt sad?”	47	Colombo et al. (2017)
53. YSR		11–18	1	Anxious/depressed, somatic complaints, attention deficit/hyperactivity, autism spectrum, oppositional defiant problems, stress problems	“I argue a lot” “I am easily distracted”	47	Colombo et al. (2017)
54. GAD-7		16+	1	Anxiety	“Feeling nervous, anxious or on edge”	16	Graham and Rose (2017)
55. PHQ-9		12+	1	Depression	“Feeling down, depressed or hopeless”	16	Graham and Rose (2017)

(continued)

Table II. (continued)

Method	Instruments	Recommended age for assessment ^b	Times used	Key feature	Examples of items	N	Key references (authors & year)
	56. AAQ	NS	1	Acceptance	"I avoid difficult thoughts and feelings- no matter the cost"	16	Graham and Rose (2017)
	57. B-IPC	NS	1	Illness perceptions	"DMD causes me distress"	16	Graham and Rose (2017)
	58. CFQ	18+	1	Cognitive diffusion	"My thoughts cause me distress or emotional pain"	16	Graham and Rose (2017)
	59. QoML	4-18	1	Global quality of life	"Overall my life is..."	85	Wei et al. (2017)
	60. Self-survey	20+	1	Neurodevelopmental, problematic and psychiatric diseases	"Experiencing problematic behaviour like suicide attempts and/or self-mutilation"	12.5	Mori-Yoshimura et al. (2018) ^a
	61. MDCCHILD	5-18	1	Health-related priorities	"How often were you unhappy or sad?"	19	Propp et al. (2018)

Note. AAQ = Acceptance and Action Questionnaire; ABS = Adaptive Behavior subtest; ADOS = Autism Diagnostic Observation Schedule; BDI = Beck Depression Inventory; B-IPC, Brief Illness perception Questionnaire; BRIEF = Behavior Rating Inventory of Executive functions; BSQ = behavior screening questionnaire; CBCL = Child Behavior Checklist; CDI = Children Depression Inventory; CFQ, Cognitive Fusion Questionnaire; CHQ-PF50 = Children's Health Questionnaire Parent Form 50; CPRS-R: L = Conners Parent Rating Scale-Revised: Long form; CPRS: S = Conners Parent Rating scale Short form; CTRS-R: L = Conners Teachers Rating Scale-Revised: Long form; DAS = Disability Assessment Schedule; DAWBA = Developmental and Well-being Assessment Diagnostic Interview; 3DI-sv = Diagnostic and Dimensional Interview- short version; DIKJ = Depressions Inventar für Kinder und Jugendliche; GAD-7 = Generalized Anxiety Disorder Questionnaire 7 items; HADS = Hospital Anxiety and Depression scale; HUI = Health Utilities Index Questionnaire; KIDSCREEN-52 = KIDSCREEN-52 items; KIDSCREEN-10 = KIDSCREEN-10 items; KSADS = The Kiddie Schedule for Affective disorders and Schizophrenia; LSIA = Life satisfaction Index for Adolescents with Duchenne muscular dystrophy; LS = Satisfaction with Life Scale; MCBC = Missouri Children's Behavior Checklist; MDCCHILD = Muscular Dystrophy Child Health Index of Life with Disabilities; MMPI = Minnesota Multiphasic Personality Inventory; OQIS = Offer Self-Image Questionnaire for Adolescents; PARS-III = Psychosocial Adjustment Rating Scale-III; PEDSQL DMD, Pediatric Quality of Life Inventory Duchenne muscular dystrophy module; PEDSQL GM = Pediatric Quality of Life Inventory generic score module; PEDSQL NMD = Pediatric Quality of Life Inventory Neuromuscular module; PODCI = Pediatric Outcomes Data Collection Instrument; PHQ-9 = Patient Health Questionnaire 9 item Scale; QoML = The Quality of My Life questionnaire; RBQ = Rutter Behavior Questionnaire (A) for parents; RBQ B = Rutter Behavior Questionnaire for teachers; RBPC = Revised Behavior Problem Checklist; RG = Rutter and Graham interviews; RCMAS = Revised Children's Manifest Anxiety Scale; SADS-L = Schedule for affective disorders and schizophrenia, Lifetime version; SCDC = Social Communication Disorder Checklist; SCQ = Social Communication Questionnaire; SDQ = Strengths and Difficulties Questionnaire; SF-36 = 36 item Short form Health Survey; SIDP-R = structured interview for DSM-III-R Personality; VABS = Vineland Adaptive Behavior Scales; WHOQOL-BREF = World Health Organization Quality of Life Brief version; YSR = Youth Self-Report; NMD = Neuromuscular disorders; DMD = Duchenne muscular dystrophy; ASD = Autism Spectrum Disorders; ADHD = Attention Deficit Hyperactivity Disorder; NA = Not available; NS = Not specified; N = sample size of study.

^aBMD studies.

^bRecommended age in years for assessment.

Table III. Excluded Instruments for Final Evaluation

Instruments	Clinical observations	(Semi) structured clinical interviews	Informant-ratings	Self-ratings
1. ADOS	x			
2. RG		x		
3. BSQ		x		
4. DAS		x		
5. SADS-L		x		
6. SIDP-R		x		
7. Three wishes		x		
8. Open- and structured questions on bodily functions, social participation and QOL		x		
9. VABS		x		
10. KSADS		x		
11. Open-and structured questions on important topics for DMD		x		
12. DAWBA		x		
13. 3Di-sv		x		
14. RBQ (A)			x	
15. RBQ (B)			x	
16. MCBC			x	
17. RBPC			x	
18. BRIEF			x	
19. SCQ			x	
20. CTRS-R: L			x	
21. HUI			x	
22. CPRS: S			x	
23. CPRS: L			x	
24. SCDC			x	
25. QoML			x	x
26. MMPI			x	
27. SWLS				x
28. RCMAS				x
29. CDI				x
30. DISABKIDS				x
31. DIKJ				x
32. BDI				x
33. HADS				x
34. AAQ				x
35. B-IPC				x
36. CFQ				x
37. Self-survey				x
38. MDCHILD				x

Note. AAQ = Acceptance and Action Questionnaire; ADOS = Autism Diagnostic Observation Schedule; BDI = Beck Depression Inventory; B-IPC = Brief Illness perception Questionnaire; BRIEF = Behavior Rating Inventory of Executive functions; BSQ = behavior screening questionnaire; CDI = Children Depression Inventory; CFQ = Cognitive Fusion Questionnaire; CPRS-R: L = Conners Parent Rating Scale-Revised: Long form; CPRS: S = Conners Parent Rating scale Short form; CTRS-R: L = Conners Teachers Rating Scale-Revised: Long form; DAS = Disability Assessment Schedule; DAWBA = Developmental and Well-being Assessment Diagnostic Interview; 3DI-sv = Diagnostic and Dimensional Interview- short version; DIKJ = Depressions Inventar für Kinder und Jugendliche; HADS = Hospital Anxiety and Depression scale; HUI = Health Utilities Index Questionnaire; KSADS = The Kiddie Schedule for Affective disorders and Schizophrenia; MCBC = Missouri Children's Behavior Checklist; MDCHILD = Muscular Dystrophy Child Health Index of Life with Dissabilities; MMPI = Minnesota Multiphasic Personality Inventory; QoML = The Quality of My Life questionnaire; RBQ = Rutter Behavior Questionnaire (A) for parents; RBQ B = Rutter Behavior Questionnaire for teachers; RBPC = Revised Behavior Problem Checklist; RG = Rutter and Graham interviews; RCMAS = Revised Children's Manifest Anxiety Scale; SADS-L = Schedule for affective disorders and schizophrenia, Lifetime version; SCDC = Social Communication Disorder Checklist; SCQ = Social Communication Questionnaire; SIDP-R = structured interview for DSM-III-R Personality; VABS = Vineland Adaptive Behavior Scales.

Psychometric Properties of Included Instruments

Table IV summarizes the psychometric properties of the 16 reviewed instruments. Values of **Table IV** are psychometric results of the total scale. Subscale information of the instruments is not included. The

different forms (i.e., informant-rating and self-rating) of the KIDSCREEN, the Pediatric Quality of Life inventory general module (PedsQL GM), the PedsQL neuromuscular module (PedsQL NMM), and the PedsQL Duchenne module (PedsQL DMD) were

Table IV. Psychometric Properties of Included Instruments

Method	Instrument	Internal consistency	Test- retest reliability	Inter-rater reliability	Convergent validity	Construct validity	Responsiveness
Informant-ratings	1. PARS-III	+	?	?	+	+	?
	2. CBCL	?	?	±/-	+/-	?	?
	3. PODCI	?	?	?	+	?	?
	4. CHQ-PF50	±	?	?	?	?	?
	5. ABS	?	?	?	?	?	-
Self-ratings	6. OSIQ	+	?	?	?	?	?
	7. LSIA	+	?	?	+	?	?
	8. YSR	?	?	±/-	?	?	?
	9. SDQ	?	?	?	+/-	?	?
	10. SF-36	?	?	?	±/-	?	?
	11. WHOQOL-BREF	?	?	?	±/-	?	?
Informant- and self-ratings	12. PEDSQL GM Informant form	+	?	-	+	?	+
	Self-rating form	+/-	?	-	?	?	+
	13. PEDSQL NMM Informant form	+	+/-	±/-	?	?	+
	Self-rating form	+	±/-	±/-	?	-	-
	14. PEDSQL DMD Informant form	+/-	?	-	?	?	?
	Self-rating form	±	?	-	?	?	?
	15. KIDSCREEN-52 Informant form	?	?	±/-	?	?	?
	Self-rating form	?	?	±/-	?	?	?
	16. KIDSCREEN-10 Informant form	±	?	?	?	?	?
	Self-rating form	±	?	?	?	?	?

Note. ABS = Adaptive Behavior subtest of Bayley; CBCL = Child Behavior Checklist; CHQ-PF50 = Children's Health Questionnaire Parent Form 50; KIDSCREEN-52 = KIDSCREEN 52 items; KIDSCREEN-10 = KIDSCREEN 10 items; LSIA = Life satisfaction Index for Adolescents with Duchenne muscular dystrophy; OSIQ = Offer Self-Image Questionnaire for Adolescents; PARS-III = Psychosocial Adjustment Rating Scale-III; PEDSQL DMD = Pediatric Quality of Life Inventory Duchenne Muscular Dystrophy Module; PEDSQL GM = Pediatric Quality of Life Inventory Generic Module; PEDSQL NMM = Pediatric Quality of Life Inventory Neuromuscular Module; PODCI = Pediatric Outcomes Data Collection Instrument; SDQ = Strengths and Difficulties Questionnaire; SF-36 = 36 item Short form Health Survey; WHOQOL-BREF = World Health Organization Quality of Life Brief version; YSR = Youth Self-Report; + = Sufficient; ± = moderate; - = insufficient; ? = Unknown/unclear.

assessed separately. As can be seen in Table IV, most psychometric information was available from the PARS-III, the PedsQL GM, and the PedsQL NMM. The PARS-III had good internal consistency (Cronbach's $\alpha = 0.91$), convergent validity ($r = -0.65$), and construct validity (factor analysis confirmed structure of instrument; comparative fit Index: 0.96, the standardized root mean square residual [SRMR]: 0.06). No information on inter-rater reliability, test-retest reliability, and responsiveness was available.

The PedsQL GM informant-rating form had good internal consistency (Cronbach's $\alpha = 0.83$) and the internal consistency of the PedsQL GM self-rating form was moderate to good (Cronbach's α ranged between 0.73 and 0.87). The inter-rater reliability of both forms was insufficient (informant-rating and self-rating ICC ranged between 0.20 and 0.64, and $r = 0.54$). Convergent validity was good for the informant-rating form ($r = 0.72$) but unknown for the

self-rating form. Responsiveness was good for both forms (informant-rating: $p < .001$, self-rating: $p = .04$). No information was available on test-retest reliability and construct validity of the informant-rating form and self-rating form. The PedsQL NMM informant-rating form and self-rating form had good internal consistency (Cronbach's α informant-rating form: 0.86–0.87 and self-rating form: 0.81–0.92). Test-retest reliability of the informant-rating form was sufficient ($r = 0.85$ –0.88) and moderate to insufficient ($r = 0.66$ –0.75) for the self-rating form. The inter-rater reliability of both forms ranged from moderate to insufficient (ICC ranged between 0.51 and 0.78). Convergent validity was good for the informant-rating form ($r = 0.60$ –0.71) and the self-rating form ($r = 0.65$ –0.67). No information was available on construct validity of the informant-rating form. Construct validity was insufficient for the self-rating form, since the structure of the form was not confirmed by principal component analysis.

Table V. Content Validity and Applicability of Included Instruments

Method	Instrument	Content validity					Applicability		
		Aim	Population	Concepts	Relevant items	Clear Items	Available by	Duration in min	Different languages
Informant-ratings	1. PARS-III	+	-	+	+	+	Author	5-10	Yes
	2. CBCL	+	-	+	±	±	Publisher	15-20	Yes
	3. PODCI	+	-	+	+	±	Website	20	Yes
	4. CHQ-PF50	+	-	+	+	±	Author	10-15	Yes
	5. ABS	+	-	+	?	?	Publisher	50 ^b	Yes
Self-ratings	6. OSIQ	+	-	+	±	±	Author	40-50	Yes
	7. LSIA	+	+	+	+	+	Author	7-10	Yes
	8. YSR	+	-	+	±	+	Publisher	15-20	Yes
	9. SDQ	+	-	+	±	±	Website	5-10	Yes
	10. SF-36	+	-	+	±	±	Website	9	Yes
Informant- and self-ratings	11. WHOQOL-BREF	+	-	+	+	±	Website	10-15	Yes
	12. PEDSQL GM								
	Informant form	+	-	+	+	+	Website	15-20	Yes
	Self-rating form	+	-	+	+	+	Website	15-20	Yes
	13. PEDSQL NMM								
	Informant form	+	- ^a	+	+	+	Website	10	Yes
	Self-rating form	+	- ^a	±	-	-	Website	10	Yes
	14. PEDSQL DMD								
	Informant form	+	+	+	+	+	Website	5	Yes
	Self-rating form	+	+	+	+	+	Website	5	Yes
	15. KIDSCREEN-52								
	Informant form	+	-	+	±	+	Website	15-20	Yes
	Self-rating form	+	-	+	±	+	Website	15-20	Yes
	16. KIDSCREEN-10								
	Informant form	+	-	+	+	+	Website	5	Yes
	Self-rating form	+	-	+	+	+	Website	5	Yes

Note. ABS = Adaptive Behavior subtest of Bayley; CBCL = Child Behavior Checklist; CHQ-PF50 = Children's Health Questionnaire Parent Form 50; KIDSCREEN-52 = KIDSCREEN 52 items; KIDSCREEN-10 = KIDSCREEN 10 items; LSIA = Life satisfaction Index for Adolescents with Duchenne muscular dystrophy; OSIQ = Offer Self-Image Questionnaire for Adolescents; PARS-III = Psychosocial Adjustment Rating Scale-III; PEDSQL DMD = Pediatric Quality of Life Inventory Duchenne muscular dystrophy module; PEDSQL GM = Pediatric Quality of Life Inventory Generic Module; PEDSQL NMM = Pediatric Quality of Life Inventory Neuromuscular Module; PODCI = Pediatric Outcomes Data Collection Instrument; SDQ = Strengths and Difficulties Questionnaire; SF-36 = 36 item Short form Health Survey; WHOQOL-BREF = World Health Organization Quality of Life Brief version; YSR = Youth Self-Report; + = Yes incorporated in the instrument, - = Not incorporated in the instrument; ± = doubtful; ? = Unknown/unclear; Aim = Aim of instrument is clarified; Population = Items of instrument are specified for dystrophinopathy; Concepts = Relevant concepts of the instruments are clarified and will be measured by separate subscales; Relevant items = Items of instrument reflect areas important for dystrophinopathy; Clear items = Items are simple and contain no jargon terms or two questions at the same time.

^aInstrument is specifically developed for patients with neuromuscular disorders not only for dystrophinopathy.

^bDuration of assessment is for total Bayley scale.

Responsiveness was good for the informant-rating form ($p = .005$) but insufficient for the self-rating form ($p = .77$).

Content Validity and Applicability of Included Instruments

Results of content validity and applicability of the 16 reviewed instruments are displayed in Table V. All instruments met criterion 1, signifying that instruments had specific measurement aims. Only two instruments were specifically developed for dystrophinopathy and met criterion 2: the Life Satisfaction Index for Adolescent with Duchenne muscular dystrophy (LSIA) and the PedsQL DMD. All instruments consisted of specified concepts, measured by separate subscales and items (criterion 3), except for the

PedsQL NMM self-rating form. Subscales of the PedsQL NMM self-rating form contain non-distinctive items, for instance, "it is hard for me to tell the doctors and nurses how I feel" and "it is hard for me to ask doctors and nurses questions." All instruments met criterion 4 and measured a degree of behavioral or psychosocial functioning relevant for the dystrophinopathy population, though items of two instruments (i.e., Child Behavior Checklist [CBCL] Offer Self-Image Questionnaire [OSIQ]) are less applicable in dystrophinopathy due to the progressive physical immobility. Less applicable items are for example "can't sit still, being restless" (CBCL-ADHD subscale), "runs away from home" (CBCL-rule breaking behavior subscale), or "I love the recent changes in my body" (OSIQ). Several instruments did not fulfill

criterion 5, since the items contain more than one question at the same time. In addition, items of the PedsQL NMM self-rating form for toddlers and young children (i.e., “hard to gain or lose weight”; do not have the equipment one needs) are not applicable or too difficult to understand. With regard to the applicability of the instruments, it was found that all instruments were available in English. Most instruments ($n = 9$) were freely available by website, four by author, and three by publisher. Administration time of nine instruments was <10 min and of seven instruments the time varied from 10 to 50 min.

Discussion

The purpose of this systematic review was to provide an overview of instruments used to assess behavioral and psychosocial functioning of patients with DMD and BMD, as well as to review the psychometric properties and applicability of these instruments. A total of 61 different instruments have been used in 51 DMD studies and 3 BMD studies. Our review showed that most previous studies provide limited psychometric information. The majority of instruments being used are informant-rating and self-rating instruments. The most common used ($N = 11$ studies) is the CBCL (Achenbach, 1991), but only two studies reported psychometric information on convergent validity (ranging from poor to moderate) and inter-rater reliability (ranging from moderate to good) (Colombo et al., 2017; Fee & Hinton, 2011). Additionally, the content validity of the CBCL is less sufficient for patients with motor impairments when questions involve normal motor abilities. This can result in underidentification of behavioral comorbidities due to lower scores in for instance hyperactive or oppositional, aggressive behavior (Cook & Beckman, 2006). Furthermore, the CBCL is developed for screening of various behavioral and emotional symptoms and is not intended to be a diagnostic instrument for establishing definite DSM-5 (American Psychiatric Association, 2013) diagnoses, such as ADHD (Goodman & Scott, 1999). To accurately diagnose ADHD clinicians should be able to differentiate symptoms of inattention, hyperactivity, and impulsivity. The symptom items of the CBCL-ADHD subscale have no conceptual link with the diagnostic criteria for ADHD (Goodman & Scott, 1999). Other instruments, for instance, the Conners Parent Rating Scale-Revised (CPRS-R) or the SDQ differentiate and ascertain ADHD symptoms more accurately. Since the items of the CPRS-R and SDQ subscales are selected on the basis of nosologically concepts (i.e., DSM criteria and International Statistical Classification of diseases and related Health Problems: ICD-10) as well as on factor analysis (Gianarris, Golden, & Greene,

2001; Goodman & Scott, 1999; Hall et al., 2019). This likely explains that Colombo et al. (2017) recently found an ADHD comorbidity of 0% in their DMD sample based on CBCL screening, while Banihani et al. (2015) found a prevalence rate as high as 32% for ADHD using CPRS-R for ADHD. These data confirm that the prevalence rates of behavioral comorbidities in DMD as previously reported largely depend on the instruments that were used and should, therefore, be considered with caution. The majority of previous studies used instruments with unevaluated or poor psychometric properties, or instruments that are not developed to differentiate behavioral symptoms.

Psychometric Properties of Included Instruments

For the current systematic review disease specific psychometric information was available for 21 DMD studies with 16 instruments being reviewed, including both informant-ratings and self-ratings. The 33 excluded studies used significantly different instruments including clinical observations and semistructured interviews, in addition to informant-rating and self-rating instruments. Thereby, this review only describes psychometric properties of informant-rating and self-rating instruments for DMD. With regard to the psychometric properties of the included instruments, more in depth evaluation was found for the PARS-III, the PedsQL GM, and the PedsQL NMM. The PARS-III proves to be an adequate instrument and is therefore recommended in the standards of care for DMD (Birnkranz et al., 2018). However, additional validation by replication of reported psychometric results is recommended as well as data on inter-rater reliability, test-retest reliability, and responsiveness. The PedsQL GM informant-rating and self-rating form have relatively good properties, both having only poor inter-rater reliability. Data on PedsQL NMM informant-rating form varies in previous studies and its inter-rater reliability is low. Moderate to poor psychometric results are noted for the PedsQL NMM self-rating form, suggesting that it is no valid instrument for DMD. The inter-rater reliability of both PedsQL modules (GM and NMM) are low, reflecting a discrepancy between patients and their parents/caregivers. This is in line with previous studies showing that parents/caregivers overestimate the magnitude of problems due to their own concerns and perceptions of the illness (Bray, Bundy, Ryan, North, & Everett, 2010b; Solden, Guilda, Antao, Street, & Sibert, 1999). This could explain the notable difference between behavioral and psychosocial problems reported by patients and caregivers (Davis et al., 2010; Houwen-van Opstal, Jansen, van Alfen, & de Groot, 2014; Lim, Vellozo, & Bendixen, 2014). Additional validation on psychometric properties of both PedsQL modules and

their separate forms is necessary, before using them for clinical or research trials. With respect to the three (SDQ, GAD-7, and PHQ-9) remaining recommended instruments of the recently published standards of care for DMD (Birnkrant et al., 2018), two instruments (GAD-7 and PHQ-9) have no disease specific psychometric information. However, previous studies reported that the GAD-7 is a valid instrument for screening of generalized anxiety disorders as well as for panic, social anxiety, and post-traumatic stress disorders (Kroenke, Spitzer, Williams, Monahan, & Löwe, 2007; Spitzer et al., 2006). Nevertheless, the GAD-7 provides only a probable diagnosis and further evaluation is necessary to diagnose anxiety. Furthermore, the psychometric properties of the PHQ-9 have been evaluated by multiple clinical studies. This instrument seems favorable in screening for depression in primary and mental health care and is equal or superior to other depression measures (Kroenke, Spitzer, Williams, & Löwe, 2010). However, previous studies on the GAD-7 and the PHQ-9 evaluated the reliability and validity of these instruments in adult patients and the usefulness for DMD should be assessed accordingly. The third instrument, the SDQ, poses limited psychometric information and Colombo et al. (2017) showed that the SDQ has a moderate to good validity in $N=47$ males with DMD. The SDQ has been extensively evaluated in children and adolescents with or without a chronic illness and it is believed that it is a reliable and valid screener for psychiatric comorbidities (Bourdon, Goodman, Rae, Simpson, & Koretz, 2005; Goodman 2001; Hysing, Elgen, Gillberg, Lie, & Lundervold, 2007; Hysing, Elgen, Gillberg, & Lundervold, 2009; Klasen, Woerner, Rothenberger, & Goodman, 2003; Smedje, Broman, Hetta, & Von Knorring, 1999). In addition, a higher specificity of the SDQ informant-rating (0.85) has been noted compared to the CBCL (0.72), meaning that the SDQ will result in less overestimation of true behavioral comorbidity. Overall, the SDQ should be considered as a valid and robust instrument for future research and clinical settings, though care should be taken when using the SDQ algorithm to screen for ADHD comorbidity (Hall et al., 2019). Further evaluation of the SDQ psychometric properties for the dystrophinopathy population is recommended.

Content Validity and Applicability of Included Instruments

The current review systematically evaluated content validity and applicability of the 16 included instruments. Only the LSIA and PedsQL DMD fulfilled the five content validity criteria and may thus be suitable for DMD care. However, our psychometric results revealed sufficient and reliable results for the LSIA, but insufficient

results for the PedsQL DMD. Additional research on the reliability of both instruments is recommended. Furthermore, of the 16 included instruments, the majority had items that were (1) limited or not applicable to the dystrophinopathy population, (2) or too difficult to answer for patients or parents/caregivers. In addition, the majority of the included instruments were relatively easily accessible by website and could be filled in quickly (<10 min).

Limitations

This review was designed to capture all behavioral and psychosocial instruments currently used in cross-sectional and longitudinal studies. As a consequence, potentially good instruments used in studies with other designs (e.g., case-report or conference abstracts) have been excluded. Furthermore, we excluded studies with possible adequate instruments when no separate information was available for the dystrophinopathy population. Excluded studies, for instance, used the Autism Diagnostic Interview-Revised (Hinton et al., 2009), Experience Sampling Method (Bray, Bundy, Ryan, & North, 2010a), California Psychological Inventory (Fowler et al., 1997), Personal Assessment Inventory-Depression (Fowler et al., 1997), Child Health Questionnaire-Child form-87 (Wilson et al., 2006), Multidimensional Scale of Perceived Social Support (Wilson et al. 2006), Quality of Life Profile Questionnaire (Dany et al., 2015, 2017), and Strips of Life with Emoticons Questionnaire (Orcesi et al., 2014) in patients with other neuromuscular disorders (e.g., spinal muscular atrophy, myopathy, and giant axonal neuropathy). An important strength of our study is the use of the psychometric property criteria. However, at the same time this approach limits the finding of adequate instruments. Previous studies, for instance, used less stringent criteria to assess inter-rater reliability between the informant-rating and self-rating form of the PedsQL GM and PedsQL NMM (Davis et al., 2010; Uzark et al., 2012). Despite the less stringent criteria, the outcomes of Davis et al. (2010) and Uzark et al. (2012) were in line with the findings of the current review concerning the poor inter-reliability between the informant-rating and self-rating form of both PedsQL modules. Furthermore, for final evaluation, we excluded studies that reported no information on psychometric properties, content validity, and applicability for the dystrophinopathy population. Therefore, all BMD studies were excluded and generalizations of our results regarding the entire dystrophinopathy population should be made with caution. Additionally, the MDCHILD could not be included in the current review, since no information was available on psychometric properties for the dystrophinopathy population (Propp et al., 2018).

Clinical Recommendations

The current review provides no gold standard for methods of assessment, but based on the findings the PARS-III, LSIA, and PedsQL GM are potentially valid instruments for screening of psychosocial problems in DMD. Nonetheless, when using the PedsQL GM we recommend to administer both the informant-rating and self-rating form, to limit the error variance caused by disagreements between patients and parents/caregivers perspectives.

Appropriate instruments for screening of behavioral problems in DMD may be the SDQ for pediatric patients and the GAD-7 and PHQ-9 for adult patients. Additional research on psychometric properties of these instruments for DMD as well for BMD is crucial and definite outcomes of these screening instruments should be considered with caution.

For definite diagnostics, we recommend to use the gold standard assessment method for behavioral disorders. This is referred to as the multimethod, multi-source, multisetting assessment, and implies using different assessment methods (e.g., behavioral observations, structured clinical interviews, informant-ratings, and self-ratings), different sources (e.g., patients, parents/caregivers, teachers, and clinicians), and different settings (e.g. at home, school, and the clinic) to get a comprehensive representation of the child's or adolescent's behavioral, emotional, and psychosocial functioning (Whitcomb & Merrel, 2013). We believe that this method is particularly important for the ageing high-risk dystrophinopathy population, whereof 20% of patients have more than one comorbid behavioral disorder (Banihani et al., 2015; Hendriksen & Vles, 2008; Hinton et al., 2009; Pane et al., 2012; Ricotti et al., 2016; Steele et al., 2008; Thompson et al., 1992; Uzark et al., 2012). This multimethod-, source-, setting assessment reduces error variance, resulting in less underidentification of behavioral comorbidities in this high-risk population. Each neuromuscular team should include a mental health professional (psychologist or psychiatrist) with experience in neurodevelopmental, behavioral, and medical conditions, who routinely screens a patients' behavioral and psychosocial functioning every 2–3 years and applies this multimethod-, source, setting assessment when necessary (Birnkrant et al., 2018). Adequate and early detection of behavioral comorbidities can greatly facilitate targeted treatment. We know, for instance, that in the general ADHD population, underdiagnosed ADHD problems put patients at higher risk for educational underachievement, behavioral comorbidities like depression, impairments in social relations, and a reduced quality of life, that can extend throughout adulthood (Hoza, 2007; Pine, Cohen, Gurley, Brook, & Ma, 1998; Woodward & Fergusson, 2001). According to our review, only one previous DMD study applied this multimethod-, source-, setting

assessment using the DSM-IV criteria together with two screening instruments, the Conners Parent and Conners Teacher Rating Scales, to diagnose ADHD comorbidity in their DMD sample (Banihani et al., 2015). Two additional studies administered this method partly and evaluated behavioral problems using structured interviews such as the DAWBA, ADOS, and 3Di-Sv in addition to multiple informant-rating and self-rating instruments (Colombo et al., 2017; Ricotti et al., 2016).

Conclusion

The present review describes a wide variety of instruments used to assess behavioral and psychosocial functioning in the dystrophinopathy population. We argue that the interpretation of behavioral disorders and psychosocial problems as reported in previous studies using these instruments should be taken with caution. Our review shows that the PARS-III, PedsQL GM, and LSIA are potentially valid instruments for psychosocial screening. The SDQ, GAD-7, and PHQ-9 may be appropriate instruments for behavioral screening. Other screening instruments that lack psychometric data for the dystrophinopathy population may be appropriate as well, but their outcomes should be considered with caution. Additional psychometric data on instruments for screening of behavioral and psychosocial problems is necessary. Since the ageing dystrophinopathy population is confronted with long-lasting or new mental health issues that negatively influence their quality of life. A mental health professional should routinely screen a patient's behavioral and psychosocial functioning, preferably every 2–3 years and particularly during transitioning (e.g., childhood to adolescence and adolescence to adulthood) (Birnkrant et al., 2018). For definite diagnostics, we believe that a multimethod-, source-, setting assessment is the most appropriate method of assessment in this unique population. Given the complexity of behavioral comorbidities, clinicians and researchers should never diagnose conditions based on the result of one screening instrument.

Supplementary Data

Supplementary data can be found at: <https://academic.oup.com/jpepsy>.

Acknowledgment

The authors would like to thank Spieren voor Spieren and Duchenne Parent Project NL for their funding. Special thanks to mw. I. Gijselhart for her help in creating the searches.

Funding

This work was supported by Spieren voor Spieren Foundation (grant number SvS15-) and Duchenne Parent

Project NL. All reimbursements were received by Kempenhaeghe, Heeze, The Netherlands. No personal financial benefits were received.

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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