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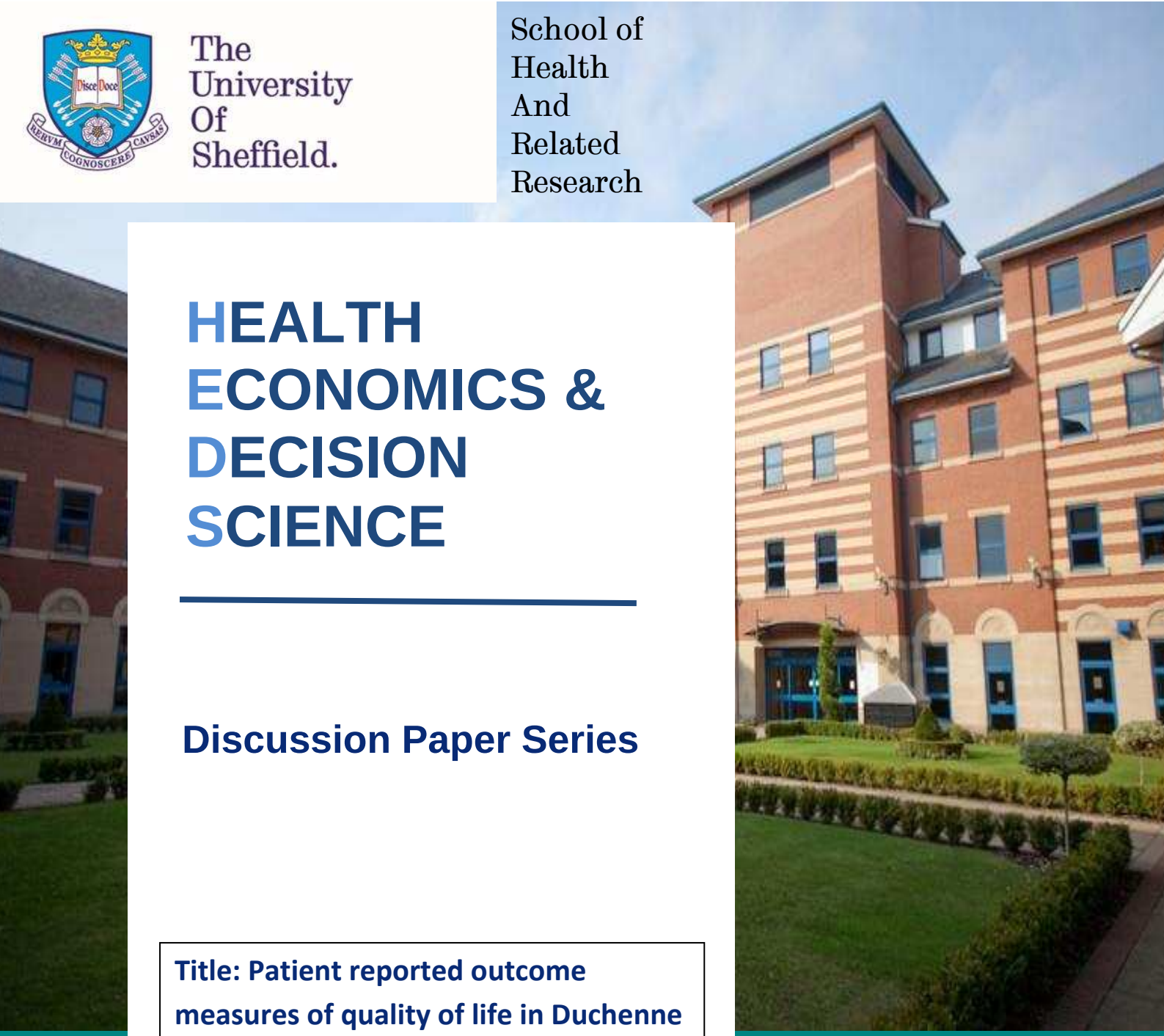

HEALTH ECONOMICS & DECISION SCIENCE

Discussion Paper Series

Title: Patient reported outcome measures of quality of life in Duchenne muscular dystrophy (DMD): a systematic review of content and structural validity using COSMIN

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This series is intended to promote discussion and to provide information about work in progress. The views expressed in this series are those of the authors. Comments are welcome, and should be sent to the corresponding author.

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Abbreviations

AUQEI	Autoquestionnaire Qualité de vie Enfant Imagé
BASC	Behavior Assessment System for Children
BDI	Beck Depression Inventory
CALI	Child Activity Limitations Interview
CAPE	Children's Assessment of Participation and Enjoyment
CHQ-PF50	Child Health Questionnaire - Parent Form 50
CMQM	Comprehensive Model of QoL in Muscular Dystrophy
COSMIN	COnsensus- based Standards for the selection of health Measurement INstruments
DCGM-37	DISABKIDS generic module
DIKJ	Depressions-Inventar für Kinder und Jugendliche
DMD	Duchenne muscular dystrophy
DUC-25	Dutch Children AZL/TNO Questionnaire Quality of Life Short Form
EQ-5D(-3L)	Euroqol instrument, Euroqol-5 dimension (3 level)
EQ-5D-Y	Euroqol instrument, Euroqol-5 dimension (youth version)
FSS	Fatigue Severity Scale
GAD-7	Generalized Anxiety Disorder Scale 7-item
GRADE	Grading of Recommendations, Assessment, Development and Evaluations
HADS	Hospital Anxiety and Depression Scale
HUI	Health Utilities Index
INQOL	Individualized Neuromuscular Quality of Life Questionnaire
LSIA	Life Satisfaction Index for Adolescents
MDCHILD	Muscular Dystrophy Child Health Index of Life with Disabilities
Neuro-QoL	Neurological Disorders Quality of Life Questionnaire
NICE	National Institute for Health and Care Excellence
OSIQ	Offer Self-Image Questionnaire
PBM	Preference based measure
PedsQL 3.0 DMD	Pediatric Quality of Life Inventory (PedsQL) 3.0 DMD module
PedsQL 3.0 MFS	PedsQL 3.0 Multidimensional fatigue scale
PedsQL 3.0 NMM	PedsQL 3.0 Neuromuscular module
PedsQL 4.0 GCS	Pediatric Quality of Life Inventory (PedsQL) 4.0 Generic Core Scales
PedsQL 4.0 SF-15	PedsQL 4.0 Generic Short-form
PHQ-9	Patient Health Questionnaire Depression Scale
PODCI	Pediatric Outcomes Data Collection Instrument
PROM	Patient reported outcome measure
PROSPERO	International Prospective Register of Systematic Reviews
PSQI	Pittsburgh Sleep Quality Index
QoL	Quality of life
Rasch PCM	Rasch Partial-credit model
SDQ	Strengths and Difficulties Questionnaire
SF-36	36-item Short-Form Health Survey
SOLE	Strips of Life with Emoticons Questionnaire

SWLS	Satisfaction with Life Scale
TAAQoL	TNO-AZL Questionnaire for Adult's Health-Related Quality of Life
TACQOL	TNO-AZL Questionnaire for Children's Health-Related Quality of Life
WHOQOL-BREF	World Health Organisation Quality of Life Scale-Brief Version

Executive summary

Duchenne muscular dystrophy (DMD) is an inherited neuromuscular disorder that predominantly affects boys and men. There is no known cure, so current clinical efforts are focused on improving the health-related quality of life (QoL) of people with DMD. Of the validated methods for assessing QoL, patient-reported outcome measures (PROMs) are the most common. In a field of many options, the choice of which PROM to use should be based predominantly on their validity and reliability for the construct, population, and context of use of interest. In the current report we critically reviewed the content and structural validity of PROMs used to assess QoL in people with DMD, using robust, updated COnsensus-based Standards for the selection of health Measurement INstruments (COSMIN) guidelines.

In this review, we defined QoL as a multidimensional construct involving physical, psychological, and social components. We conducted a systematic search of the published literature for self-reported, multi-item PROMs assessing at least one aspect of QoL in a sample of at least 75% boys and men with DMD. We further refined these results for studies with evidence of content and structural validity, including development studies. The resulting PROMs and related studies were systematically rated, in terms of methodological quality, and evidence for content and structural validity, as per the latest COSMIN procedures.

From an initial 1,752 records, and 5 additional records identified through citation tracking, 60 published primary research articles were identified that had used a PROM to assess at least one aspect of QoL in people with DMD, of which 5 articles presented evidence on content or structural validity. A further 36 articles were identified through Google Scholar searching and citation tracking presenting content validity information on the development of the PROMs, resulting in a final selection of 41 articles for review. From the articles identified, 40 PROMs were extracted, of which 26 were taken forward for COSMIN assessment.

The results of the assessment for content validity revealed a modal COSMIN quality rating of inadequate, primarily due to the PROM development study not being performed in a sample of patients representing the target population (of people with DMD). The second most common rating was one of doubtful, due to at least some unclear details/suspected problems within the qualitative methods used. Only the KIDSCREEN family of measures received an adequate rating for concept elicitation and PROM design. Only two published articles had independently assessed the content

validity of the QoL PROMs (LSIA, PedsQL 3.0 NMM) in samples of people with DMD, and both received ratings of doubtful due to at least some unclear details/suspected problems with the qualitative methods used. In terms of the evidence on content validity assessed, the KIDSCREEN measures and the LSIA were the only PROMs to receive satisfactory results for all three dimensions of content validity. These reflect relevance (whether items are relevant for the construct, target, population, and context of use of interest); comprehensiveness (the extent to which all key aspects of the construct of interest are covered); and comprehensibility (the understanding of items and response options by the population of interest).

Two studies had investigated the structural validity of the included PROMs (PedsQL 4.0 GCS, PedsQL 3.0 NMM) in people with DMD, of which one received a very good COSMIN quality rating for its methodological content. Nevertheless, an assessment of the evidence for structural validity revealed a rating of indeterminate for the PedsQL 4.0 GCS, as key details of the results from the Rasch model were not reported, and a rating of unsatisfactory for the PedsQL 3.0 NMM, due to psychometric criteria for good measurement properties not being met.

The results of this review suggest that evidence on the content and structural validity of PROMs assessing QoL in people with DMD is sparse, and further research is needed. In the absence of further evidence, we recommend that the KIDSCREEN is used to assess QoL in children and adolescents with DMD. It is difficult to recommend an adult measure, as insufficiencies are evident in content and/or structural validity, but in terms of precedent, the PedsQL is widely used, which has a young adult and adult variant. Furthermore, these two PROMs can be used in cost effectiveness analyses via mapping algorithms. Limitations of this review include the potential harshness of the worst score counts COSMIN system of assessment of methodological quality; the restriction to two, albeit the most important, measurement properties; and the potential restrictive nature of the inclusion criteria, which could be broadened to consider related neuromuscular disorders or mixed samples, if deemed theoretically appropriate.

1 Introduction

Duchenne muscular dystrophy (DMD) is an inherited neuromuscular disorder that predominantly affects boys and men. It has an estimated incidence of 1:3800 to 1:6300 in live births.[1] The disease causes progressive muscle weakness due to an absence of the dystrophin protein, which functions to help keep muscle cells intact. Diagnostic symptoms and functional impairment are evident from as early as two years old and average life expectancy of people with DMD is approximately 25 years,[2] although increasingly people with DMD are surviving into their fourth and even fifth decades.[3] The disease progresses through four recognised clinical stages characterised by increased muscle weakness, impaired ambulation and motor functioning, and cardiovascular and respiratory problems.[4] There is no known cure for the disease. Current clinical efforts are thus focused on improving the health-related quality of life (QoL) of people with DMD, and health interventions are necessarily evaluated for their cost effectiveness against this objective.

In order to attempt to measure QoL in people with DMD a number of condition-specific and generic questionnaires are used. For NICE, the institution responsible for making decisions on the funding of NHS health interventions, there is a stated preference that health-related QoL data comes from the EQ-5D.[5] The EQ-5D is a generic, preference-based measure of health-related QoL with 5 dimensions covering mobility, self-care, usual activities, pain/discomfort, and anxiety and depression. In the case of DMD, however, there are a number of concerns that measures like the EQ-5D are insufficient to adequately assess QoL in this population, based on the aspects of QoL that matter to people living with DMD.[6] For example, DMD is recognised to have an impact on aspects of daily life, such as participation, friendships, independence and dignity which may not be fully captured by generic measures, such as the EQ-5D (or its equivalent for children, the EQ-5D-Y).

In order to satisfy NICE requirements, in cases where the EQ-5D is considered insufficient as a measure of health-related QoL for a particular population, and thus other measures may be more appropriate, evidence must be provided. Such evidence largely centres on the demonstrable validity of a measure, including its content validity and/or psychometric performance in the population of interest. Where it can be evidenced that the EQ-5D is inappropriate as a measure of health-related QoL, alternative preference-based measures, with superior validity, can be used to generate utility values, including condition-specific preference-based measures.

Given that a number of generic and condition-specific questionnaires are available for use in attempting to assess QoL in people with DMD, evidence is desperately needed on the relative validity and psychometric performance of these instruments, when it comes to assessing QoL in DMD. While we are aware of a number of reviews exploring QoL and associated measures in DMD, with some providing very basic information on their psychometric properties,[6,7] no reviews to date have appropriately evaluated the content validity of available measures when it comes to assessing QoL in DMD. This is a striking omission; content validity has been defined by the COnsensus-based Standards for the selection of health Measurement INstruments (COSMIN) group as the most important property of a patient reported outcome measure (PROM).[8] Furthermore, prior reviews on QoL in neuromuscular disorders have either not referred to, or used an outdated version of, COSMIN guidance, which is considered a rigorous approach to the systematic assessment(s) of the validity and reliability of PROMs. Updated and expanded COSMIN guidance and documentation for the evaluation of PROMs was published earlier this year, and, as a consequence of its importance in determining measure selection, this included a dedicated manual on assessing the content validity of PROMs.[9]

Content validity refers to the extent that the content of a PROM adequately reflects the target construct that is being measured.[10] It can be meaningfully subdivided into the relevance, comprehensiveness, and comprehensibility of a PROM, for assessing the construct of interest in a target population and context.[9] Here, relevance of a PROM refers to whether the items are relevant for the construct, target population, and context of use of interest; the response options and recall period of a PROM should also be appropriate and relevant. Comprehensiveness is used to describe the extent to which all key aspects of the construct of interest are covered in the PROM. Finally, comprehensibility pertains to understanding of the items and response options by the population of interest.[9]

A thorough assessment of a PROM's content validity should crucially include studies presenting information on content validity in the population of interest, but also consider the initial PROM development paper(s) and the content of the PROM itself.[9] Content validity should form the first step of the assessment of the validity of a PROM, as it is integral to that PROM's usefulness in doing the job it was designed to do, and influences all other measurement properties.[11] For example, a psychometrically responsive and internally consistent instrument is of little use if it is not measuring what it is intended to measure in the first place.

According to COSMIN guidance, the second most important form of the validity assessment of a PROM is structural validity.[11] Structural validity describes the extent that scores derived from a measure adequately reflect the dimensionality of the construct being measured.[12] Quality of life is usually defined, and thus measured, as a multidimensional construct. Therefore, PROMs that feature multiple dimensions of quality of life should be dutifully assessed to check they accurately represent the multidimensional structure of quality of life in the population of interest. If PROMs are designed to target a single dimension of quality of life, assessments should be undertaken to empirically demonstrate their unidimensional nature in the target population. If such assessments are not undertaken, subsequent interpretation of the data (e.g. through generating dimensional scores) may be inaccurate.

This systematic review has been designed to evaluate the content and structural of QoL measures used in people with DMD using updated COSMIN guidance.[9,13] A similar approach has recently been undertaken and published by members of the COSMIN group when evaluating PROMs for physical functioning used in people with low back pain.[11] It forms part of an ongoing project funded by Duchenne UK on producing a preference-based measure (PBM) for people with DMD as part of the Project HERCULES initiative.

For the purposes of this review, we define QoL as a multidimensional construct involving physical (e.g., pain, fatigue), psychological (e.g., mood, self-efficacy), and social (e.g., participation, stigma) components, based on the Comprehensive Model of QoL in Muscular Dystrophy (CMQM).[6] However, here we operationalise QoL as a subjective construct and do not include purely functional performance or assessment scales that may impact on QoL. In this review, we consider multi-item PROMs that assess *at least* one aspect of QoL in people with DMD.

2 Objectives

- To identify PROMs that are used in people with DMD to measure QoL
- To assess the content validity of PROMs that are used in people with DMD
- To assess the structural validity of PROMs that are used in people with DMD
- To synthesise the evidence and make a recommendation on the use of available PROMs to assess QoL in people with DMD

3 Methodology

3.1 Protocol registration

The protocol was registered with PROSPERO, an international database of prospectively registered systematic reviews in health and social care, welfare, public health, education, crime, justice and international development, where there is a health related outcome. The protocol is accessible at: https://www.crd.york.ac.uk/PROSPERO/display_record.php?RecordID=93062

3.2 Search strategy and information sources

3.2.1 Searches

A ScHARR information specialist was consulted in developing the appropriate search strategy and was responsible for conducting the main database searches.

Search terms in this review included:

I) Duchenne muscular dystrophy (and derivatives);

II) A robust search filter developed by the PROM Group at the University of Oxford to identify PROMs;

III) Patient-reported outcome measures known to be used with people with DMD based on an earlier rapid review of the literature; and

IV) A robust search filter by the COSMIN Group for identifying studies on measurement properties, as recommended by the COSMIN Group.[14]

A two-stage search strategy was used, where in the first stage, the search terms (I) AND ((II) OR (III)) were combined to identify articles using PROMs to measure QoL in DMD. In the second stage, search terms (I) AND (III) AND (IV) were combined with the names of all PROMs identified in Stage one of the search to identify articles reporting on the measurement properties of these instruments in DMD. No restrictions on time or language were applied to the search strategy. The two-stage search strategy has the advantage of allowing us to identify which PROMs have been used in published, peer-reviewed articles on DMD, in the absence of any evidence of content or structural validity for their use in this population.

3.2.2 Electronic databases

The electronic databases searched for the systematic review are outlined in Table 1. All databases were searched from inception.

Table 1. Electronic databases for the primary searches.

Database	Platform	Span of search	Date searched (Stage 1)	Date searched (Stage 2)
EMBASE	Ovid SP	From 1974	April 2018	September 2018
MEDLINE	Ovid SP	From 1946	April 2018	September 2018
CINAHL	EBSCOhost	From 1981	April 2018	September 2018
PsycINFO	Ovid SP	From 1967	April 2018	September 2018
Cochrane library	Wiley	From 1989	April 2018	September 2018

3.2.3 Search strings

3.2.3.1 Stage 1 search strategy:

Database: Ovid MEDLINE
Platform: Ovid SP

Table 2. MEDLINE search string.

	Query	Results
#1	Muscular Dystrophy, Duchenne/	4700
#2	duchenne*.mp.	11822
#3	#1 or #2	11822
#4	(HR-PRO or HRPRO or HtaRQL or HRQoL or QL or QoL).ti,ab. or quality of life.mp. or (health index* or health indices or health profile*).ti,ab. or health status.mp. or ((patient or self or child or parent or carer or proxy) adj (appraisal* or appraised or report or reported or reporting or rated or rating* or based or assessed or assessment*)).ti,ab. or ((disability or function or functional or functions or subjective or utility or utilities or wellbeing or well being) adj2 (index or indices or instrument or instruments or measure or measures or questionnaire* or profile or profiles or scale or scales or score or scores or status or survey or surveys)).ti,ab.	649346
#5	'Pediatric Quality of Life Inventory'.mp. (	884
#6	PedsQL.mp.	1094
#7	(SF-36 or EQ-5D*).mp.	24322
#8	"World Health Organization Quality of Life".mp.	1373
#9	(KIDSCREEN or Pittsburgh Sleep Quality questionnaire or PSQI).mp. [mp=title, abstract, original title, name of substance word, subject heading word, floating sub-heading word, keyword heading word, protocol supplementary concept word, rare disease supplementary concept word, unique identifier, synonyms]	2710
#10	"Childrens Assessment of Participation and Enjoyment".mp.	96
#11	(CAPE or "Child Health Questionnaire" or CHQ* or Health Utilities Index Questionnaire or HUI*).mp.11	17397

#12	(Fatigue Severity Scale or FSS or "Hospital Anxiety and Depression Scale" or HADS or COPE Inventory or "Quality of Life in Neuromuscular Disease").mp.	10638
#13	(QoL-NMD DISABKIDS or "Depressionsiventar fur kinder und Jugendliche" or DIKJ or Beck Depression Inventory or BDI or CARE-NMD or State-Trait Anxiety Inventory or STAI or "Life Satisfaction Index for Adolescents" or LSI-A or Quality of Life Evaluation Scale or AUQUEI or Activity Limitations Questionnaire or ACTIVLIM).mp.	17368
#14	(sf12 or sf 12 or short form 12 or shortform 12 or sf twelve or sftwelve or shortform twelve or short form twelve).mp.	4980
#15	(sf36 or sf 36 or short form 36 or shortform 36 or sf thirtysix or sf thirty six or shortform thirtysix or shortform thirty six or short form thirtysix or short form thirty six).mp.	23191
#16	#5 or #6 or #7 or #8 or #9 or #10 or #11 or #12 or #13 or #14 or #15	80226
#17	#4 or #16	685940
#18	#3 and #17	523

3.2.3.2 Stage 2 search strategy:

Database: Ovid MEDLINE
Platform: Ovid SP

Table 3. MEDLINE search string.

	Query	**Results**
#1	Muscular Dystrophy, Duchenne	4638
#2	duchenne*.mp.	11693
#3	#1 or #2	11693
#4	Autoquestionnaire Qualite de vie Enfant Image.mp.	18
#5	(Behavior Assessment System for Children or BASC or Parent Form 50 or PF50 or DUX-25 or EuroQoL 5-domain or Functional Independence Measure* or FIM).mp.	4877
#6	(WeeFIM or Life Satisfaction Index or LSI or LSIA or Neurological Disorders Quality of Life Questionnaire or NeuroQOL or pediatric NeuroQOL or Offer Self-Image Questionnaire for Adolescents or OSIQ).mp.	1602
#7	(Pediatric Outcomes Data Collection Instrument or PODCI or Neuromuscular module or DMD module or Multidimensional Fatigue Scale or Generic Short-Form or SF15).mp.	411
#8	(SDQ or 'Strips of Life with Emoticons Questionnaire' or SOLE or 'World Health Organisation Quality of Life Scale-Brief Version' or WHOQOL-BREF).mp.	53728
#9	('Strength and Difficulties Questionnaire').mp. [mp=title, abstract, original title, name of substance word, subject heading word, floating sub-heading word, keyword heading word, protocol supplementary concept word, rare disease supplementary concept word, unique identifier, synonyms]	223
#10	"Individualized Neuromuscular Quality of Life Questionnaire".mp.	9
#11	(INQOL or Child Activity Limitations Interview or CALI or "Satisfaction with Life Scale" or SWLS).mp.	1461
#12	'Pediatric Quality of Life Inventory'.mp.	871
#13	("Pediatric Orthopedic Society of North America Pediatric Musculoskeletal Functional Health Questionnaire" or POSNA or Pittsburgh Sleep Quality Index).mp.	3411
#14	PedsQL.mp.	1078

#15	(SF-36 or EQ-5D*).mp.	23965
#16	"World Health Organization Quality of Life".mp.	1349
#17	(KIDSCREEN or Pittsburgh Sleep Quality questionnaire or PSQI).mp. [mp=title, abstract, original title, name of substance word, subject heading word, floating sub-heading word, keyword heading word, protocol supplementary concept word, rare disease supplementary concept word, unique identifier, synonyms]	2640
#18	"Childrens Assessment of Participation and Enjoyment".mp.	96
#19	(CAPE or "Child Health Questionnaire" or CHQ* or Health Utilities Index Questionnaire or HUI*).mp.	17206
#20	(Fatigue Severity Scale or FSS or "Hospital Anxiety and Depression Scale" or HADS or COPE Inventory or "Quality of Life in Neuromuscular Disease").mp.	10441
#21	(QoL-NMD DISABKIDS or "Depressionsiventar fur kinder und Jugendliche" or DIKJ or Beck Depression Inventory or BDI or CARE-NMD or State-Trait Anxiety Inventory or STAI or "Life Satisfaction Index for Adolescents" or LSI-A or Quality of Life Evaluation Scale or AUQUEI or Activity Limitations Questionnaire or ACTIVLIM).mp.	17136
#22	(sf12 or sf 12 or short form 12 or shortform 12 or sf twelve or sftwelve or shortform twelve or short form twelve).mp.	4886
#23	(sf36 or sf 36 or short form 36 or shortform 36 or sf thirtysix or sf thirty six or shortform thirtysix or shortform thirty six or short form thirtysix or short form thirty six).mp.	22934
#24	#4 or #5 or #6 or #7 or #8 or #9 or #10 or #11 or #12 or #13 or #14 or #15 or #16 or #17 or #18 or #19 or #20 or #21 or #22 or #23	140059
#25	#3 and #24	82

Full copies of the search strategies for Stage 1 and Stage 2 can be found in Appendix A.

3.2.4 Additional searches

Following establishes procedures,[11] we searched Google Scholar (last searched 30th January 2019) with the names of the PROMs identified in Stage 1 in order to identify potential development papers for the assessment of content validity. The first 100 hits on Google Scholar were screened for inclusion. Citation tracking, by screening of references and Google Scholar citations of included articles, was conducted on the full text articles meeting eligibility criteria at Stage 2 (last searched 6th February 2019), as a supplementary measure to identify any additional studies not captured by the database searching.[15]

3.3 Eligibility criteria

The following selection criteria was applied to the search results at Stage 1 (identifying PROMs):

- Published in English as a full-text original research article (i.e. not including abstracts, editorials, or reviews).

- Used a self-reported, multi-item PROM to assess *at least one aspect of* QoL in boys and/or men diagnosed with DMD (assisted or proxy-reported versions of PROMs were considered for inclusion so long as a self-report version of that PROM exists).
- At least 75% of the sample, on which data from the PROM is reported, was male diagnosed with DMD.

Additional selection criteria were applied at Stage 2 (content and structural validity):

- Described data on the content and/or structural validity of the PROMs identified in Stage 1 in boys and/or men diagnosed with DMD.
- Development studies on the PROMs identified in Stage 1, to assist with the assessment of content validity, were included in any published form (i.e. journal article, book chapter, user manual).

3.4 Selection process

In order to apply the eligibility criteria for the selection of papers from search results, the following steps were performed:

I) Inclusion criteria (Stage 1) were applied to the titles and abstracts of hits from the Stage 1 searches (and any additional papers identified in the Stage 2 searches or through citation tracking) by two independent reviewers. Records were selected for full-text review if deemed relevant, potentially relevant, or if doubt existed. Any discrepancy was resolved by a third reviewer.

II) Full-text articles identified in (I) were screened for selection using the Stage 1 inclusion criteria by two independent reviewers. Any disagreements were resolved by a third reviewer through discussion.

III) The PROMs identified in the articles in (II) were reviewed to ensure they met the requisite inclusion criteria (i.e. assessing an aspect of QoL). If a validated English PROM was not available for review, the corresponding articles were excluded.

IV) Full-text articles identified in (II) and (III) meeting the Stage 1 inclusion criteria AND identified as containing measurement properties using the COSMIN filter were screened for content and structural evidence using the Stage 2 inclusion criteria by two independent reviewers. Any disagreements were resolved by a third reviewer.

V) In order to identify key development papers for the PROMs identified in (II) and (III), Google Scholar was searched with the names of the PROMs and the first 100 hits were screened for inclusion. Results of the searches were screened for inclusion by two reviewers.

VI) Finally, citation tracking of all eligible articles at Stage 2 was conducted by reviewing references and citations on Google Scholar for any articles not identified in the initial searches that may meet the eligibility criteria for Stage 1 and/or Stage 2. Results of the searches were screened for inclusion by two reviewers.

3.5 Data extraction and quality appraisal

Data extraction was carried out by two reviewers using a pre-prepared data extraction sheet, with consensus on any ambiguities reached through discussion. The data extraction sheet was informed by the tools developed by COSMIN on reporting guidance: https://www.cosmin.nl/tools/guideline-conducting-systematic-review-outcome-measures/

The methodological quality of the PROM development papers, and studies on content and structural validity were assessed using up-to-date COSMIN standards via the new COSMIN risk of bias checklist.[16] Thirty-five items are used to assess the development studies, comprised of a separate rating of the quality of the concept elicitation process with patients (i.e. item generation for a new PROM), and a rating of the quality of the cognitive interview study (if present) evaluating comprehensiveness and comprehensibility of the PROM with patients.[16] Thirty-eight items are used to assess studies on content validity, made of one set of items assessing studies with patients about relevance, comprehensiveness, and comprehensibility, and the other set assessing studies with professionals (if present) about relevance and comprehensiveness.[16] A total rating for relevance, comprehensiveness, and comprehensibility of a PROM is determined separately. A separate rating is also determined for studies with patients or professionals. Finally, four items are used to assess the methodological quality of a structural validity study.[16]

When rating the methodological quality of the studies, each COSMIN standard (or item) is ranked on a 4-point scale: “very good”, “adequate”, “doubtful”, and “inadequate”. Total ratings are determined using the lowest rating for any item for that study (i.e. worst score counts).[17] Studies were initially rated independently by two reviewers, and, in the case of divergence, consensus was reached in a subsequent face-to-face meeting.

3.6 Evidence synthesis

In order to synthesise and assess evidence on content validity, two reviewers independently rated the results of PROM development studies, content validity studies, and the content of the PROM itself on

10 COSMIN criteria, split into 5 on relevance, 1 on comprehensiveness, and 4 on comprehensibility.[9] Ratings for each source of evidence could either be positive (+), negative (−), or indeterminate (?). Following this an overall judgment on the relevance, comprehensiveness, and comprehensibility of each PROM was made, which could be sufficient (+), insufficient (−), or inconsistent (±). Evidence on structural validity was assessed against the updated COSMIN criteria for good measurement properties, using the same rating scale as above.[13] Finally, the quality of the evidence was graded using a modified GRADE approach,[18] as either "high", "moderate", "low", or "very low". The GRADE approach takes into account the risk of bias of studies (or study quality); (in)consistency across studies; imprecision (based on sample sizes); and indirectness (of evidence).[13]

For quality assurance purposes, the quality of this systematic review itself was appraised against a recently developed COSMIN checklist to assess the quality of systematic reviews of health-related PROMs.[14]

4 Results

4.1 Identification of included studies

From an initial 1,752 records identified through database searching at Stage 1 and 2, 1,536 were excluded at the title/abstract review stage, leaving 216 papers for full-text review. Of these 216 papers, 87 were excluded as they were not full-text published research articles; 26 did not meet the required sample criteria of at least 75% of the sample being boys or men with DMD; 21 were judged not to be assessing QoL; 16 were not published in English; and finally 11 papers did not feature a multi-item PROM. Accordingly, a total of 55 records from the initial searches met the eligibility criteria for Stage 1. The PROMs extracted from these papers are summarised in Section 4.2 below. The observed proportionate agreement between reviewers was 92.4% at title/abstract, with Cohen's κ = 0.51 or "moderate agreement" and is similar to other published reviews.[19-20] At full-text review, the observed proportionate agreement was 93.5% with Cohen's κ = 0.82 or "almost perfect agreement". A further 5 articles that met the eligibility criteria for Stage 1 were added as a result of citation tracking.

Of the papers that initially met the review criteria at Stage 1, subsequent to a review of the PROM itself, 5 were excluded as not containing content assessing QoL; 4 were not taken forward as no free/review copy was available; 3 were excluded because no validated English copy of the PROM was available; and 1 was excluded as the particular variant of the PROM used (of a potential large item bank) was not clear. Of the resultant 47 records, 20 were identified by the COSMIN measurement properties filter[14] as potentially containing measurement properties. Following review, 11 of these records were excluded for containing information on measurement properties other than content and structural validity; and 4 were excluded as not containing information on measurement properties. The remaining 5 papers featured evidence on content validity (*n* = 3, of which one was classified as a development paper) and structural validity (*n* = 2). Finally, 33 PROM development papers were identified through a review of Google Scholar search results and 3 PROM development papers were identified through citation tracking, resulting in a final selection of 41 papers that met the eligibility for Stage 2 (see Figure 1).

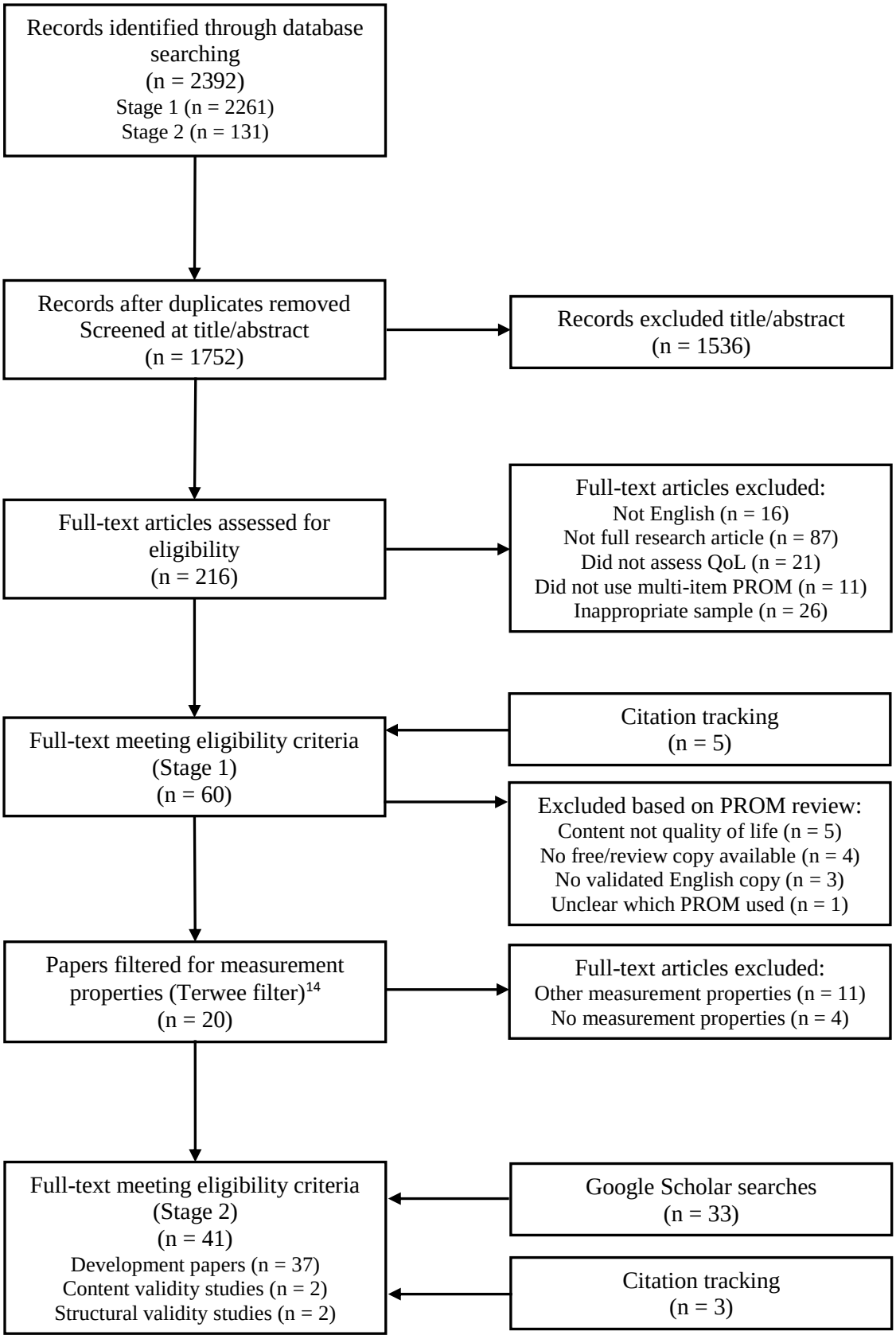

Figure 1. Flow diagram of search strategy and selection of papers.

4.2 PROMs used to measure quality of life in DMD

Table 4 summarises the PROMs used to assess QoL in DMD from the full-texts meeting the initial eligibility criteria at Stage 1 (*n* = 60).

Table 4. PROMs assessing QoL identified in published articles (*n* = 60) including samples of people with DMD.

PROM	Respondent type	Recall Period	*N* dimensions (items)	Dimensions of quality of life assessed	Response options	Total score range	Origin	Validated English copy and development papers freely available for review?
36-item Short-Form Health Survey (SF-36) v1.0[21-28]	Adult self-report	Varies by dimension	8 + a single item of perceived change in health (36 items)	Physical functioning; bodily pain; role limitations due to physical health problems; role limitations due to personal or emotional problems; emotional well-being; social functioning; energy/fatigue; general health perceptions	Varies by dimension	No total score calculated	USA	Yes
SF-36 Health Survey v2.0[29]	Adult self-report	Varies by dimension	8 + a single item of perceived change in health (36 items)	Assumed same as SF-36 v1.0	Varies by dimension	No total score calculated	USA	No
Autoquestionnaire Qualité de vie Enfant Imagé (AUQEI)[30]	Child self-report	Unknown/undefined	4 + a total score (26 items)	Autonomy; leisure; functioning; family; total	0 - 3 rating scale (with pictures)	0 – 78 (raw)	France	No
Beck Depression Inventory I (BDI)[25]	Adult interview or self-report	Present/today	1 total score (21 items)	Depression	0 - 3 rating scale	0 – 63 (raw)	USA	Yes

Behavior Assessment System for Children (BASC) first edition[31]	Child self-report Parent report Teacher report	Unknown (withdrawn from use, superseded by BASC-II and BASC-III)	Unknown (withdrawn from use, superseded by BASC-II and BASC-III)	Unknown (withdrawn from use, superseded by BASC-II and BASC-III)	Unknown (withdrawn from use, superseded by BASC-II and BASC-III)	Unknown (withdrawn from use, superseded by BASC-II and BASC-III)	USA	No
Child Activity Limitations Interview (CALI)[31]	Child interview or self-report	Last 4 weeks	1 total score (8 items chosen from a set of 21)	Activity limitations	0 - 4 rating scale	0 – 32 (raw)	USA	Yes
Child Health Questionnaire - Parent Form 50 (CHQ-PF50)[32-33]	Parent self-report	Last 4 weeks (past year for change in health)	14 (50 items)	Physical functioning; role/social limitations – physical; role/social limitations – emotional; role/social limitations – behavioral; general health perceptions; bodily pain/discomfort; family activities; parent impact – time; parent impact – emotion; self-esteem; mental health; behaviour; family cohesion; change in health	Varies by dimension	No total score calculated	USA	No
Children's Assessment of Participation and Enjoyment (CAPE)[34-36]	Child and young adults self-report or interview	Unknown/undefined	5 + a total score (55 items)	Diversity of activities; intensity of activities (frequency of participation); enjoyment of activities; with whom; where; total participation	Varies by dimension	0 – 55 (raw)	USA	No
Depressions-Inventar für Kinder und Jugendliche (DIKJ) 2nd edition[25]	Child self-report	Unknown/undefined	1 total score (26 items)	Depression	Unknown/undefined	0 – 46 (raw)	Germany	No

DISABKIDS generic module (DCGM-37)[25]	Child self-report Proxy report	Past 4 weeks	6 + a total score (37 items)	Independence; emotion; social inclusion; social exclusion; limitation; treatment; total	1 – 4 rating scale	37 – 148 (raw)	Multi-country	Yes
DISABKIDS – Smileys[25]	Child self-report Proxy report	Assumed same as DCGM-37	Assumed same as DCGM-37 (12 items)	Assumed same as DCGM-37	Assumed same as DCGM-37 (with pictures)	12 – 48 (raw)	Multi-country	No
Dutch Children AZL/TNO Questionnaire Quality of Life Short Form (DUC-25)[36]	Child self-report	Unknown/undefined	4 + a total score (25 items)	Physical; emotional; social; home functioning; total	0 - 4 rating scale	0 – 100 (raw)	Netherlands	No
EuroQoL 5-domain 3-Level (EQ-5D-3L)[37-38]	Adult self-report Proxy report	Present/today	5 (5 items) + self-rated health VAS	Mobility; self-care; usual activities; pain/discomfort; anxiety/depression	1 – 3 rating scale	−0.594 – 1 (utility scores)	Multi-country	Yes
Fatigue Severity Scale (FSS)[27]	Adult self-report	Within last week	1 total score (9 items) + global fatigue VAS	Fatigue severity	1 – 7 rating scale	1 – 63 (raw)	USA	Yes
Generalized Anxiety Disorder Scale 7-item (GAD-7)[39]	Adult self-report	2 weeks	1 total score (7 items)	Anxiety	0 – 3 rating scale	0 – 21 (raw)	USA	Yes
Health Utilities Index Questionnaire mark 2 (HUI-2) 15Q[40-41]	Child self-report Adult self-report Proxy report	During past 4 weeks	7 (15 items)	Sensation; mobility; emotion; cognition; self-care; pain; fertility	Varies by dimension	−0.03 – 1 (utility scores)	Canada	Yes

Health Utilities Index Questionnaire mark 3 (HUI-3) 15Q[40-41]	Child self-report Adult self-report Proxy report	During past 4 weeks	8 (15 items)	Vision; hearing; speech; ambulation; dexterity; emotion; cognition; pain	Varies by dimension	−0.36 – 1 (utility scores)	Canada	Yes
Hospital Anxiety and Depression Scale (HADS)[27,38]	Adult self-report	Last week	2 (14 items)	Anxiety; depression	0 – 3 rating scale	No total score calculated	UK	Yes
Individualized Neuromuscular Quality of Life Questionnaire (INQOL)[29]	Adult self-report	At the moment	10 + a total score (45 items)	Weakness; locking; pain; fatigue; activities; independence; social relationships; emotions; body image; treatment; total	7-point rating scale, varies by dimension	Scoring unclear	UK	Yes
KIDSCREEN-10[42]	Child self-report Proxy report	Last week	1 total score (10 items)	Health-related quality of life	1 – 5 rating scale	10 – 50 (raw)	Multi-country	Yes
KIDSCREEN-27[43]	Child self-report Proxy report	Last week	5 (27 items)	Physical well-being; psychological well-being; autonomy and parent relation; social support and peers; school environment	1 – 5 rating scale	No total score calculated	Multi-country	Yes
KIDSCREEN-52[44]	Child self-report Proxy report	Last week	10 (52 items)	Physical well-being; psychological well-being; moods and emotions; self-perception; autonomy; parent relation and home life; financial resources; social support and peers; school environment; social acceptance (bullying)	1 – 5 rating scale	No total score calculated	Multi-country	Yes

Life Satisfaction Index for Adolescents (LSIA)[45-48]	Child and young adults self-report	At present	5 + a total score (45 items)	General well-being; interpersonal relationships; personal development; personal fulfilment; leisure and recreation; total	1 – 5 rating scale (plus 0 = N/A)	0 – 225 (raw)	Canada	Yes
Muscular Dystrophy Child Health Index of Life with Disabilities (MDCHILD)[49]	Child self-report	Past 4 weeks	7 + a total score (47 items)	Activities of daily living & independence; positioning, transferring, & mobility; comfort & endurance; emotions & behaviour; social interaction & school; health; your overall quality of life; total	Varies by dimension	0 – 100 (transformed)	Canada	Yes
Neurological Disorders Quality of Life Questionnaire (Neuro-QoL)[26]	Adult self-report	Varies by dimension	Up to 16 (up to 564 items in item banks)	Ability to participate in social roles and activities; anxiety; bowel function; cognitive function; communication; depression; emotional and behavioral dyscontrol; fatigue; lower extremity function – mobility; positive affect and well-being; satisfaction with social roles and activities; sleep disturbance; sexual function; stigma; upper extremity function – fine motor, ADL; urinary/bladder function	1 – 5 rating scale	No total score calculated	USA	Yes
Offer Self-Image Questionnaire for Adolescents (OSIQ)[45,48]	Child and young adult self-report or interview	Unknown/undefined	11 + a total score (130 items)	Impulse control; emotional tone; body and self-image; social relationships; morals; vocational and educational goals; family relationships; mastery of the external world; psychopathology; superior adjustment; total	1 – 6 rating scale	130 – 780 (raw)	USA	No

Patient Health Questionnaire 9-item (PHQ-9)[39]	Adult self-report	2 weeks	1 total score (9 items)	Depression	0 – 3 rating scale	0 – 27 (raw)	USA	Yes
Pediatric Neurological Disorders Quality of Life Questionnaire (Pediatric Neuro-QoL)[26]	Child self-report	Varies by dimension	Up to 11 (up to 161 items in item banks)	Anger; anxiety; cognitive function; depression; fatigue; lower extremity – mobility; pain; social relations – interaction with adults; social relations – interaction with peers; stigma; upper extremity – fine motor, ADL	1 – 5 rating scale	No total score calculated	USA	Yes
Pediatric Outcomes Data Collection Instrument (PODCI)[50-53]	Child self-report Proxy report	Varies by dimension	7 (86 items)	Global function & comfort; upper extremity function; physical function and sport; transfers and mobility; comfort; POSNA happy and satisfied; POSNA expectations	Varies by dimension	No total score calculated	USA	Yes
Pediatric Quality of Life Inventory (PedsQL) 3.0 DMD module[54-55]	Child and young adult self-report Proxy report	Past month or past 7 days (acute version)	4 (18 items)	Daily activities; treatment barriers; worry; communication	0 – 4 rating scale	No total score calculated	USA	Yes
PedsQL 3.0 Multidimensional fatigue scale (MFS)[55-56]	Adult self-report Child and young adult self-report Proxy report	Past month or past 7 days (acute version)	3 + a total score (18 items)	General fatigue; sleep/rest fatigue; cognitive fatigue; total fatigue	0 – 4 rating scale	0 – 100 (transformed)	USA	Yes
PedsQL 3.0 Neuromuscular module (NMM)[26,41-42,55-61]	Child and young adult self-report Proxy report	Past month or past 7 days (acute version)	3 + a total score (25 items)	About my/my child's neuromuscular disease; communication; about our family resources; total	0 – 4 rating scale	0 – 100 (transformed)	USA	Yes

Pediatric Quality of Life Inventory (PedsQL) 4.0 Generic Core Scales (GCS)[26,34,42,50,53-57,59,62-69]	Adult self-report Child and young adult self-report Proxy report	Past month or past 7 days (acute version)	5 + a total score (23 items)	Physical health; psychosocial health; emotional functioning; social functioning; school functioning; total	0 – 4 rating scale	0 – 100 (transformed)	USA	Yes
PedsQL 4.0 Generic Short-form (SF-15)[70]	Adult self-report Child and young adult self-report Proxy report	Past month or past 7 days (acute version)	5 + a total score (15 items)	Physical health; psychosocial health; emotional functioning; social functioning; school functioning; total	0 – 4 rating scale	0 – 100 (transformed)	USA	Yes
Pittsburgh Sleep Quality Index (PSQI)[26,66]	Adult self-report	Past month	8 + a total score (10 items)	Subjective sleep quality; sleep latency; sleep duration; sleep efficiency; sleep disturbance; use of sleep medication; daytime dysfunction; total	Varies by dimension	0 – 21 (raw)	USA	Yes
Satisfaction with Life Scale (SWLS)[39,45]	Adult self-report	Undefined/present time	1 total score (5 items)	Life satisfaction	1 – 7 rating scale	5 – 35 (raw)	USA	Yes
Strength and Difficulties Questionnaire (SDQ)[71-73]	Child self-report Proxy report	Last 6 months	5 + a total score (25 items) + an impact supplement	Emotional symptoms; conduct problems; hyperactivity/inattention; peer relationship problems; total difficulties; prosocial behaviour	0 – 2 rating scale	0 – 40 (raw)	UK	Yes
Strips of Life with Emoticons Questionnaire (SOLE)[74]	Child self-report	Specific scenarios	1 total score (33 items)	Quality of life	0 – 2 rating scale (with pictures)	0 – 66 (raw)	Italy	No

TNO-AZL Children's Quality of Life questionnaire (TACQoL)[75]	Child self-report Proxy report	The last few weeks	7 (56 items)	Physical functioning; motor functioning; independent daily functioning; cognitive functioning and school performance; social contacts; positive moods; negative moods	Varies by dimension	No total score calculated	Netherlands	No
TNO-AZL Adult Quality of Life questionnaire (TAAQoL)[75]	Adult self-report	In the last month	12 (45 items)	Gross motor functioning; fine motor functioning; cognition; sleep; pain; social contacts; daily activities; sex; vitality; happiness; depressive mood; anger	Varies by dimension	No total score calculated	Netherlands	No
World Health Organisation Quality of Life Scale-Brief Version (WHOQOL-BREF)[24,26-28]	Adult self-report or interview Proxy report	2 weeks	4 (26 items, 24 items make up domain scores)	Physical health; psychological; social relationships; environment	1 – 5 rating scale	No total score calculated	Multi-country	Yes

Note. References next to PROM names represent published studies where the PROM has been used in a sample of people with DMD.

A total of 40 PROMs used to assess at least one aspect of QoL in DMD were identified in published research articles through database searching (the two HUI classification systems use the same 15-item PROM). The majority of the PROMs were multidimensional (*n* = 32), designed to assess a range of different facets of QoL. The remaining unidimensional scales were designed to assess: activity limitations (CALI); anxiety (GAD-7); depression (BDI, DIKJ, PHQ-9); fatigue severity (FSS); life satisfaction (SWLS); or quality of life/health-related quality of life unidimensionally (KIDSCREEN, SOLE). Twenty-four of the PROMs had versions designed for completion by adult or young adult respondents, and 26 had versions designed for children. The most popular PROMs used in published research articles assessing QoL in people with DMD were the PedsQL 4.0 GCS (18 articles); PedsQL 3.0 NMM (10 articles); and the SF-36 (8 articles).

In the current review, 26 PROMs were taken forward for COSMIN quality assessment on content and structural validity in DMD. The remaining 14 PROMs were not assessed for the following reasons: a copy of the PROM itself and/or necessary development papers were not freely accessible for review (CAPE, CHQ-PF50, DISABKIDS Smileys, OSIQ, SF-36 v2); no formally validated English copy of the PROM was available or in use (AUQEI, DIKJ, DUC-25, SOLE, TAAQoL, TACQoL); the PROM was no longer available or recommended for use (BASC 1st edition, which has been superseded by the BASC 2); or it was unclear from the study which of a large number of possible variants of a PROM were used (pediatric Neuro-QoL, Neuro-QoL). Table 5 lists the PROMs taken forward for review.

4.3 Content validity

4.3.1 Appraisal of PROM development studies

Table 5 summarises key characteristics and COSMIN quality assessment of the development of the PROMs included in the review, this includes the definition of the construct intended to be measured, target population, and intended context of use of the PROM. Five PROMs were developed to be intended for use specifically within neuromuscular disorders (INQoL, PedsQL 3.0 NMM) or DMD (LSIA, MDCHILD, PedsQL 3.0 DMD module; Table 5). Eleven PROMs either had no patients involved in their development, or it was unclear if patients were involved.

The joint most common COSMIN quality rating assigned to the PROMs for concept elicitation was inadequate (*n* = 12). This was primarily due to: the PROM development study not being performed in a sample of patients representing the target population (BDI, EQ-5D-3L, GAD-7, HADS, HUI 15Q,

PedsQL 3.0 MFS, PHQ-9, SDQ, SF-36, and SWLS); or inadequacies within the details of the qualitative methods used (FSS, INQoL). The concept elicitation study of 11 further PROMs was rated as doubtful due to at least some unclear details/suspected problems within the qualitative methods used (CALI, DCGM-37, LSIA, MDCHILD, PODCI, PedsQL 3.0 NMM, PedsQL 3.0 DMD, PedsQL 4.0 GCS, PedsQL 4.0 SF-15, PSQI, WHOQOL-BREF). Only the KIDSCREEN family of measures (*n* = 3) received an adequate rating for concept elicitation and PROM design. However, the KIDSCREEN measures received a doubtful rating for the overall PROM development study, for failing to provide evidence that comprehensibility and comprehensiveness were assessed in the cognitive interview/pilot study of the PROM.

Table 5. Characteristics and assessment of development papers for PROMs included in the review.

						Concept elicitation study	
PROM	**Reference(s)**	**Original language**	**Construct definition**	**Target population**	**Intended context of use**	**COSMIN quality rating**	**Were patients involved?**
BDI	Beck et al. 1961[76]	English (US)	"the items were chosen on the basis of their relationship to the overt behavioral manifestations of depression and do not reflect any theory regarding the etiology or the underlying psychological processes in depression"	Adult patients with suspected symptoms of depression	Quantitative assessment of the intensity of depression in diagnostic and research settings	Inadequate	No
CALI	Palermo et al. 2004[77]	English (US)	"functional impairment, defined as difficulty in performing age-appropriate physical, mental, and social activities in daily life due to physical health status (…) functional impairment due to pain (…) specific areas of functioning that are important to children and adolescents with recurrent and chronic pain"	School-age children and adolescents with recurrent and chronic pain	Research and clinical care	Doubtful	Yes
DCGM-37	Petersen et al. 2005[78] Ravens-Sieberer et al. 2007[79]	English (UK)	"a multidimensional construct with social, physical, emotional, and functional domains"	Children aged 4-7 years and 8-16 years with chronic health conditions	Clinical studies or surveys	Doubtful	Yes
EQ-5D-3L*	EuroQol Group 1990[80] Brooks et al. 1996[81]	Multiple, including English (UK)	"Health-related quality of life"	"Large-scale surveys of the community and (…) for use in postal surveys"	"Complement other quality of life measures, collection of common data set for reference. Generate cross-national comparisons of health state valuations."	Inadequate	No
FSS	Krupp et al. 1989[82]	English (US)	"Fatigue"	Patients with "clinical disorders"	Clinical research studies and surveys	Inadequate	No

GAD-7	Spitzer et al. 2006[83]	English (US)	"We first selected potential items for a brief GAD [Generalized Anxiety Disorder] scale (…) that reflected all of the *Diagnostic and Statistical Manual of Mental Disorders, Fourth Edition (DSM-IV)* symptom criteria for GAD and (…) on the basis of review of existing anxiety scales."	General adult population	Clinical practice and research	Inadequate	No
HADS	Zigmond & Snaith 1983[84]	English (UK)	"depression subscale were largely based on the anhedonic state (…) psychic manifestations of anxiety neurosis"	Patients under investigation and treatment in medical and surgical departments in non-psychiatric hospital departments	Clinical/screening use within non-psychiatric hospital departments	Inadequate	No
HUI-2 / HUI-3 (15Q)	Feeny et al. 1995[85] Torrance et al. 1996[86]	English (US)	"The HUI Mark II and Mark III systems are based on concepts of functional capacity rather than performance (…) generic health profile measures that also permit the computation of a single summary score quantifying health-related quality of life"	Originally survivors of childhood cancer (HUI-2), extended to adults	Clinical evaluative and population health survey studies, in clinical trials, and cost-utility analyses	Inadequate	Unknown
INQoL	Vincent et al. 2007[87]	English (UK)	"The structure of INQoL was based on the ICIDH-2 model of disease incorporating the concepts of Impairment, Activities, and Participation."	Adults with neuromuscular disorders (16+ years)	Clinical and research use	Inadequate	Yes

KIDSCREEN-52	Ravens-Sieberer et al. 2001[88] Ravens-Sieberer et al. 2005[89] Detmar et al. 2006[90]	Multiple, including English (UK)	"Health-related quality of life is described as a multidimensional construct covering physical, emotional, mental, social, and behavioral components of well-being and function as perceived by patients and/or individuals (…) agreement was reached that the questionnaire should aim to measure HRQOL as a generic construct in largely healthy children, thus more emphasis was given to the inclusion of psychosocial domains, and less to domains of physical functioning or symptoms such as pain."	Healthy and chronically-ill children and adolescents between 8 and 18 years	Epidemiological and paediatric studies, clinical settings (healthcare system), and health services research	Adequate	Yes
KIDSCREEN-27	Ravens-Sieberer et al. 2006[91]	Assumed the same as KIDSCREEN-52	Assumed the same as KIDSCREEN-52	Assumed the same as KIDSCREEN-52	Assumed the same as KIDSCREEN-52	Adequate	Yes
KIDSCREEN-10	Ravens-Sieberer et al. 2006[91]	Assumed the same as KIDSCREEN-52	Assumed the same as KIDSCREEN-52	Assumed the same as KIDSCREEN-52	Assumed the same as KIDSCREEN-52	Adequate	Yes

LSIA	Reid & Renwick 1994[45]	English (US)	"quality of life is to conceptualize it as a subjective phenomenon. Specifically, it is viewed in terms of the individual's feelings and evaluations of his or her life circumstances. Many researchers who study quality of life within this perspective emphasize the importance of measuring the individual's degree of life satisfaction. In other words, they are interested in how pleased an individual feels about particular aspects of his or her life"	"Individuals between the ages of 12 and 19 years who have DMD"	Research instrument and potentially useful as a clinical measure	Doubtful	Yes
MDCHILD	Propp, 2017[92] Propp et al. 2019[49]	English (UK)	"Health-related priorities for children with DMD (…) defined as concerns, desires, and expectations arising from the lived experience of that condition"	Children with DMD (assumed 5 – 18 years)	Cohort studies, clinical trials, and clinical decision-making	Doubtful	Yes
PedsQL 3.0 DMD	Uzark et al. 2012[54]	English (US)	"Health-related quality of life (QoL), a multidimensional construct that includes physical, psychological, and social functioning, has emerged as an important outcome in pediatric populations with chronic health conditions."	Children with DMD from 2 – 18 years	Assumed the same as PedsQL 4.0 GCS	Doubtful	Yes
PedsQL 3.0 MFS	Varni et al. 2002[93]	English (US)	"designed to measure child and parent perceptions of fatigue in pediatric patients"	Assumed the same as PedsQL 4.0 GCS	"may be utilized as outcome measures in pediatric cancer clinical trials, research, and clinical practice for HRQOL"	Inadequate	Yes

PedsQL 3.0 NMM	Iannaccone et al. 2009[94]	English (US)	"HRQOL is a multidimensional construct, consisting at the minimum of physical, psychological (including emotional and cognitive), and social health dimensions delineated by the World Health Organization. HRQOL has emerged as the most appropriate term for quality of life dimensions that represent a patient's perceptions of the impact of an illness and its treatment on their own functioning and well-being and which are within the scope of healthcare services and medical products."	Children and young people with neuromuscular disorders, in particular spinal muscular atrophy	Assumed the same as PedsQL 4.0 GCS	Doubtful	Yes
PedsQL 4.0 GCS	Varni et al. 1999[95]	English (US)	"The PedsQL measures the patient's and the parent's perceptions of the patient's HRQOL, as defined in terms of the impact of disease and treatment on an individual's physical, psychological, and social functioning, and by disease/treatment-specific symptoms."	Children aged 8 – 18 across various pediatric chronic health conditions	Epidemiological studies, clinical trials, and performance improvement studies	Doubtful	Yes
PedsQL 4.0 SF-15	Varni et al. 1999[95]	English (US)	Assumed the same as PedsQL 4.0 GCS	Assumed the same as PedsQL 4.0 GCS	Assumed the same as PedsQL 4.0 GCS	Doubtful	Yes

PHQ-9	Spitzer et al. 1999[96] Kroenke et al. 2001[97]	English (US)	"Depression (…) using diagnostic criteria from the *Diagnostic and Statistical Manual of Mental Disorders, Revised Third Edition (DSM-III-R)* and *Diagnostic and Statistical Manual of Mental Disorders, Fourth Edition (DSM-IV)."*	General adult population	Clinical practice and research	Inadequate	No
PODCI	Daltroy et al. 1998[98]	English (US)	"The POSNA outcomes instrument scales assess upper extremity function, transfers and mobility, physical function and sports, comfort (painfree), happiness and satisfaction, and expectations for treatment. A POSNA global scale combines the three function subscales and comfort."	Children aged 2-18 years with musculoskeletal disorders	"Patient-based instrument"	Doubtful	Yes (assumed)
PSQI	Buysse et al. 1989[99]	English (US)	"sleep quality is a readily accepted clinical construct, it represents a complex phenomenon that is difficult to define and measure objectively. 'Sleep quality' includes quantitative aspects of sleep, such as sleep duration, sleep latency, or number of arousals, as well as more purely subjective aspects, such as "depth" or "restfulness" of sleep"	Clinical/psychiatric populations	Psychiatric clinical practice and research activities	Doubtful	No
SDQ	Goodman 1997[100]	English (UK)	"young people's behaviours, emotions, and relationships"	Children and young people (aged 4-16 years)	"to meet the needs of researchers, clinicians, and educationalists"	Inadequate	No

SF-36 v1.0*	Ware & Sherbourne, 1992[101] Hays et al. 1993[102] Jenkinson et al. 1999[103] Ware 2000[104]	English (US)	""Health", eight concepts: physical functioning, social and role functioning, mental health, general health perceptions, bodily pain, and vitality."	"General population and patients"	"Clinical practice and research, healthy policy evaluations, and general population surveys"	Inadequate	No
SWLS	Diener et al. 1985[105]	English (US)	"Life satisfaction refers to a cognitive, judgmental process. Shin and Johnson (1978) define life satisfaction as "a global assessment of a person's quality of life according to his chosen criteria" (p. 478)"	Unclear	Unclear	Inadequate	No

WHOQOL-BREF	WHOQOL Group 1994[106] WHOQOL Group 1995[107] Skevington et al. 1997[108] WHOQOL Group 1998[109]	Multiple, including English (UK)	" It is a broad ranging concept incorporating, in a complex way, the person's physical health, psychological state, level of independence, social relationships, personal beliefs, and relationship to salient features of the environment (…) At minimum, quality of life includes the following dimensions: physical (individuals' perception of their physical state), psychological (individuals' perception of their cognitive and affective state) and social (individuals' perception of the interpersonal relationship relationships and social roles in their life). (…) The WHOQOL includes a spiritual dimension (the person's perception of 'meaning in life', or the overarching personal beliefs that structure and qualify experience)."	“assess the quality of life of chronic disease sufferers, informal caregivers of the sick and disabled, people living in high-stress conditions like refugees, and 'healthy' people”	"in routine clinical work, large scale epidemiological studies and in clinical trials"	Doubtful	Yes

Note. *PROM development information obtained from prior COSMIN review[110], not re-extracted or re-rated in this review, based on COSMIN guidance[9]

4.3.2 Appraisal of content validity studies

Aside from development studies, only 2 published articles had independently assessed the content validity of the QoL PROMs in samples of people with DMD, and the details of these studies and their COSMIN quality assessment are described in Table 6. Neither of these studies were conducted in a UK context, and instead were cross-cultural validation studies. Hu et al. (2013)[59] assessed the relevance, comprehensiveness, and comprehensibility of the PedsQL 3.0 NMM in Chinese children with DMD. Simon et al. (2017)[47] assessed comprehensibility of the LSIA in Brazilian children with DMD, and comprehensiveness in professionals. However, both of these studies received ratings of doubtful due to at least some unclear details/suspected problems within the qualitative methods used.

Table 6. Characteristics and assessment of content validity papers in DMD samples for PROMs included in the review.

PROM	Reference	Language (Country)	DMD sample characteristics			COSMIN rating			Results (synthesis)
			N	Age (years, ±SD)	% ambulatory	Relevance	Comprehensiveness	Comprehensibility	
LSIA	Simon et al. 2017[47]	Brazilian Portuguese (Brazil)	43	11.4 ± 3.38	Not stated	/	Doubtful	Doubtful	The level of comprehension reached via the final Probe technique was 97% for the parent version and 95% for the patient version, which is above the minimum of 85% required.
PedsQL 3.0 NMM	Hu et al. 2013[59]	Chinese (China)	56	7.54 ± 4.06	37 children "could climb stairs"	Doubtful	Doubtful	Doubtful	Cognitive debriefing was conducted with six children with DMD and their parents to confirm that the final Chinese version was understandable and acceptable.

Note. / = content validity aspect not evaluated.

4.3.3 Evidence synthesis

The evidence from the PROM development papers and content validity studies was combined with reviewer ratings of the PROMs to produce a synthesis of the available evidence using the 10 COSMIN criteria for good content validity.[9] Most of the quality of the evidence was downgraded from High to Low or Very Low due to the assessment being based on development studies of doubtful or inadequate quality, respectively.[9] Only the LSIA and the PedsQL 3.0 NMM had moderate supporting evidence, featuring independent content validity studies as well as development papers. The KIDSCREEN measures and the LSIA were the only PROMs to receive satisfactory results for all three dimensions of content validity: relevance; comprehensiveness; and comprehensibility, based on the evidence available. Full synthesised results are presented in Table 7.

Table 7. Evidence synthesis on the content and structural validity of PROMs used to assess QoL in people with DMD.

	Content validity				Structural Validity	
PROM	**Relevance**	**Comprehensiveness**	**Comprehensibility**	**Quality of evidence**	**Rating of results**	**Quality of evidence**
BDI	±	−	±	Very low	?	?
CALI	+	−	±	Low	?	?
DCGM-37	±	+	+	Low	?	?
EQ-5D-3L	+	−	+	Very low	?	?
FSS	±	−	±	Very low	?	?
GAD-7	+	−	+	Very low	?	?
HADS	−	−	−	Very low	?	?
HUI-2 / HUI-3 (15Q)	−	−	−	Very low	?	?
INQoL	±	±	+	Very low	?	?
KIDSCREEN-52	+	+	+	Low	?	?
KIDSCREEN-27	+	+	+	Low	?	?
KIDSCREEN-10	+	+	+	Low	?	?
LSIA	+	+	+	Moderate	?	?
MDCHILD	±	+	+	Low	?	?
PedsQL 3.0 DMD	±	?	±	Very low	?	?
PedsQL 3.0 MFS	±	−	±	Very low	?	?
PedsQL 3.0 NMM	±	?	±	Moderate	−	High
PedsQL 4.0 GCS	±	+	±	Low	?	Very low
PedsQL 4.0 SF-15	±	+	±	Low	?	?
PHQ-9	+	−	±	Very low	?	?
PODCI	±	+	±	Very low	?	?
PSQI	±	−	±	Very low	?	?
SDQ	−	−	+	Very low	?	?
SF-36 v1.0	+	+	±	Very low	?	?
SWLS	−	−	±	Very low	?	?
WHOQOL-BREF	+	+	±	Very low	?	?

Note. + = satisfactory results; − = unsatisfactory results; ± = inconsistent results; ? = indeterminate.

4.4 Structural validity

4.4.1 Appraisal of structural validity studies

Two studies had assessed the structural validity of the PROMs included in this review in samples of people with DMD (Table 8). Both of these were conducted using English versions of the PROMs and either in the UK or USA. Lim et al. (2014)[64] assessed the structural validity of the PedsQL 4.0 GCS using an unspecified Rasch model in 63 boys with DMD. This study received a COSMIN quality rating of doubtful because it was doubtful that the sample size included in the analysis was adequate. Landfeldt et al. (2018)[58] assessed the structural validity of the PedsQL 3.0 NMM using a Rasch partial-credit model (PCM) in 278 people with DMD. This study received a very good COSMIN quality rating for its methodological content.

Table 8. Characteristics, assessment, and results of structural validity papers in DMD samples for PROMs included in the review.

			Patient characteristics						
PROM	**Reference**	**Country (language)**	**N**	**Age (yr, M ±SD)**	**% ambulatory**	**PROM score**	**COSMIN Quality Rating**	**Analysis – model**	**Results (synthesis)**
PedsQL 4.0 GCS	Lim et al. 2014[64]	USA (English)	63 boys with DMD (and up to 50 parents, not necessarily matched)	10.2 ± 2.5	95.24	Child: M = 64.5, SD = 15.3. Parent: M=56.2, SD=12.9.	Doubtful	Rasch (model not specified)	Model misfit for items determined with infit > 1.4 and outfit > 2.0 MnSq values and standardized scores > 2.0. All items fit in parent proxy-reports of physical health scale and child self-reports of psychosocial health scale. 2 out of 8 items showed high infit statistics in child self-reports of the physical health scale (taking a bath or shower; doing chores around the house). In addition 2 out of 15 items showed high infit for the parent proxy-reports of the psychosocial health scale (trouble sleeping; keep up with school work).
PedsQL 3.0 NMM	Landfeldt et al. 2018[58]	UK / USA (English)	278 (95 UK)	16 ± 7	40% not "full-time wheelchair dependent"	Not reported	Very good	Rasch PCM	Eight items displayed inadequate fit (χ^2: p > 0.01). Six items had fit residuals ≤ −2.5 or ≥ 2.5 (4 significant at p < .05). Inadequate overall fit (χ^2 item-trait interaction: p = < .001). Disordered thresholds for 22 of 25 items. Suboptimal targeting.

4.4.2 Evidence synthesis

Of the 2 studies that assessed the structural validity of the PedsQL 4.0 GCS and PedsQL 3.0 NMM in people with DMD, neither provided satisfactory results (Table 7). First, the structural validity of the PedsQL 4.0 GCS in people with DMD received an indeterminate rating, as key details of the results from the Rasch model denoting good measurement properties were not reported. Due to the risk of bias assessment of Lim et al. (2014)[64] the quality of the evidence supporting this indeterminate conclusion was rated as very low. Second, the structural validity of the PedsQL 3.0 NMM in people with DMD received an unsatisfactory rating, as the psychometric criteria for good measurement properties were not met. The favourable risk of bias assessment for Landfeldt et al. (2018)[58] meant that the quality of evidence supporting this conclusion was graded as high.

4.5 Quality assurance of the review

The quality of this review was self-assessed against a newly derived COSMIN checklist[14], designed to evaluate the quality of systematic reviews of health-related PROMs. The results are displayed in Table 9. In general, the review meets numerous quality indicators as defined by the COSMIN team, including the elements included in the research aim, search strategies, article selection, and assessment of measurement properties and quality. In a couple of instances, criteria have been partly met. For example, in this review all instruments were included where a *validated English copy was freely available for review*. It is possible that additional instruments could have been included if licenses were paid for to access the relevant PROMs and development materials. Second, citation tracking (i.e. reference checking) was conducted on the final set of articles eligible at Stage 2 of the searches (*n* = 41), but not on results eligible for inclusion at Stage 1.

Table 9. Quality assessment of this systematic review against COSMIN guidance.

COSMIN criteria	Review meets criteria
Elements included in the research aim:	
Construct of interest	+
Population of interest	+
Type of measurement instrument of interest	+
Measurement properties of interest	+
All available instruments included	±
Only instruments included that have at least some evidence of measurement properties	+
Search strategy described	+
No search terms or validated search filter used for:	
Measurement properties	–
Type of instrument	–
Number of databases searched:	5
Search in at least 2 databases	+
MEDLINE/PubMed	+
EMBASE	+
Additional databases	+
Reference checking used	±
No time limits used or good arguments for a time limit	+
No language restrictions used	+
Inclusion and exclusion criteria clearly described	+
Reasons for excluding articles reported	+
Abstract selection by at least 2 reviewers?	+
Full-text article selection by at least 2 reviewers?	+
Abstract and full-text article selection by at least 2 reviewers?	+
Methodological quality of studies assessed	+
Quality assessment of studies done by at least 2 reviewers	+
Data on measurement properties extracted by at least 2 reviewers	+
Quality of the instrument (measurement properties) assessed	+
Quality assessment of the instrument by at least 2 reviewers	+
Results from multiple studies on the same instrument somehow combined (e.g., best evidence synthesis or pooling)	+
Data synthesis was performed:	
Per measurement property	+
Only for domains (reliability, validity, responsiveness)	
Only for the whole instrument	
Recommendation provided for the best instrument:	
One instrument is recommended per construct	+
More instruments are recommended per construct	
No recommendation for the best instrument	
Results for the measurement properties reported as raw data	+
Number of measurement properties reported	2
Conflict of interest or funding source declared	+
One of the authors of the review is also the developer of one of the instruments evaluated in the review	–

Note. + = criterion met; − = criterion not met; ± = criterion partly met

5 Discussion and conclusion

In this systematic review, the published scientific evidence on the content and structural validity of PROMs used to measure at least one aspect of QoL in people with DMD was thoroughly evaluated. The overriding theme was one of sparse evidence. Many PROMs that are being used to assess aspects of QoL in people with DMD are being utilised without the accompanying good quality evidence that supports their validity for this task. Only five of the PROMs uncovered in this review were specifically designed for use in people with neuromuscular problems (three for DMD), and only two of these have had their content and/or structural validity independently assessed in this population (with the content validity studies involving translated versions). When the evidence is available, most of it is either of a low quality, featuring insufficient detail in the published articles to make thorough and comprehensive assessments of content and structural validity as demanded by COSMIN,[13] leading to doubtful ratings. Indeed, one of the highest quality pieces of evidence reviewed in terms of reported methodology, Landfeldt et al. (2018)[58], reported insufficient structural validity of the PedsQL 3.0 Neuromuscular module (NMM) in DMD.

The results from the review should not be viewed as surprising. Many of the PROMs identified are what could be described as “legacy” measures. They were developed at a time when the science of construct and item generation was largely overlooked. The content of instruments was largely defined by clinical or expert opinion, with little explanation of what that entailed. The reporting of such stages in publications or questionnaire manuals was not commonplace. The transparency of reporting on the early stages of PROM development has only gained traction in the last decade or so. Whilst this is a positive step for researchers, clinicians and users alike, progress can be limited by journal restrictions on word count and remit. It is however possible for such legacy measures to be appropriately validated (or have their validity assessed) in properly designed studies assessing content or structural validity in modern samples of people with DMD. The problem observed in this review is that researchers are likely using such measures as a consequence of precedent or tradition, rather than a supportive evidence base. Thus the first recommendation from this review is for more research into the content and structural validity of QoL PROMs used in DMD, and, if the PROMs are found to be insufficient on these criteria, for additional PROM development in DMD samples.

The limitations of sparse evidence notwithstanding, some PROMs performed better than others under COSMIN assessment. First, the KIDSCREEN instrument (all versions) does show some evidence of applicability given that it covers many aspects of QoL. The PROM development study for the

KIDSCREEN instrument was the only one rated as adequate, it was designed to assess QoL in children and adolescents with chronic illnesses, and the ratings for the content validity of the measure were positive (based on the available evidence in the measure's development). However, it must also be borne in mind that there is little or no direct evidence to support the content or structural validity in DMD, specifically. The original KIDSCREEN instrument (52-item version) was designed to assess multiple aspects of QoL, namely: physical well-being; psychological well-being; moods and emotions; self-perception; autonomy; parent relation and home life; financial resources; social support and peers; school environment; and social acceptance (bullying), covering much of the CMQM framework.[6] The conceptual framework of the instrument is thus intuitively applicable to the Duchenne community; however the measurement of impact may be limited due to the target age range of the PROM itself (8 - 18 years). While this is not uncommon (i.e. differences in measuring QoL from child to adulthood), there is some question of the applicability for the broader DMD population given the lower age target. While we would recommend this PROM, above all others tested in the review, for measuring QoL in children and adolescents with Duchenne, more research is needed to definitively support the use of KIDSCREEN (and its derivatives) within DMD.

The second-best performing PROM in this review was the LSIA, which received a satisfactory score for relevance, comprehensiveness, and comprehensibility in terms of content validity, based on the information available and reviewers' ratings of the PROM itself. However, the development study for this paper lacked key details necessary in good PROM development, and thus was rated as doubtful. Furthermore, while the LSIA was one of few measures to feature a content validity study, it was a cross-cultural adaptation study of a Brazilian version of the measure, and the results of the formal assessment of this study were doubtful. Thus, the LSIA is possibly a good candidate for measuring QoL in DMD, but more evidence is needed. While the measure is comprehensive, it only comes in a 45-item version, which is potentially quite burdensome. Furthermore, the measure is designed for use in children and young adults only, and may not generalise to adults with DMD.

The most recent PROM developed specifically for use in children and adolescents with DMD was the MDCHILD. Although the PROM is designed to measure "health-related priorities",[49] much of the content maps onto the CMQM framework[6] and thus covers QoL. While the MDCHILD had many commendable strengths in PROM design, the overall rating of the PROM development, based on the COSMIN worst score counts system,[17] was rated as doubtful due to lack of details reported in the development papers. For example, it was unclear if skilled interviewer(s) were used; to what degree data was coded independently; and to what degree, if at all, at least two researchers were involved in

the data analysis. This led to a low quality of evidence. Further, because the target population of interest was not clearly defined (i.e. age ranges were not specified), despite performing well in other areas, the PROM received an inconsistent rating for relevance. These results speak to the potential harshness of a worst score counts system advocated by COSMIN, which we discuss further below. Further, because the PROM is new, there is a lack of published content validity studies that may improve the quality of evidence for the MDCHILD going forward, such as that contained in Chapter 7 of a non-peer-reviewed thesis,[92] not eligible for inclusion in the current review.

The PedsQL and associated modules were the most commonly used out of all the PROMs identified within the review. It should be noted that the development studies of the PedsQL were rated as doubtful. There was little evidence to support the content validity of the neuromuscular module of the PedsQL 3.0 (NMM). Furthermore, the psychometric properties of the NMM were not well supported by Landfeldt et al. (2018)[58]. The inclusion of PedsQL within clinical practice, cohort studies or pragmatic trials in DMD thus appears to be based upon precedent and common use, rather than published empirical evidence of suitability, based on content and structural validity. A notable advantage of the PedsQL (and its derivatives) is the young child (via proxy report), child (self and proxy report), young adult forms (self-report), and adult forms, which have now been developed. A further consideration is that the PedsQL scales are designed to be used in parallel (e.g. the generic core scales with the NMM or DMD modules), but were assessed individually under COSMIN guidance. Thus comprehensiveness may be improved by using these scales together. Nevertheless, in the absence of further evidence, it is difficult to recommend the routine use of the PedsQL to assess QoL in people with DMD on content and structural grounds. Instead, the findings of this review support the need for further PROM development, which is able to accurately assess the impact of DMD upon people's QoL.

The search identified some PROM instruments that we were unable to obtain. Access to the PROM and/or associated development papers was limited due to licensing requirements, and therefore it was not possible to include these instruments within the review. It is unlikely that these instruments are commonly used within research and/or clinical practice due to the difficulties around access. Their suitability for the DMD population cannot formally be determined; however, their use is likely to be limited by a lack of accessibility derived from license restrictions, reflected in the few citations in which they appeared.

Another consideration relevant to the selection of QoL PROMs for use in the DMD population is whether the PROM has accompanying utility weights to make it preference-based and amenable to

use in economic valuation. Most of the PROMs assessed in this review, except the EQ-5D-3L, HUI, and SF-36 (via the SF-6D)[111] were not preference-based measures. Further, these preference-based measures did not perform well on assessments of content validity. However, alternative strategies are possible in order to obtain utility weights for some of the alternative PROMs discussed in this review, including our recommended PROM (KIDSCREEN) and the most widely-used PROM (PedsQL). These include mapping algorithms to a preference-based measure,[112] enabling there use in cost-effective analysis. Nevertheless, these kinds of solutions are to a degree imperfect, and there is clearly room for the development of a new preference-based measure of QoL in people with DMD.

This review adopted guidance developed by the COSMIN initiative, and has adhered to their recommended methods in identification of evidence, data extraction, data assessment and data synthesis. Whilst the appropriateness of these robust methods cannot be questioned, this has resulted in relatively low ratings of the PROMs included within the review. It is important to recognise that this does not suggest categorically that the instruments used within published and/or current studies are not appropriate or fit for purpose; content and structural validity only form one component of PROM suitability within a population. Furthermore, as stated, many of the instruments were developed at a time when instrument development methods and procedures were not reported – that is not to say the development of the instruments is flawed, just that an assessment of them cannot be made. The COSMIN appraisal tools assume a worst score counts system for the rating of the methodological quality of studies.[17] This means that, in theory, a study could be rated as very good or adequate on all but one criteria, on which it is rated as doubtful or inadequate, and the overall score is thus reduced to the latter lower-quality rating. Sometimes this can be because key details, such as whether skilled interviewers were used, are not reported. We thus think that there is room for constructive debate on the usefulness of retaining a worst score counts system, as opposed to an alternative procedure that better reflects the variance or range of ratings across the COSMIN criteria. An example of this could be to use numerical scores to represent ratings for each of the COSMIN criteria and to calculate a (weighted) total, within which ranges or bands of scores reflect sequential improvements in methodological quality, from inadequate to very good.

Given that DMD is a rare condition, the development and validation of PROMs that measure the impact of the condition on QoL is challenging. The number of participants included within various phases of PROM development and validation will be lower than that of a condition such as diabetes, asthma or eczema. Some of the studies identified within the review included participants with other similar conditions. As part of the methods employed within the review, the inclusion criteria stated at

least 75% of the sample were to include men/boys with DMD. It is possible to expand the scope of the search to include a more "relaxed" criterion; however, it is not known how appropriate this would be. It can be postulated that other neuromuscular disorders could imply similar impacts upon QoL, however this has not been explored within the context of this review.

The rarity of DMD also limits assessment of cross-cultural applicability of items, concepts and indeed the PROMs. Very little (or no) data was identified that explored this issue. Few studies reported on participant ethnicity, nor discussed any potential issues that may differ between different ethnic groups. Some instruments were identified within the search that were not included within the review as no English version of the questionnaire was available or had been developed. Such instruments may be applicable to the DMD population, however this has yet to be determined and can only be assessed following a robust translational study.

The focus of this review was to report on the content and structural validity of PROM instruments that have been used to quantify the impact of DMD on individual's QoL. However, content and structural validity only address some aspects of PROM suitability, and further work could be undertaken to formally appraise the instruments described. Other measurement properties, such as psychometric performance, could be considered. Furthermore, the inclusion of subsidiary samples such as other neuromuscular disorders, may be of interest. One of the inclusion criteria of this review was a 75% DMD sample (as recommended within the COSMIN guidance), however given the rarity of DMD it may be appropriate to relax this approach to include a wider sample of neuromuscular disorders.

This review is not without its limitations. While the methodological approach of the review is robust and follows the recommendations of COSMIN and that of other published reviews, it must be acknowledged that the rating criteria of the PROMs identified can be viewed as harsh. The COSMIN approach encourages researchers and reviewers to critically appraise evidence of PROM development – however the presence of evidence within published literature is sparse. That is not to say that the development phases did not occur, merely that they are not reported and/or not reported in sufficient detail as required by COSMIN assessment. To critique a PROM's applicability using this criterion could be perceived as being unduly critical; more recent PROMs tend to report the early stages of instrument development, and we are assessing all PROMs by modern standards. Similarly, the descriptions of PROMs themselves are often lacking. Basic information such as number of items, recall period, domain structure and scoring procedure were noted to be sporadically reported, although better in recent literature. The COSMIN-recommended reviewer rating of the identified PROMs for suitability for DMD (as reported in Table 7) has a large subjective component. Whilst this was completed as per the

COSMIN guidelines (with two reviewers and discrepancies reconciled following discussion), some of the ratings are at risk of bias based on the team of raters. For example, it is not known whether similar ratings of suitability would be achieved if reviewed by an individual with DMD, a family member or carer of a person with DMD, or a clinician. This is further exacerbated when we consider what QoL is – for the purpose of this review it was a multidimensional construct, PROMs that measure a subset of interest (such as depression) may be appropriate to include within studies as part of a host/suite of measures.

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Appendix A: Full search strategies

Embase Stage One

1. Muscular Dystrophy/
2. duchenne*.mp.
3. 1 or 2
4. (HR-PRO or HRPRO or HRQL or HRQoL or QL or QoL).ti,ab. or quality of life.mp. or (health index* or health indices or health profile*).ti,ab. or health status.mp. or ((patient or self or child or parent or carer or proxy) adj (appraisal* or appraised or report or reported or reporting or rated or rating* or based or assessed or assessment*)).ti,ab. or ((disability or function or functional or functions or subjective or utility or utilities or wellbeing or well being) adj2 (index or indices or instrument or instruments or measure or measures or questionnaire* or profile or profiles or scale or scales or score or scores or status or survey or surveys)).ti,ab.
5. 'Pediatric Quality of Life Inventory'.mp.
6. PedsQL.mp.
7. (SF-36 or EQ-5D*).mp.
8. "World Health Organization Quality of Life".mp.
9. (KIDSCREEN or Pittsburgh Sleep Quality questionnaire or PSQI).mp. [mp=title, abstract, heading word, table of contents, key concepts, original title, tests & measures]
10. "Childrens Assessment of Participation and Enjoyment".mp.
11. (CAPE or "Child Health Questionnaire" or CHQ* or Health Utilities Index Questionnaire or HUI*).mp.
12. (Fatigue Severity Scale or FSS or "Hospital Anxiety and Depression Scale" or HADS or COPE Inventory or "Quality of Life in Neuromuscular Disease").mp.
13. (QoL-NMD DISABKIDS or "Depressionsiventar fur kinder und Jugendliche" or DIKJ or Beck Depression Inventory or BDI or CARE-NMD or State-Trait Anxiety Inventory or STAI or "Life Satisfaction Index for Adolescents" or LSI-A or Quality of Life Evaluation Scale or AUQUEI or Activity Limitations Questionnaire or ACTIVLIM).mp.
14. (sf12 or sf 12 or short form 12 or shortform 12 or sf twelve or sftwelve or shortform twelve or short form twelve).mp.
15. (sf36 or sf 36 or short form 36 or shortform 36 or sf thirtysix or sf thirty six or shortform thirtysix or shortform thirty six or short form thirtysix or short form thirty six).mp.
16. 5 or 6 or 7 or 8 or 9 or 10 or 11 or 12 or 13 or 14 or 15
17. 4 or 16
18. 3 and 17

Embase Stage Two

1. Duchenne muscular dystrophy/
2. duchenne*.mp.
3. 1 or 2
4. Autoquestionnaire Qualite de vie Enfant Image.mp.

5. (Behavior Assessment System for Children or BASC or Parent Form 50 or PF50 or DUX-25 or EuroQoL 5-domain or Functional Independence Measure* or FIM).mp.

6. (WeeFIM or Life Satisfaction Index or LSI or LSIA or Neurological Disorders Quality of Life Questionnaire or NeuroQOL or pediatric NeuroQOL or Offer Self-Image Questionnaire for Adolescents or OSIQ).mp.

7. (Pediatric Outcomes Data Collection Instrument or PODCI or Neuromuscular module or DMD module or Multidimensional Fatigue Scale or Generic Short-Form or SF15).mp.

8. (SDQ or 'Strips of Life with Emoticons Questionnaire' or SOLE or 'World Health Organisation Quality of Life Scale-Brief Version' or WHOQOL-BREF).mp.

9. ('Strength and Difficulties Questionnaire').mp. [mp=title, abstract, heading word, drug trade name, original title, device manufacturer, drug manufacturer, device trade name, keyword, floating subheading word, candidate term word]

10. "Individualized Neuromuscular Quality of Life Questionnaire".mp.

11. (INQOL or Child Activity Limitations Interview or CALI or "Satisfaction with Life Scale" or SWLS).mp.

12. 'Pediatric Quality of Life Inventory'.mp.

13. ("Pediatric Orthopedic Society of North America Pediatric Musculoskeletal Functional Health Questionnaire" or POSNA or Pittsburgh Sleep Quality Index).mp.

14. PedsQL.mp.

15. (SF-36 or EQ-5D*).mp.

16. "World Health Organization Quality of Life".mp.

17. (KIDSCREEN or Pittsburgh Sleep Quality questionnaire or PSQI).mp. [mp=title, abstract, heading word, drug trade name, original title, device manufacturer, drug manufacturer, device trade name, keyword, floating subheading word, candidate term word]

18. "Childrens Assessment of Participation and Enjoyment".mp.

19. (CAPE or "Child Health Questionnaire" or CHQ* or Health Utilities Index Questionnaire or HUI*).mp.

20. (Fatigue Severity Scale or FSS or "Hospital Anxiety and Depression Scale" or HADS or COPE Inventory or "Quality of Life in Neuromuscular Disease").mp.

21. (QoL-NMD DISABKIDS or "Depressionsiventar fur kinder und Jugendliche" or DIKJ or Beck Depression Inventory or BDI or CARE-NMD or State-Trait Anxiety Inventory or STAI or "Life Satisfaction Index for Adolescents" or LSI-A or Quality of Life Evaluation Scale or AUQUEI or Activity Limitations Questionnaire or ACTIVLIM).mp.

22. (sf12 or sf 12 or short form 12 or shortform 12 or sf twelve or sftwelve or shortform twelve or short form twelve).mp.

23. (sf36 or sf 36 or short form 36 or shortform 36 or sf thirtysix or sf thirty six or shortform thirtysix or shortform thirty six or short form thirtysix or short form thirty six).mp.

24. 12 or 14 or 15 or 16 or 17 or 18 or 19 or 20 or 21 or 22 or 23

PsychINFO Stage One

1. Muscular Dystrophy/

2. duchenne*.mp.

3. 1 or 2

4. (HR-PRO or HRPRO or HRQL or HRQoL or QL or QoL).ti,ab. or quality of life.mp. or (health index* or health indices or health profile*).ti,ab. or health status.mp. or ((patient or self or child or parent or carer or proxy) adj (appraisal* or appraised or report or reported or reporting or rated or rating* or based or assessed or assessment*)).ti,ab. or ((disability or function or functional or functions or subjective or utility or utilities or wellbeing or well being) adj2 (index or indices or instrument or instruments or measure or measures or questionnaire* or profile or profiles or scale or scales or score or scores or status or survey or surveys)).ti,ab.

5. 'Pediatric Quality of Life Inventory'.mp.

6. PedsQL.mp.

7. (SF-36 or EQ-5D*).mp.

8. "World Health Organization Quality of Life".mp.

9. (KIDSCREEN or Pittsburgh Sleep Quality questionnaire or PSQI).mp. [mp=title, abstract, heading word, table of contents, key concepts, original title, tests & measures]

10. "Childrens Assessment of Participation and Enjoyment".mp.

11. (CAPE or "Child Health Questionnaire" or CHQ* or Health Utilities Index Questionnaire or HUI*).mp.

12. (Fatigue Severity Scale or FSS or "Hospital Anxiety and Depression Scale" or HADS or COPE Inventory or "Quality of Life in Neuromuscular Disease").mp.

13. (QoL-NMD DISABKIDS or "Depressionsiventar fur kinder und Jugendliche" or DIKJ or Beck Depression Inventory or BDI or CARE-NMD or State-Trait Anxiety Inventory or STAI or "Life Satisfaction Index for Adolescents" or LSI-A or Quality of Life Evaluation Scale or AUQUEI or Activity Limitations Questionnaire or ACTIVLIM).mp.

14. (sf12 or sf 12 or short form 12 or shortform 12 or sf twelve or sftwelve or shortform twelve or short form twelve).mp.

15. (sf36 or sf 36 or short form 36 or shortform 36 or sf thirtysix or sf thirty six or shortform thirtysix or shortform thirty six or short form thirtysix or short form thirty six).mp.

16. 5 or 6 or 7 or 8 or 9 or 10 or 11 or 12 or 13 or 14 or 15

17. 4 or 16

18. 3 and 17

PsycINFO Stage Two

1. Muscular Dystrophy/

2. duchenne*.mp.

3. 1 or 2

4. Autoquestionnaire Qualite de vie Enfant Image.mp.

5. (Behavior Assessment System for Children or BASC or Parent Form 50 or PF50 or DUX-25 or EuroQoL 5-domain or Functional Independence Measure* or FIM).mp.

6. (WeeFIM or Life Satisfaction Index or LSI or LSIA or Neurological Disorders Quality of Life Questionnaire or NeuroQOL or pediatric NeuroQOL or Offer Self-Image Questionnaire for Adolescents or OSIQ).mp.

7. (Pediatric Outcomes Data Collection Instrument or PODCI or Neuromuscular module or DMD module or Multidimensional Fatigue Scale or Generic Short-Form or SF15).mp.

8. (SDQ or 'Strips of Life with Emoticons Questionnaire' or SOLE or 'World Health Organisation Quality of Life Scale-Brief Version' or WHOQOL-BREF).mp.

9. ('Strength and Difficulties Questionnaire').mp. [mp=title, abstract, heading word, table of contents, key concepts, original title, tests & measures]

10. "Individualized Neuromuscular Quality of Life Questionnaire".mp.

11. (INQOL or Child Activity Limitations Interview or CALI or "Satisfaction with Life Scale" or SWLS).mp.

12. 'Pediatric Quality of Life Inventory'.mp.

13. ("Pediatric Orthopedic Society of North America Pediatric Musculoskeletal Functional Health Questionnaire" or POSNA or Pittsburgh Sleep Quality Index).mp.

14. PedsQL.mp.

15. (SF-36 or EQ-5D*).mp.

16. "World Health Organization Quality of Life".mp.

17. (KIDSCREEN or Pittsburgh Sleep Quality questionnaire or PSQI).mp. [mp=title, abstract, heading word, table of contents, key concepts, original title, tests & measures]

18. "Childrens Assessment of Participation and Enjoyment".mp.

19. (CAPE or "Child Health Questionnaire" or CHQ* or Health Utilities Index Questionnaire or HUI*).mp.

20. (Fatigue Severity Scale or FSS or "Hospital Anxiety and Depression Scale" or HADS or COPE Inventory or "Quality of Life in Neuromuscular Disease").mp.

21. (QoL-NMD DISABKIDS or "Depressionsiventar fur kinder und Jugendliche" or DIKJ or Beck Depression Inventory or BDI or CARE-NMD or State-Trait Anxiety Inventory or STAI or "Life Satisfaction Index for Adolescents" or LSI-A or Quality of Life Evaluation Scale or AUQUEI or Activity Limitations Questionnaire or ACTIVLIM).mp.

22. (sf12 or sf 12 or short form 12 or shortform 12 or sf twelve or sftwelve or shortform twelve or short form twelve).mp.

23. (sf36 or sf 36 or short form 36 or shortform 36 or sf thirtysix or sf thirty six or shortform thirtysix or shortform thirty six or short form thirtysix or short form thirty six).mp.

Cochrane Library Stage One
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ID Search
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#3 #1 or #2
#4 (HR-PRO or HRPRO or HRQL or HRQoL or QL or QoL):ti,ab
#5 (quality of life):ti,ab,kw
#6 (health index* or health indices or health profile*):ti,ab,kw
#7 (health status):ti,ab,kw
#8 ((patient or self or child or parent or carer or proxy) near (appraisal* or appraised or report or reported or reporting or rated or rating* or based or assessed or assessment*)):ti,ab
#9 ((disability or function or functional or functions or subjective or utility or utilities or wellbeing or well being) near/2 (index or indices or instrument or instruments or measure or measures or questionnaire* or profile or profiles or scale or scales or score or scores or status or survey or surveys)) .ti,ab
#10 #4 or #5 or #6 or #7 or #8 or #9
#11 ("Pediatric Quality of Life Inventory" or PedsQL or SF-36 or EQ-5D* or "World Health Organization Quality of Life" or WHOQoL or KIDSCREEN or Pittsburgh Sleep Quality questionnaire or PSQI or "Children's Assessment of Participation and Enjoyment" or CAPE or "Child Health

Questionnaire" or CHQ* or Health Utilities Index Questionnaire or HUI* or Fatigue Severity Scale or FSS or "Hospital Anxiety and Depression Scale" or HADS or COPE Inventory or "Quality of Life in Neuromuscular Disease" or QoL-NMD DISABKIDS or "Depressionsiventar fur kinder und Jugendliche" or DIKJ or Beck Depression Inventory or BDI or CARE-NMD or State-Trait Anxiety Inventory or STAI or "Life Satisfaction Index for Adolescents" or LSI-A or Quality of Life Evaluation Scale or AUQUEI or Activity Limitations Questionnaire or ACTIVLIM):ti,ab,kw
#12 (sf12 or sf 12 or short form 12 or shortform 12 or sf twelve or sftwelve or shortform twelve or short form twelve):ti,ab,kw
#13 (sf36 or sf 36 or short form 36 or shortform 36 or sf thirtysix or sf thirty six or shortform thirtysix or shortform thirty six or short form thirtysix or short form thirty six):ti,ab,kw
#14 #11 or #12 or #13
#15 #10 or #14
#16 #3 and #15

Cochrane Library Stage Two
Search Name: Duchenne Stage Two for PP September 2018
Last Saved: 18/09/2018 15:23:27
Comment:

ID Search
#1 MeSH descriptor: [Muscular Dystrophy, Duchenne] this term only
#2 (duchenne*):ti,ab,kw
#3 #1 or #2
#4 (Autoquestionnaire Qualite de vie Enfant Image):ti,ab,kw
#5 ("Behavior Assessment System for Children"):ti,ab,kw
#6 (BASC):ti,ab,kw
#7 (Parent Form 50):ti,ab,kw
#8 (PF50 or DUX-25):ti,ab,kw
#9 ("EuroQoL 5-domain"):ti,ab,kw
#10 (Functional Independence Measure* or FIM):ti,kw,ab
#11 (WeeFIM or Life Satisfaction Index or LSI or LSIA or Neurological Disorders Quality of Life Questionnaire or NeuroQOL or pediatric NeuroQOL or Offer Self-Image Questionnaire for Adolescents or OSIQ):ti,ab,kw
#12 (Pediatric Outcomes Data Collection Instrument or PODCI or Neuromuscular module or DMD module or Multidimensional Fatigue Scale or Generic Short-Form or SF15):ti,ab,kw
#13 (SDQ or 'Strips of Life with Emoticons Questionnaire' or SOLE or 'World Health Organisation Quality of Life Scale-Brief Version' or WHOQOL-BREF):ti,ab,kw
#14 ('Strength and Difficulties Questionnaire'):ti,ab,kw
#15 ("Individualized Neuromuscular Quality of Life Questionnaire"):ti,ab,kw
#16 (INQOL or Child Activity Limitations Interview or CALI or "Satisfaction with Life Scale" or SWLS):ti,ab,kw
#17 ('Pediatric Quality of Life Inventory'):ti,ab,kw
#18 ("Pediatric Orthopedic Society of North America Pediatric Musculoskeletal Functional Health Questionnaire" or POSNA or Pittsburgh Sleep Quality Index):ti,ab,kw
#19 (PedsQL):ti,ab,kw
#20 (SF-36 or EQ-5D*):ti,ab,kw
#21 ("World Health Organization Quality of Life"):ti,ab,kw
#22 (KIDSCREEN or Pittsburgh Sleep Quality questionnaire or PSQI):ti,ab,kw
#23 ("Childrens Assessment of Participation and Enjoyment"):ti,ab,kw
#24 (CAPE or "Child Health Questionnaire" or CHQ* or Health Utilities Index Questionnaire or HUI*):ti,ab,kw

#25 (Fatigue Severity Scale or FSS or "Hospital Anxiety and Depression Scale" or HADS or COPE Inventory or "Quality of Life in Neuromuscular Disease"):ti,ab,kw
#26 (QoL-NMD DISABKIDS or "Depressionsiventar fur kinder und Jugendliche" or DIKJ or Beck Depression Inventory or BDI or CARE-NMD or State-Trait Anxiety Inventory or STAI or "Life Satisfaction Index for Adolescents" or LSI-A or Quality of Life Evaluation Scale or AUQUEI or Activity Limitations Questionnaire or ACTIVLIM):ti,ab,kw
#27 (sf12 or sf 12 or short form 12 or shortform 12 or sf twelve or sftwelve or shortform twelve or short form twelve):ti,ab,kw
#28 (sf36 or sf 36 or short form 36 or shortform 36 or sf thirtysix or sf thirty six or shortform thirtysix or shortform thirty six or short form thirtysix or short form thirty six):ti,ab,kw
#29 #4 or #5 or #6 or #7 or #8 or #9 or #10 or #11 or #12 or #13 or #14 or #15 or #16 or #17 or #18 or #19 or #20 or #21 or #22 or #23 or #24 or #25 or #26 or #27 or #28
#30 #3 and #29
#31 "construct validity" or "content validity" or "criterion validity" or "inter rater reliability" or "interrater reliability"
#32 #30 and #31

CINAHL Stage One

Search ID#	Search Terms	Search Options	Last Run Via	Results
S1	(MH "Muscular Dystrophy, Duchenne")	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	Display
S2	duchenne*	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	Display
S3	S1 OR S2	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	Display
S4	HR-PRO	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen -	Display

			Advanced Search Database - CINAHL with Full Text	
S5	((disability or function or functional or functions or subjective or utility or utilities or wellbeing or well being) N2 (index or indices or instrument or instruments or measure or measures or questionnaire* or profile or profiles or scale or scales or score or scores or status or survey or surveys))	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	Display
S6	HRPRO or HRQL or HRQoL	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	Display
S7	quality of life or health index* or health indices or health profile* or health status	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	Display
S8	TI QL	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	Display
S9	TI QoL	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	Display

S10	((patient or self or child or parent or carer or proxy) and (appraisal* or appraised or report or reported or reporting or rated or rating* or based or assessed or assessment*))	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	Display
S11	S4 OR S5 OR S6 OR S7 OR S8 OR S9 OR S10	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	Display
S12	(Pediatric Quality of Life Inventory or PedsQL or SF-36 or EQ-5D* or World Health Organization Quality of Life or WHOQoL or KIDSCREEN or Pittsburgh Sleep Quality questionnaire or PSQI or Childrens Assessment of Participation and Enjoyment or CAPE or Child Health Questionnaire or CHQ* or Health Utilities Index Questionnaire or HUI* or Fatigue Severity Scale or FSS or Hospital Anxiety and Depression Scale or HADS or COPE Inventory or Quality of Life in Neuromuscular Disease or QoL-NMD DISABKIDS or Depressionsiventar fur kinder und Jugendliche or DIKJ or Beck Depression Inventory or BDI or CARE-NMD or State-Trait Anxiety Inventory or STAI or Life Satisfaction Index for Adolescents or LSI-A or Quality of Life	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	Display

	Evaluation Scale or AUQUEI or Activity Limitations Questionnaire or ACTIVLIM)			
S13	(sf12 or sf 12 or short form 12 or shortform 12 or sf twelve or sftwelve or shortform twelve or short form twelve)	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	Display
S14	(sf36 or sf 36 or short form 36 or shortform 36 or sf thirtysix or sf thirty six or shortform thirtysix or shortform thirty six or short form thirtysix or short form thirty six)	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	Display
S15	S12 OR S13 OR S14	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	Display
S16	S11 OR S15	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	Display
S17	S3 AND S16	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	754

CINAHL Stage Two

Search ID#	Search Terms	Search Options	Last Run Via	Results
S1	(MH "Muscular Dystrophy, Duchenne")	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	1,330
S2	duchenne*	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	1,800
S3	S1 OR S2	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	1,800
S4	Autoquestionnaire Qualite de vie Enfant Image	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	3
S5	(Behavior Assessment System for Children or BASC or Parent Form 50 or PF50 or DUX-25 or EuroQoL 5-domain or Functional Independence Measure* or FIM)	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	3,288
S6	(WeeFIM or Life Satisfaction Index or LSI or LSIA or Neurological Disorders Quality of Life Questionnaire or NeuroQOL or pediatric	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search	559

	NeuroQOL or Offer Self-Image Questionnaire for Adolescents or OSIQ)		Database - CINAHL with Full Text	
S7	(Pediatric Outcomes Data Collection Instrument or PODCI or Neuromuscular module or DMD module or Multidimensional Fatigue Scale or Generic Short-Form or SF15)	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	197
S8	(SDQ or 'Strips of Life with Emoticons Questionnaire' or SOLE or 'World Health Organisation Quality of Life Scale-Brief Version' or WHOQOL-BREF)	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	6,403
S9	('Strength and Difficulties Questionnaire')	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	948
S10	"Individualized Neuromuscular Quality of Life Questionnaire"	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	3
S11	(INQOL or Child Activity Limitations Interview or CALI or "Satisfaction with Life Scale" or SWLS)	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	837
S12	'Pediatric Quality of Life Inventory'	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen -	521

			Advanced Search Database - CINAHL with Full Text	
S13	("Pediatric Orthopedic Society of North America Pediatric Musculoskeletal Functional Health Questionnaire" or POSNA or Pittsburgh Sleep Quality Index)	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	1,548
S14	PedsQL	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	651
S15	(SF-36 or EQ-5D*)	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	16,749
S16	"World Health Organization Quality of Life"	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	623
S17	(KIDSCREEN or Pittsburgh Sleep Quality questionnaire or PSQI)	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	1,040
S18	"Childrens Assessment of Participation and Enjoyment"	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen -	0

			Advanced Search Database - CINAHL with Full Text	
S19	(CAPE or "Child Health Questionnaire" or CHQ* or Health Utilities Index Questionnaire or HUI*)	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	3,751
S20	(Fatigue Severity Scale or FSS or "Hospital Anxiety and Depression Scale" or HADS or COPE Inventory or "Quality of Life in Neuromuscular Disease")	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	530,689
S21	(QoL-NMD DISABKIDS or "Depressionsiventar fur kinder und Jugendliche" or DIKJ or Beck Depression Inventory or BDI or CARE-NMD or State-Trait Anxiety Inventory or STAI or "Life Satisfaction Index for Adolescents" or LSI-A or Quality of Life Evaluation Scale or AUQUEI or Activity Limitations Questionnaire or ACTIVLIM)	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	9,541
S22	(sf12 or sf 12 or short form 12 or shortform 12 or sf twelve or sftwelve or shortform twelve or short form twelve)	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	524
S23	(sf36 or sf 36 or short form 36 or shortform 36 or sf thirtysix or sf thirty six or shortform thirtysix or shortform	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen -	2,253

	thirty six or short form thirtysix or short form thirty six)		Advanced Search Database - CINAHL with Full Text	
S24	S4 OR S5 OR S6 OR S7 OR S8 OR S9 OR S10 OR S11 OR S12 OR S13 OR S14 OR S15 OR S16 OR S17 OR S18 OR S19 OR S20 OR S21 OR S22 OR S23	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	561,269
S25	S3 AND S24	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	250
S26	TX "construct validity" or "content validity" or "criterion validity" or "inter rater reliability" or "interrater reliability"	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	55,290
S27	S25 AND S26	Search modes - Boolean/Phrase	Interface - EBSCOhost Research Databases Search Screen - Advanced Search Database - CINAHL with Full Text	5