Study report

Deliverable 2

EMA/2020/46/TDA, Lot 5 (Pharmacoepidemiological research)

The Danish Medicines Agency, Data Analytics Center (DAC)

"Description and assessment of fitness-for-purpose of real-world data (RWD) sources on Duchenne Muscular Dystrophy for regulatory decision-making"

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Table of contents

Abstract 4
List of abbreviations 5
1. Background and rationale 6
2. Objectives 7
3. Methods 7
3.1. Literature review 7
3.2. Clinical expert assessment of variable list – step 1 9
3.3. Clinical expert assessment of variable list – step 2 workshop 10
4. Results and discussion 11
5. References 12
6. Appendices 13
6.1. List of supportive files 18

Abstract

Duchenne Muscular Dystrophy (DMD) is a severe and rare progressive inherited neuromuscular disorder for which there is currently no cure. Several new types of therapies are in drug development pipeline, or under evaluation by the European Medicines Agency (EMA) or have recently been approved under conditional marketing authorisation. In order to confirm the efficacy and safety of existing and future medicines, there is therefore a need to supplement the clinical trial data with long-term real-world safety and effectiveness evidence to support regulatory decisions.

The objective of this first part of the study was to define a list of core variables necessary to answer regulatory research questions and supplement clinical trial data. We used a two-step approach combining a comprehensive literature review with expert clinical assessment to define a list of core variables with focus on specific key regulatory research question. This core variable list will be used to map the current real-world data (RWD) landscape within DMD, assess the fit-for purpose of such data and identify gaps in current data capturing that can help improve data collection in the future to support the feasibility of answering regulatory research questions.

List of abbreviations

4SC	4-stair climb
6MWT	6-minute walk-test
DMD	Duchenne muscular dystrophy
EMA	European Medicines Agency
FDA	Food and Drug Administration
NCBI	National Center for Biotechnology Information
NSAA	North Star Ambulatory Assessment
PMDA	Pharmaceuticals and Medical Devices Agency
PROM	Patient-reported outcome measures
RCFM	The Danish Rehabilitation Centre for Neuromuscular Diseases
RWD	Real-world data
RWE	Real-world evidence
SOC	Standard of care
TREAT-NMD	Translational Research in Europe for the Assessment and Treatment for Neuromuscular Disorders

1. Background and rationale

Duchenne muscular dystrophy (DMD) is a severe and rare progressive inherited neuromuscular disorder, leading to loss of ambulation and premature mortality (Duan et al., 2021). DMD is inherited in an X-linked recessive manner and therefore primarily affects boys. There is currently no medical cure for DMD; instead, treatments focus on managing symptoms and improving quality of life. Recently, newer therapies such as gene therapy, antisense oligonucleotides and myostatin inhibitors have received restricted approval through the Accelerated Approval pathway (FDA), Conditional Early Approval System (PMDA) or as Conditional Marketing Authorization (EMA). However, uncertainty remains regarding their long-term safety and effectiveness - particularly in real-world settings and across the full disease trajectory. As such, there is a growing need to supplement clinical trial data with real-world evidence (RWE) to support regulatory decisions, including those related to marketing authorisations and post-authorisation requirements.

As a first step towards conducting a RWD study, identification and assessment of the quality and fit-for-purpose of data sources is essential. As part of the fit-for-purpose assessment of data sources, it is key to identify which variables are needed to be assessed. For this purpose, EMA has listed four research questions that the variables should be able to cover:

1. How has the <critical clinical outcome, e.g. NSAA total score, 6MWT, 4SC, respiratory function tests, Stride Velocity 95th centile, etc.> evolved from the patient's date of birth or from the relevant index date (e.g. as defined by age at symptoms onset, date of initial diagnosis) over the disease lifespan? The tenderer should describe data availability so that the results to the above question can be stratified by:
    - genotype
    - disease severity (ambulant patients vs non-ambulant patients)
    - proposed indications in marketing authorisation applications
    - DMD treatments (considering changes over time and standalone treatment vs combination)
    - age at disease onset / at diagnosis

2. How has loss of ambulation in DMD patients evolved over time? Is there a difference depending on genotypes; depending on evolving standard of care (SOC) (e.g., steroids, ACE inhibitors and/or beta inhibitors to maintain cardiac functions, and/or other disease management options such as physiotherapy and other care measures reported by caregivers); depending on proposed indications in marketing authorisation applications; depending on ongoing patient's treatment as well as past treatments (distinguishing between standalone combinations), and depending on age at disease onset or baseline?

3. What is the impact of the evolving clinical guidelines on disease management, i.e., on the choice of SOC as well as other types of care such as physiotherapy and other care measures reported by caregivers?

4. Is there an increase in the occurrence of important identified or potential risks observed over time in DMD treated patients? The tenderer should describe data availability so that the results to the above question can be stratified by:
    - genotype
    - disease severity (ambulant patients vs non-ambulant patients)

- proposed indications in marketing authorisation applications
- DMD treatments (considering changes over time and standalone treatment vs combination)
- age at disease onset / at diagnosis

2. Objectives

To identify a list of core variables to be used for future RWD studies in DMD patients with focus on the four regulatory research questions provided by EMA.

3. Methods

Our main strategy was

1. to identify variables that are commonly used for regulatory purposes for assessment of efficacy and/or safety of pharmaceutical products in DMD patients
2. to select variables that are collected in clinical practice
3. to validate the variables by clinical experts in the DMD field

3.1. Literature review

Initially, a literature review was conducted with the aim of collecting variables used in clinical trials as either inclusion- or exclusion criteria, stratification or endpoints related to clinical efficacy and safety (i.e., not biological efficacy and safety). Variables related to tolerability, pharmacokinetic /pharmacodynamics and biological efficacy (i.e., exon skipping and dystrophin production) were not included. For inclusion- and exclusion criteria, we focused on key variables as the level of information required in clinical trials is not compatible with information collected in a real-world setting. From regulatory documents, variables related to indications were also collected.

In Figure 1, an illustration of the literature review is shown. In terms of sources, PubMed (https://pubmed.ncbi.nlm.nih.gov/) was used to search for peer-reviewed published clinical trials, whereas official regulatory websites where searched for regulatory assessment documents encompassing results from clinical trials (FDA: https://www.fda.gov/ ; EMA: https://www.ema.europa.eu/en/homepage ; PMDA: https://www.pmda.go.jp/english/). The following filters were added to the initial NCBI search (see "Records marked as ineligible by automation tools" in Figure 1):

- ✓ Published within 10 years
- ✓ Full text
- ✓ Clinical trial
- ✓ Clinical trial, phase II
- ✓ Clinical trial, phase III
- ✓ Clinical trial, phase IV
- ✓ Controlled clinical trial
- ✓ Multicenter study
- ✓ Randomized controlled trial

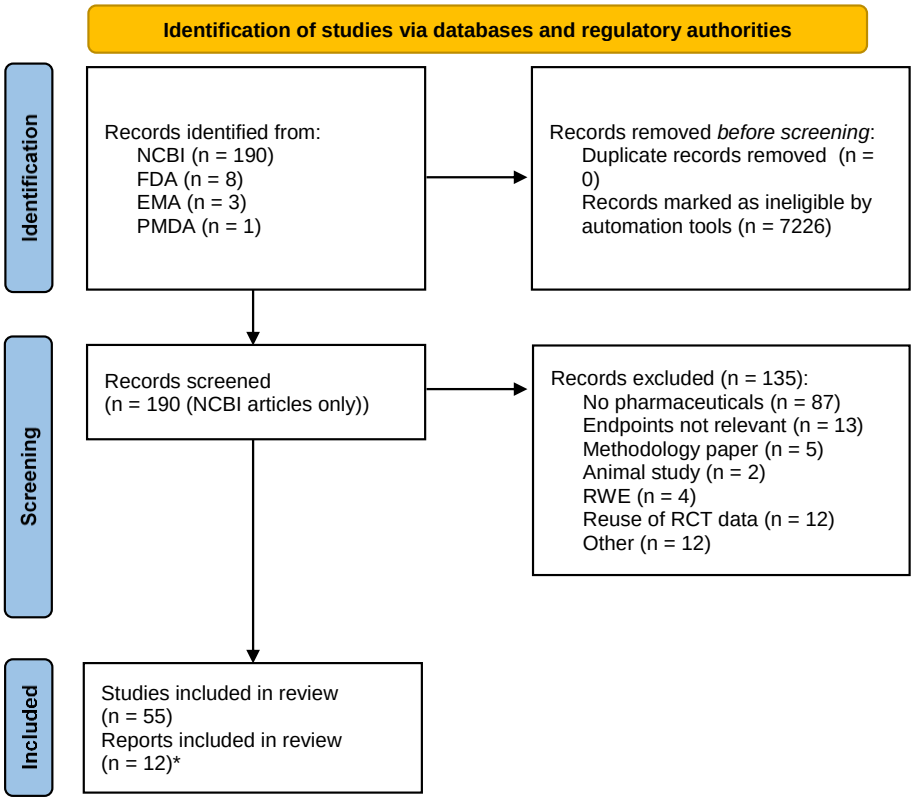

Figure 1. PRISMA 2020 flow-chart of search for published clinical trials and regulatory documents on approved pharmaceutical products for DMD patients. Notes: Documents from regulatory authorities includes several studies each of which may also be included in the studies from NCBI. We did not try to check for duplicates as the results from regulatory documents may differ slightly from those published as peer-reviewed papers. Also, the titles of studies differed which made identification of duplicates difficult. Based on (Page et al., 2021).

The pharmaceutical products assessed for market authorization purpose in DMD are listed in Table 1.

Table 1. List of DMD-specific pharmaceuticals assessed for market authorization used in the present study.

Brand name	Ingredient	Assessed in country	Year approval	Mode of action	Regulatory documents retrieved
Elevidys	Delandistrogene moxeparvovec-rokl	US	2024	AAV-based gene-therapy	FDA
Agamree	Vamorolone	US, EU	2023	Synthetic corticosteroid	EMA, FDA
Duvyzat	Givinostat	FDA, EU	2024, 2025	Oral HDAC-inhibitor	EMA, FDA
Emflaza and more	Deflazacort	US	2017	Glucocorticoid	FDA

Exondys 51	Eteplirsen	US	2016	Mutation specific antisense therapy	FDA
Amondys 45	Casimersen	US	2021	Mutation specific antisense therapy	FDA
Vyondys 53	Golodirsen	US	2019	Mutation specific antisense therapy	FDA
Viltepso	Viltolarsen	US, Japan	2020	Mutation specific antisense therapy	PMDA, FDA
Translarna	Ataluren	EU	2014	Targets non-sense mutations	EMA

Notes: Viltepso is also approved in Russia, and Translarna is approved in Russia and Brazil. However, we were not been able to retrieve documentations for these approvals.

A total of 94 variables were identified in the literature review as illustrated in Supplementary Table 1.

We also examined the published articles from the consensus program on care considerations in DMD disease (Birnkrant et al., 2018b, 2018a, 2018c) for variables recommended to regularly assess in clinical practice. Variables recommended to assess in clinical practice is documented in File 1 (see next section) along with recommended frequency of assessment.

We categorised all variables into 13 sub-categories in line with TREAT-NMD harmonized data set structure (https://www.treat-nmd.org/what-we-do/core-datasets/dmd/):

1. Demographics
2. Living status
3. Diagnosis
4. Clinical observations
5. Bone
6. Motor function
7. Wheelchair use
8. Pulmonary function
9. Cardiac function
10. Therapies and medication
11. Hospitalisations and comorbidities
12. Clinical research
13. PROM and cognition

Also, some variables within the same category were combined (in line with the TREAT-NMD structure) including variables describing motor ability and patient-reported outcome measures (PROM).

3.2. Clinical expert assessment of variable list – step 1

Clinical experts from The Danish Rehabilitation Centre for Neuromuscular Diseases (RCFM) scrutinized the variable list for relevance. Variables that are not routinely collected in clinical practice were removed from the list and variables were generally refined to reflect how data are entered in a real-world setting. As most clinical trials include ambulatory patients early in their disease course, including variables used for non-

ambulatory patients were also emphasised. Although the two experts are mostly familiar with Danish practice of collecting data in DMD disease, international non-Danish practice was also considered.

In Supplementary Table 1, reasons for exclusion from the list as well as other relevant comments are documented and the final list of variables is shown in File 1. Three columns were added with indication of whether the variable in question was used in clinical trials (i.e., based on the literature review), whether it was mentioned in the consensus program and/or included in the TREAT-NMD data set. Finally, four columns were added upon request from EMA – one for each regulatory research question proposed as example – to indicate whether the variable in question is needed for the specific research question.

3.3. Clinical expert assessment of variable list – step 2 workshop

A modified list of suggested variables in Word format (File 2) was sent to EMA experts four weeks prior to the workshop on December 17. The aim was to collect comments from the experts prior to the workshop with the goal of having focused discussions.

In File 3, all comments received by email prior to the workshop are documented. Many comments described in detail how the variables should be recorded, and many additional variables were suggested. These comments will be used in next steps of the project, to ensure that future data collected in real-world data sources can effectively support regulatory needs while advancing patient care and treatment development.

For the workshop and in an attempt to stay focused on the four research questions, each question was rephrased to these more specific research questions (see also File 4):

1. *What is the median change from <date of birth /disease onset/date of diagnosis> in <NSAA total score, 6MWT, 4SC, respiratory function tests, Stride Velocity 95th centile> in DMD children, stratified by age at disease onset/at diagnosis, genotype, disease severity (measured by xxx), and DMD treatment?*

2. *What is the time to loss of ambulation as measured by NSAA score (NSAA score < XX value) from disease onset/from diagnosis in DMD children, stratified by age at disease onset/at diagnosis, genotype, disease severity (measured by xxx), and DMD treatment?*

3. *What are treatment choices and standard of care for DMD children over the last xx years, stratified by age at disease onset/at diagnosis, genotype, disease severity (measured by xxx), country*

4. *What is the incidence of <safety events/outcomes> (to be listed) in DMD patients following treatment initiation, stratified by treatment, age at diagnosis/disease onset, genotype?*

Additionally, variables were allocated to the four categories for each research question (see File 4):

1. Primary clinical outcomes (longitudinal)
2. Key stratification variables (to ensure clinically interpretable subgroup analyses)
3. Safety and risk variables (pharmacovigilance relevant)
4. Care and standard of care (SOC) context (co interventions and management)

File 4 was used at the workshop as point of discussion. A few additional variables were added to the list during the workshop. However, the main discussions revolved around the need for standardization of variable definition and data collection methods, and the opportunity of using this project to identify data

gaps to drive improvements in future data collection practices. Also, refinements of the research questions were made. Minutes from the workshop as well as updated research questions and variable list is documented in File 5 and File 6, respectively.

4. Results and discussion

The final core variable list for the fit-for-purpose assessment includes variables that are both scientifically and clinically relevant to collect in real-world data sources, particularly disease registries. A “question-led” approach was used to identify variables of interest enabling selection of variables ensuring focus on the regulatory research question of interest.

Limitations of the study include the lack of consensus on standardized definitions and collection methods. Definitions of key milestones like loss of ambulation differ in clinical practice and between registries and trials. Some use functional tests while others rely on patient or caregiver reports. Even when the same assessment tools are used, their implementation and interpretation may vary. Despite ambitions for standardization, clinical practice varies considerably across countries, healthcare systems, and even individual centers, creating significant challenges for data comparability.

In the workshop discussion with experts, concerns about narrowing the scope too early, advocating for broad data collection and later refinement were raised, while others emphasized the need to balance ambition with feasibility and data quality. The consensus emerged that the project should adopt a forward-looking approach. While acknowledging current limitations and shortcomings in data collection, the focus should be on defining ideal data elements needed to address key questions. This "question-led" rather than "data-led" approach ensures that the exercise will identify gaps and drive improvements in future data collection practices. These gaps will be explicitly highlighted to influence collection of missing information, potentially through discussions at the planned end-of-project multi-stakeholder workshop.

In conclusion, this part of the project has established a vision of what variables are ideally needed to address key regulatory research questions, ensuring the next steps of the project. These include an assessment of what data is currently available, the identification of data gaps, limitations in feasibility, and opportunities for improvement on future data collection within DMD.

5. References

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6. Appendices

Supplementary Table 1. List of all variables identified through literature review.

Variable	Description	Included in draft variable list (yes/no)	Comments
100MTT/100MWR	100-Meter Timed Test / 100-meter Walk/Run	No	Not used in clinical practice
10MWT/TTRW	10-Metre Walk Test / Time to run/walk 10 m	Yes	
2MWT	2-Minute Walk Test	No	Not specifically included, but part of NSAA under "Motor function"
4SC/TTCLIMB	4-stair climb	Yes	
6MWT	6-Minute Walk Test	Yes	
9-hole peg test		No	Not DMD specific, not used in clinical practice
ACTH stimulation test/Synachten	Adrenocorticotropic hormone stimulation test	No	RCT specific, not regularly assessed in clinical practice
ADL	Activities of Daily Living by Barthel Index Total Score	No	Not DMD specific
Adverse events	Adverse events (any)	Yes	As part of SAE or comorbidities under "Hospitalisations comorbidities"
Age		Yes	
Age at onset		Yes	Age and date of onset combined
Ambulatory status		Yes	
Ankle range of motion		Yes	As part of MFM under "Motor function"
Antibody titers		No	RCT specific, not relevant for RWE
Any surgery		Yes	Included under "Bone" (scoliosis surgery) and "Hospitalisations comorbidities" (reason for hospitalisation)
Assisted ventilation	Any type	Yes	
BMC/BMD	Bone Mineral Content/Bone Mineral Density	Yes	
Brooke	Brooke Scale of Upper Extremity Function	Yes	
Cardiac imaging	Any type (incl. Echocardiogram, MRI)	Yes	

Cardiac treatment		Yes	
Chemistry lab test results		No	RCT specific, not feasible for RWE
Chest imaging	Check for tuberculosis	No	RCT specific, not regularly tested in clinical practice
Clinical trial participation		Yes	
Cognitive function	Any test	Yes	
Comorbidities	Incl. contraindications	Yes	
Corticosteroid dosage		Yes	
Corticosteroid usage		Yes	
Death		Yes	
Descend 4 stairs		Yes	As part of NSAA under "Motor function"
DEXA scan		Yes	Included under "Bone" Bone mineral density
DMD	Duchenne Muscular Dystrophy	Yes	
DMT status/usage	Incl other DMD drugs	Yes	
ECG/QT interval	Electrocardiogram	Yes	
EK2	Egen Klassifikation Scale version 2	Yes	
Falls		No	Unless patient is hospitalised then recorded under "Hospitalisations comorbidities" (reason for hospitalisation)
Fractures		Yes	
FVC	Forced vital capacity (all types)	Yes	
Genetic abnormalities		Yes	If recorded as comorbidity under "Hospitalisations comorbidities"
Genetic confirmation		Yes	
Genotype		Yes	
Get up from chair		Yes	
GMSS	Bayley-III Gross Motor scale	No	Not DMD specific
Height		Yes	

Hemotology lab test results		No	RCT specific, not feasible for RWE
Injury		Yes	As part of Hospitalisation under "Hospitalisations comorbidities" (reason for hospitalisation) - require hospitalisation though
Life expectancy		No	RCT specific, not recorded in clinical practice
LVEF	Left ventricular ejection fraction	Yes	
MDFRS score	muscular dystrophy-specific functional rating scale	No	Not DMD specific
Medication/treatment history		Yes	
MFM-32	Motor Function Measurement (all types)	Yes	
MMT	muscle score	Yes	As part of QMT and/or MFM under "Motor function"
Muscle biopsy measures	All tests (MFA%, MFAF, CSA etc) by MRI scan	No	Not suitable for RWE
Muscle imaging (MRI)	Fat fraction incl VLFF (vastus lateralis fat fraction)	No	Not suitable for RWE
Mutation in the *DMD* gene	For some, specific mutation (nonsense, exon 45 skipping etc)	Yes	
MyoSet tool	Moviplate 30 second finger tapping score, key pinch strength, hand grip strength (i.e., upper limb strength measurements)	Yes	As part of QMT under "Motor function"
Neutralizing antibodies		No	RCT specific, not regularly assessed in clinical practice
NSAA	North Star Ambulatory Assessment	Yes	
Ophthalmology assessment		No	Not regularly assessed in clinical practice according to consensus guidelines
PARS–III	Personal Adjustment and Role Skills Scale, 3rd edition	Yes	As part of Cognition/mental health under "PROM and cognition"
PCF	Peak cough flow	Yes	As part of Lung function under "Pulmonary function"

Peak torque	the maximum rotational or twisting force that a motor, engine, or muscle can generate for a short period	Yes	As part of QMT and/or MFM under "Motor function"
PedsQL	Pediatric Quality of Life Inventory	Yes	As part of Patient-reported outcome measure (PROM) under "PROM and cognition"
PFT	Pulmonary function tests	Yes	As part of Lung function under "Pulmonary function"
Physiotherapy		Yes	
PODCI	Pediatric Outcomes Data Collection Instrument	Yes	As part of Patient-reported outcome measure (PROM) under "PROM and cognition"
PROMIS Mobility and Upper Extremity		Yes	As part of Patient-reported outcome measure (PROM) under "PROM and cognition"
PUL (all)	Performance of Upper Limb (all types)	Yes	
QMT	Quantitative muscle test. Incl. elbow flexors, knee extensors, range of motion by use of myometer or dynamometer	Yes	
Race		Yes	
Respiratory muscle function	Incl. percent predicted maximal inspiratory pressure (MIP %p), percent predicted maximal expiratory pressure (MEP %p)	Yes	As part of Lung function under "Pulmonary function"
Respiratory support	Any type of ventilation	Yes	
RFF/TTR	Time to rise from floor	Yes	As part of NSAA under "Motor function"
Rise from floor without assistance		Yes	As part of NSAA under "Motor function"
SAM	StepWatch Activity Monitor	No	Not DMD specific, not used in clinical practice
Sex		Yes	
Spinal surgery	Any type (scoliosis, fracture, non-fracture etc)	Yes	Under Scoliosis surgery under "Bone" and/or Hospitalisation under "Hospitalisations comorbidities" (reason for hospitalisation)
Stand without support	For instance used as Time to loss of standing	Yes	As part of NSAA under "Motor function"
Surgery		Yes	As part of Hospitalisation under "Hospitalisations comorbidities" (reason for hospitalisation)

SV95C	Stride Velocity 95th Centile	No	No regularly used in clinical practice
Tanner staging		Yes	As part of Pubertal status under "Clinical observations"
TFT	Timed Function Tests	Yes	
Time spend in wheelchair		Yes	
Time to stand from supine		Yes	As part of NSAA under "Motor function"
Time to wheelchair		Yes	
Treatment dose	Of investigated treatment	Yes	
Treatment history/concurrent medication	Treatments beyond those mentioned in the DMD data set	Yes	
TSQM	Treatment Satisfaction Questionnaire	No	RCT specific, not used in clinical practice
TTSTAND	Time to stand from supine	Yes	As part of NSAA under "Motor function"
TU&G	Timed ‘‘up & go’’ test	Yes	
Vaccination history		Yes	
Vital signs	Bp, HR, Resp, Temp etc	No	RCT specific, not consistently used in clinical practice
Vitamin D deficiency		No	Not feasible for RWE (is also not included in TREAT-NMD core data set)
Walk X metres without assistance	Various lengths are used	Yes	As part of NSAA under "Motor function"
Weight		Yes	

6.1. List of supportive files

File 1. List of core variables for DMD RWE 19.01.2026 (final version).

List of core variable list for DMD RWE_1

File 2. Variable list 14.11.2025 (Variable list sent to EMA experts for commenting prior to workshop).

Variable list 14.11.2025.docx

File 3. DMD variable list comments from experts compiled stratified (Initial draft variable list with expert comments).

DMD variable list comments from expe

File 4. DMD research question and variable list_workshop-final 17.12.2025 (Research questions and variable list used for the workshop).

DMD research questions and varial

File 5. Workshop meeting minutes (Meeting minutes with comments from experts)

Workshop meeting minutes_final.docx

File 6. DMD research question and variable list for review_26.01.2026 (Research questions and variable list used for the workshop and reviewed by experts).

DMD research questions and varial