

# INTRODUCTION TO



# PATIENT REPORTED OUTCOMES

Open  
Academy

Master  
class



May  
2023





**Dagmara Kulis**  
**EORTC**



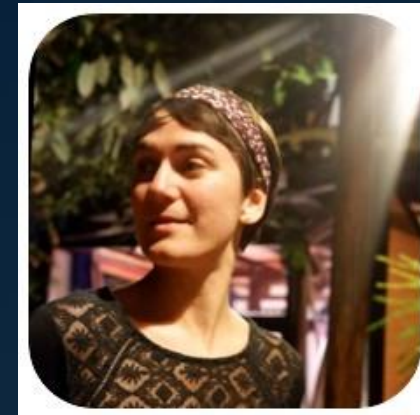
**Elizabeth Vroom**  
**Duchenne Parent Project**



**Mariangela Pellegrini**  
**ERN EuroBloodnet**



**Gavin McDonough**  
**Orphanet**



**Céline Desvignes-Gleizes**  
**MAPI Research Trust**



## Agenda

**European Organisation for  
Research and Treatment of Cancer  
(EORTC)**

**Duchenne Parent Project**

**(15min break)**

**European Rare Disease Research  
Coordination and Support Action  
consortium (ERICA)**

**Final Q&A**

# EURODIS MASTERCLASS

Dagmara Kuliś  
Quality of Life Department  
European Organisation for Research and Treatment of Cancer



## PRO

Patient-reported outcome

An outcome reported directly by patients themselves and not interpreted by an observer

## PROM

Patient-reported outcome measure

Tool used to assess a PRO

# A need to assess QOL (1)

According to the World Health Organization (WHO), the main goals of cancer diagnosis and treatment programmes are to:

- cure **Progression free survival (PFS)**
- considerably prolong the life of patients **Overall Survival (OS)**
- ensure the best possible quality of life for cancer survivors.  
**Health-related quality of life (HRQOL)**

# A need to assess QOL (2)

- Clinicians, nurses, palliative care doctors, psycho-oncologists, researchers recognised the need to ask patients about their QOL in a standardised way.
- 1980: Formation of the EORTC Quality of Life Group network
- 1988: Published strategy on how to assess QOL
  - Core cancer questionnaire + disease-specific questionnaire
- 1993: Guidelines on how to develop quality of life questionnaires



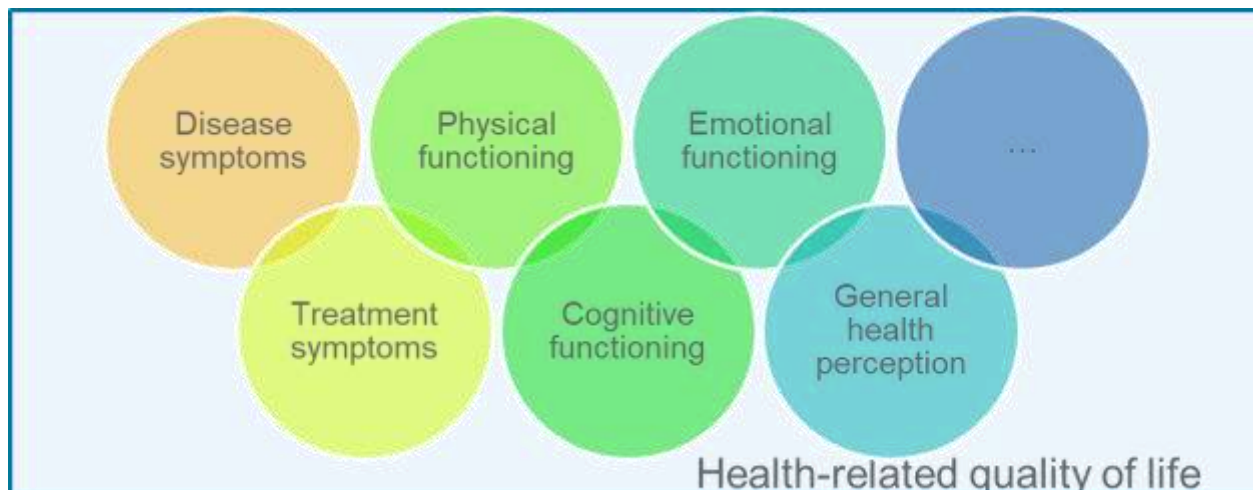
# An accepted working definition of HRQOL

Subjective

=Patient-reported

Multi-dimensional

Dynamic



But... how do you measure these various health-related quality of life aspects?



# Physical functioning

1. Do you have any trouble doing strenuous activities, like carrying a heavy shopping bag or a suitcase?
2. Do you have any trouble taking a long walk?
3. Do you have any trouble taking a short walk outside of the house?
4. Do you need to stay in bed or a chair during the day?
5. Do you need help with eating, dressing, washing yourself or using the toilet?

Not at All	A Little	Quite a Bit	Very Much
1	2	3	4
1	2	3	4
1	2	3	4
1	2	3	4
1	2	3	4



Person A

1. Do you have any trouble doing strenuous activities, like carrying a heavy shopping bag or a suitcase?
2. Do you have any trouble taking a long walk?
3. Do you have any trouble taking a short walk outside of the house?
4. Do you need to stay in bed or a chair during the day?
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Not at All	A Little	Quite a Bit	Very Much
1	2	3	4
1	2	3	4
1	2	3	4
1	2	3	4
1	2	3	4



Person B

Between groups of people

# Physical functioning

	Not at All	A Little	Quite a Bit	Very Much
1. Do you have any trouble doing strenuous activities, like carrying a heavy shopping bag or a suitcase?	1	2	3	4
2. Do you have any trouble taking a <u>long</u> walk?	1	2	3	4
3. Do you have any trouble taking a <u>short</u> walk outside of the house?	1	2	3	4
4. Do you need to stay in bed or a chair during the day?	1	2	3	4
5. Do you need help with eating, dressing, washing yourself or using the toilet?	1	2	3	4



At cancer diagnosis

	Not at All	A Little	Quite a Bit	Very Much
1. Do you have any trouble doing strenuous activities, like carrying a heavy shopping bag or a suitcase?	1	2	3	4
2. Do you have any trouble taking a <u>long</u> walk?	1	2	3	4
3. Do you have any trouble taking a <u>short</u> walk outside of the house?	1	2	3	4
4. Do you need to stay in bed or a chair during the day?	1	2	3	4
5. Do you need help with eating, dressing, washing yourself or using the toilet?	1	2	3	4



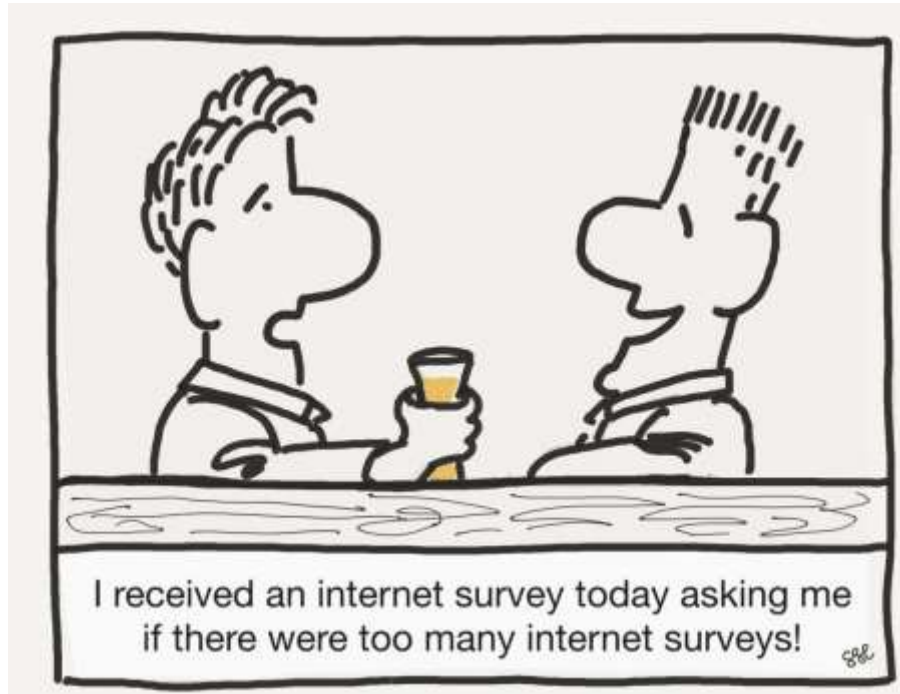
Month 3 on treatment

	Not at All	A Little	Quite a Bit	Very Much
1. Do you have any trouble doing strenuous activities, like carrying a heavy shopping bag or a suitcase?	1	2	3	4
2. Do you have any trouble taking a <u>long</u> walk?	1	2	3	4
3. Do you have any trouble taking a <u>short</u> walk outside of the house?	1	2	3	4
4. Do you need to stay in bed or a chair during the day?	1	2	3	4
5. Do you need help with eating, dressing, washing yourself or using the toilet?	1	2	3	4



At end of treatment

**Across time**



How to get a good PROM that measures what it is supposed to measure?

# EORTC questionnaires

## Disease-specific module

Breast, Lung, Head and Neck...



## Population-specific module

Elderly, AYA...

## Symptom-specific module

Fatigue, Cachexia, CIPN...

## Other

Communication, Information, Spiritual  
Well-Being...

# Item Library

157 questionnaires, 1028 questions

Include custom questionnaires from the community

Official questionnaires

Custom questionnaires

Item classification



+Add questionnaire

A



Anal  
module  
(28 questions)

B



Bladder



Bone Metastases

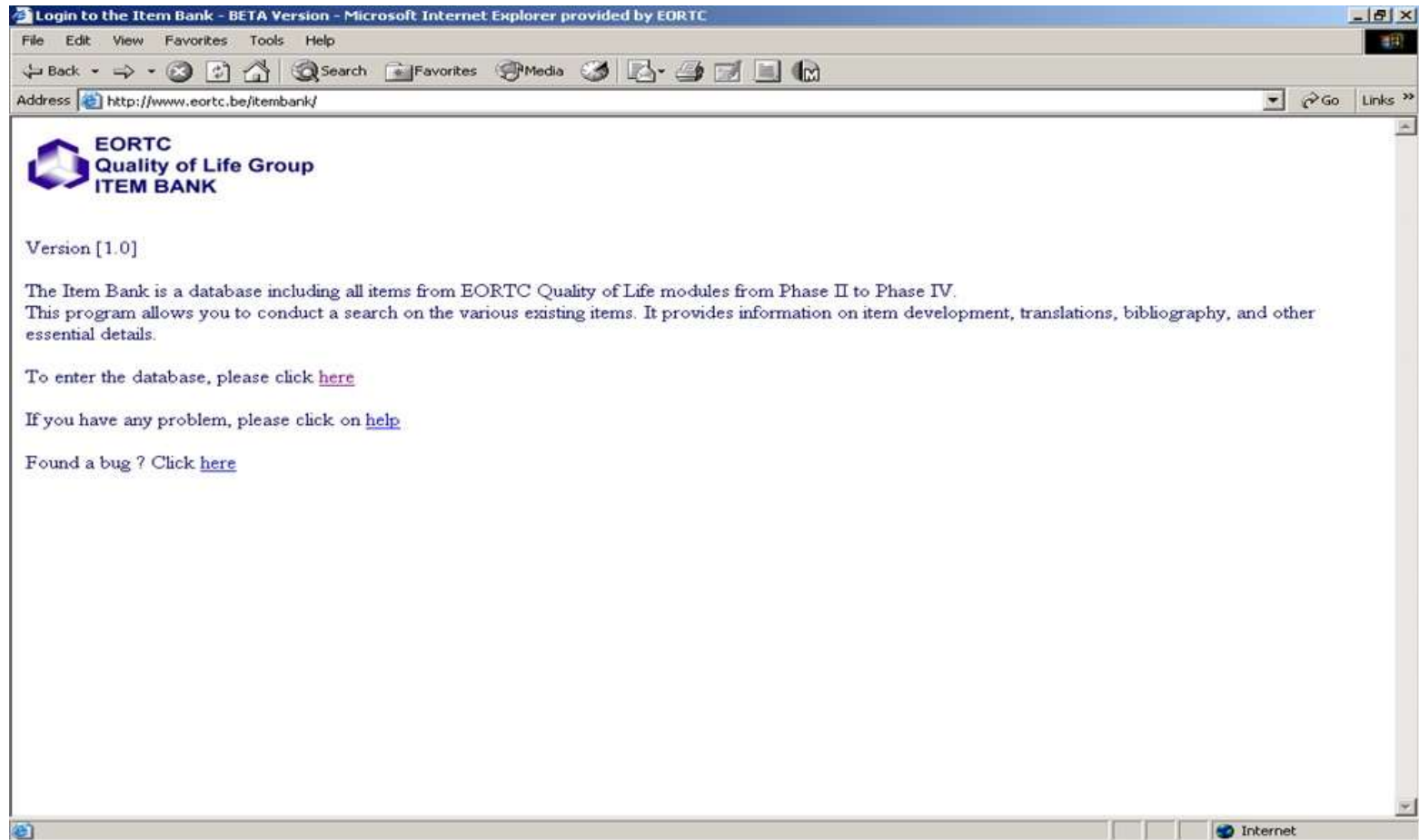
## Welcome to the Item Library!

The **Core questionnaire** QLQ-C30 has been developed to assess the quality of life of cancer patients.

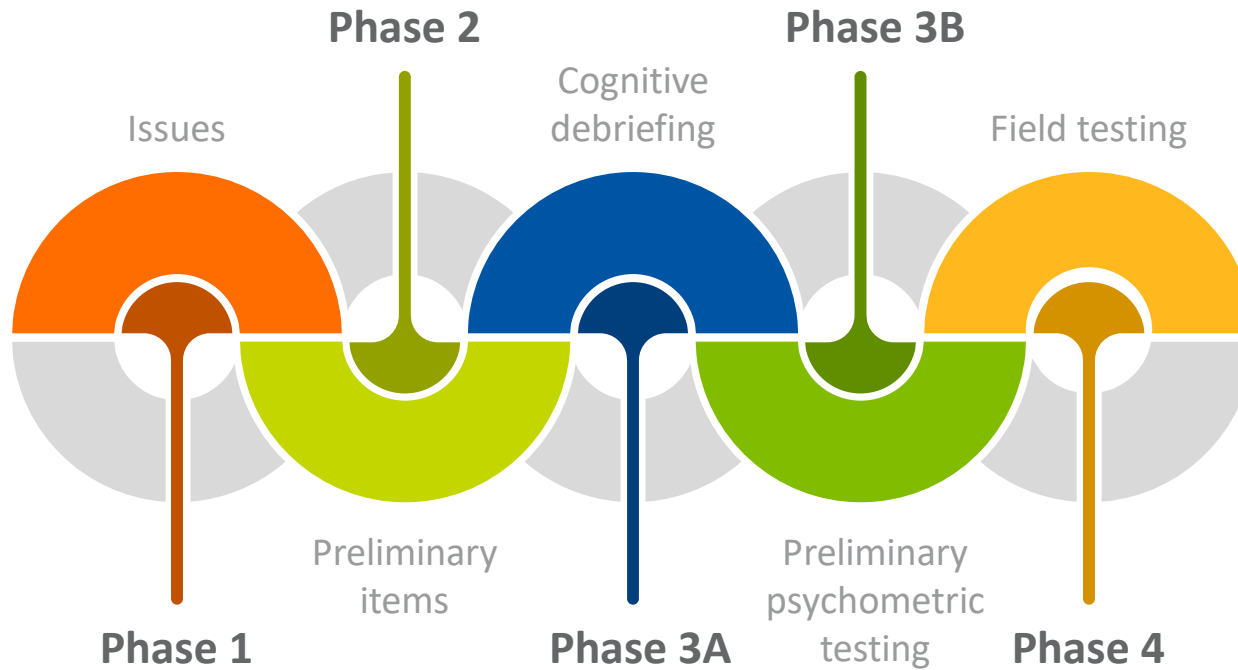
It is supplemented by disease-, symptom- and population-specific questionnaires called **modules**. You can also find general questionnaires that can be used without the Core questionnaire, called **standalones**.

**Previous versions** are questionnaires that have been updated.

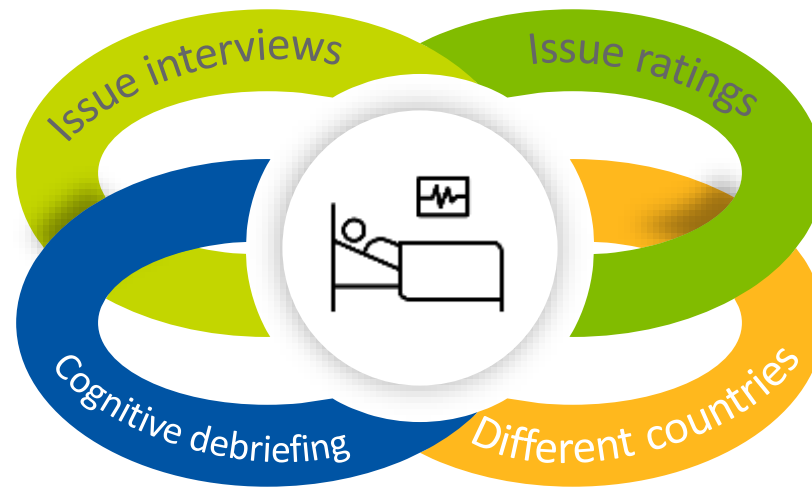
# How it started



# Module development



# Patient-centredness





# Issues



# Issues

1	employment
2	being judged
3	being a burden to others
4	social support from families and friends
5	social support from health care providers
6	sudden attacks of tiredness
7	mentally exhausted
8	physically exhausted
9	problems with weight gain
10	problems with weight loss
11	pain in the throat
12	discomfort in the neck
13	headaches

14	muscle pain
15	pain in the joints
16	bone pain
17	coughing
18	difficulty breathing
19	altered taste
20	appetite changes
21	chewing
22	problems with gum
23	problems with teeth
24	speech problems
25	hoarseness
26	voice changes

# Issue → Item



# Issue



## Presence

Have you had fevers?  
Yes / No; Not at all – A little –  
Quite a bit – Very much

## Severity

Have you choked when  
swallowing?  
Not at all – A little – Quite a bit –  
Very much

## Burden


How much has your treatment been  
a burden to you?  
Not at all – A little – Quite a bit –  
Very much

🔍 swallowing


Include custom questionnaires from the community

## Search results for "swallowing"


Add 0 selected to questionnaire

-  **choked when swallowing**  
Have you choked when swallowing?  
in Full Item List, Head & Neck, Head & Neck, and 7 others...


---

-  **problem swallowing**  
Have you had problems swallowing?  
in Cachexia, Eating difficulties during Chemotherapy/Immunotherapy, Full Item List, and 10 others...


---

-  **problem swallowing liquid**  
Have you had problems swallowing liquids?  
in Full Item List, Head & Neck, Head & Neck, and 4 others...


---

-  **problem swallow solid food**  
Have you had problems swallowing solid foods?  
in Full Item List, Head & Neck, Head & Neck, and 4 others...


---

-  **problem pureed food**  
Have you had problems swallowing pureed food?  
in Full Item List, Head & Neck, Head & Neck, and 3 others...

---

-  **problem swallow saliva**  
Have you had trouble with swallowing your saliva?  
in Full Item List, Oesophagael, Oesophago-Gastric

---

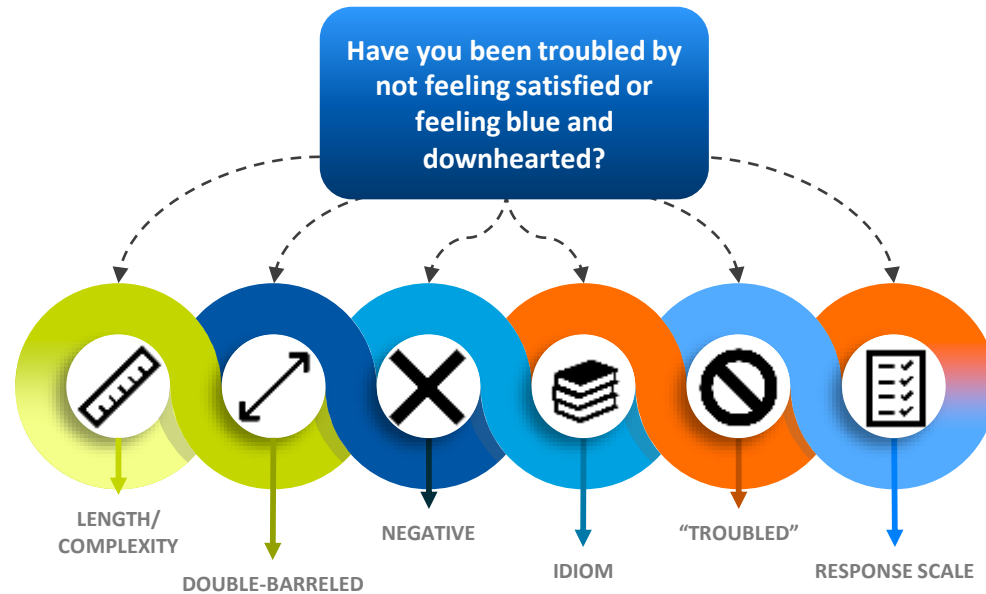
-  **problem eating solid food**  
Have you had problems eating solid foods?  
in Full Item List, IL25 - Gastric Cancer Module, Oesophago-Gastric, and 3 others...

### Filters

- Condition (0)
- Instruction (0)
- Question (7)
- Questionnaire (0)
- Response scale (0)
- Domain scale (2)
- Time scale (0)



## Translatability Assessment



# Translation process



# Pilot-testing

**Pilot-Testing:**

**Sample of a patient response sheet <sup>(1)</sup>  
(completed by interviewer)**

Question number \_\_\_\_\_

**Comments**

a. Difficulty? Yes

---

---

b. Confusing? Yes

---

---

c. Difficult words? Yes

---

---

d. Upsetting? Yes

---

---

e. How would you ask this question?

---

---

---



# Results – changes

Ok, we can reword it.

I do not understand this question. What is it about?

We can add examples of these in parentheses, so that it says “household tasks (such as cleaning or doing laundry)”.

I do not know what you mean with “household tasks”.

This item makes no sense. I do not worry about it.

We will delete the item as not relevant.

I do not worry about it either.

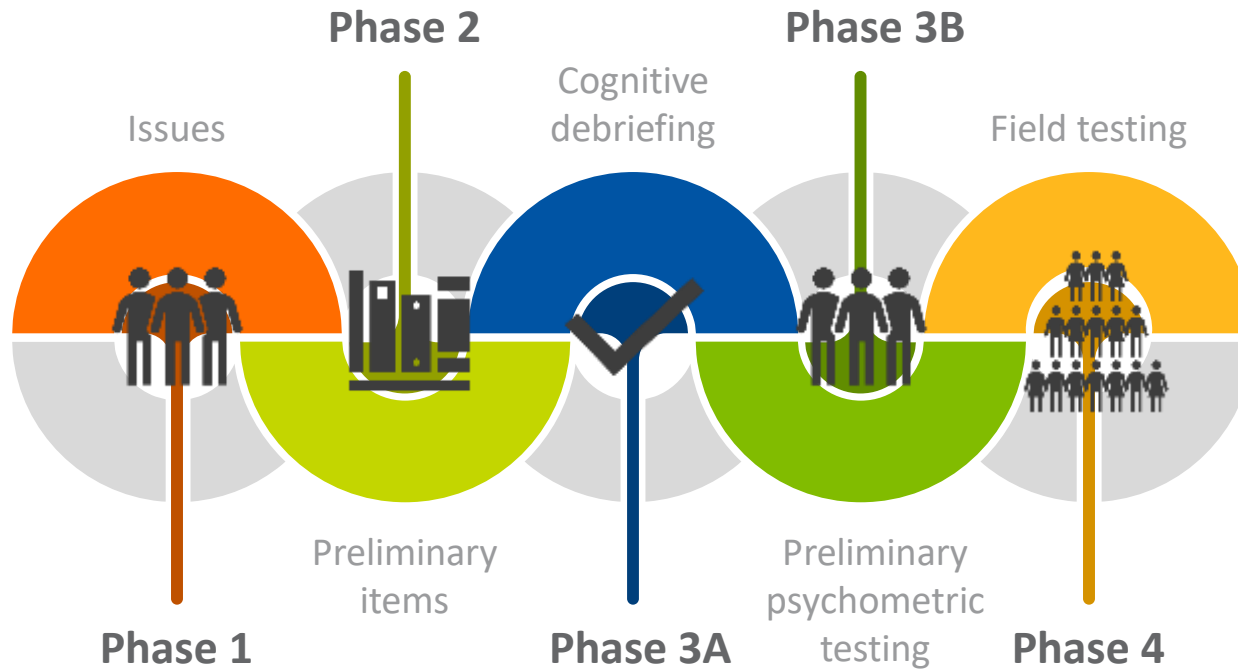
# Initial psychometric testing and changes



# Field testing



Can I use the Item Library to develop  
a PROM in an easier/faster way?



## 157 questionnaires, 1028 questions

Include custom questionnaires from the community

Official questionnaires

Custom questionnaires

Item classification



+ Add questionnaire

A



**Anal**  
module  
(28 questions)

B



**Bladder**



**Bone Metastases**

## Welcome to the Item Library!

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**Previous versions** are questionnaires that have been updated.

Official questionnaires

Custom questionnaires

Item classification

Standard classification | CTCAE classification

- + Cardiac disorders (6)
- + Ear and labyrinth disorders (5)
- + Endocrine disorders (3)
- + Eye disorders (85)
- + Gastrointestinal disorders (308)
- + General disorders and administration site conditions (320)
- + Immune system disorders (2)
- + Infections and infestations (13)
- + Injury, poisoning and procedural complications (24)
- + Investigations (33)
- + Metabolism and nutrition disorders (18)
- + Musculoskeletal and connective tissue disorders (101)
- + Nervous system disorders (286)
- + Not classified (406)
- + Psychiatric disorders (109)
- + Renal and urinary disorders (30)
- + Reproductive system and breast disorders (93)
- + Respiratory, thoracic and mediastinal disorders (118)
- + Skin and subcutaneous tissue disorders (75)
- + Surgical and medical procedures (8)
- + Vascular disorders (11)

# Item list

**Items from H&N43**



**Items from THY34**



**Items from FA12**





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## Recommendations on the use of item libraries for patient-reported outcome measurement in oncology trials: findings from an international, multidisciplinary working group



*Claire Piccinin, Ethan Basch, Vishal Bhatnagar, Melanie Calvert, Alicyn Campbell, David Cella, Charles S Cleeland, Corneel Coens, Anne-Sophie Darlington, Amylou C Dueck, Mogens Groenvold, Ralf Herold, Bellinda L King-Kallimanis, Paul G Kluetz, Dagmara Kuliš, Daniel O'Connor, Kathy Oliver, Madeline Pe, Bryce B Reeve, Jaap C Reijneveld, Xin Shelley Wang, Andrew Bottomley*

***Lancet Oncol 2023; 24: e86–95***

Thank you!

# EURORDIS MASTERCLASS

22<sup>nd</sup> May 2023

## By ERICA WP3 - Patient-Centered Research in Rare Diseases

*Mar Mañú Pereira, Ana Rath, Mariangela Pellegrini, Céline Desvignes-Gleizes, Gavin McDonough, Caterina Lucano, Benoit Arnould*

## Presenters



**Céline Desvignes-Gleizes**

Patient-Centred  
Outcomes specialist,  
Mapi Research Trust



**Mariangela Pellegrini,**

Educational and patients  
project manager,  
ERN-EuroBloodNet



**Gavin McDonough**

Disability Project Manager,  
Orphanet

## Learning objectives

- ❖ **Main objective:** The impact of PROMs and the benefits of using them for advocacy
  
- ❖ **Addressed questions:**
  - Considerations before using PROMs
  - Why are PROMs so crucial for advocacy: Case study
  - ERICA project - Tools to select adequate PROMs
  - How patient advocates can contribute?

# **Considerations before using PROMs**

# Measuring what ?

*“ One size doesn't fit all ”*



**Clarification of your PROMS-related objective is a prerequisite!**





## “Measuring What?”: What do you need to hear from patients?



**Describe disease burden** on patients' quality of life



**Assess quality of care, communication with health care staff and support medical decision-making**



**Evaluate treatment impact**

# The context of use determines your PROMs selection



## The context of use determines your PROMs selection

### *Example: Measure of depression*

#### ■ **Screening:**

- Hospital Anxiety and Depression Scale (HADS - 14 items) : Quick completion time and immediate scoring

#### ■ **Assessment of programs – Measure of depression impact on QoL:**

- Quality of Life in Depression Scale (QLDS - 34 items)
- SF-36 (36 items)
- Work Productivity & Activity Impairment Questionnaire (WPAI:D – 6 items)

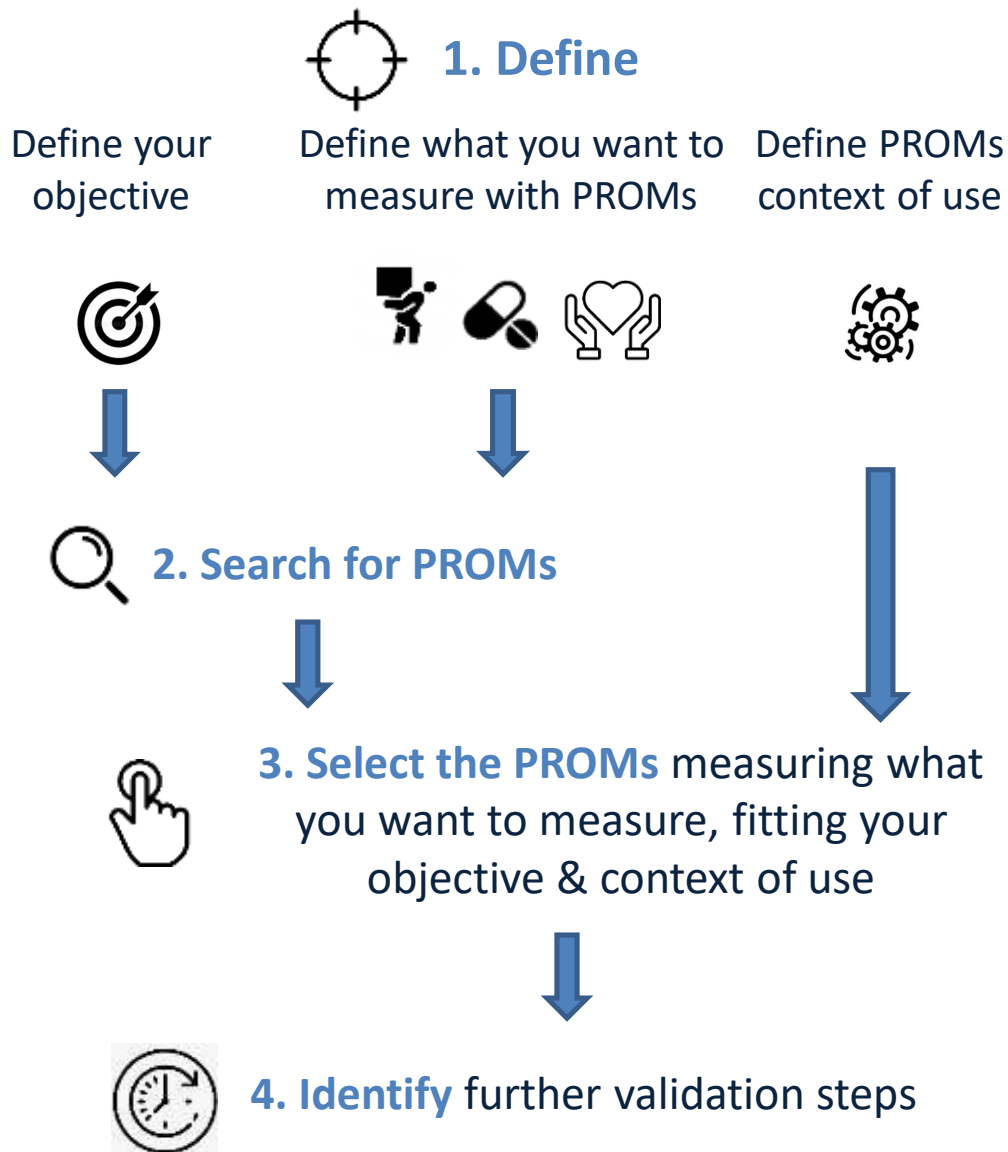
#### ■ **Assessment of mid-term outcomes of care on all patients from hospital:**

- Motivation and Energy Inventory (MEI-SF - 18 items)

#### ■ **Assessment of intervention:**

- Montgomery-Asberg Depression Rating Scale (MADRS – 10 items/signs and symptoms)

# In a nutshell



**Beyond PROMs validity, acceptance by patients and adoption by clinicians is critical**



## Criteria to consider to favor acceptance by patients and adoption by clinicians

- **Feasible:** PROM should be **easy-to-use** (*e.g. NRS vs VAS*)
- **Actionable:** PROM score should **easily lead to decision-making and action** (*eg for Diagnosis: PHQ-9 thresholds 5, 10, 15 and 20 represented mild, moderate, moderately severe and severe depression, respectively*)
- **In-Context:** PROM should be incorporated into the provider's **routine process** of care (*e.g. SF-36 good for research but complex scoring doesn't allow routine use*)
- **Useful:** PROM collection and processing should contribute to **better healthcare**



## **Importance of early patients' engagement in this process**

# **Why are PROMs so crucial for advocacy: example study**



# **PROMs provide data and evidence on subjective needs and feelings**



# Case study



## ENERCA Patient Questionnaire

### “Patients’ Needs and Expectations of Expert Centres in Haemoglobin Disorders”

This questionnaire should be answered by patients over 15 years old, or parents of patients under the age of 15. Please read the accompanying letter before answering. All information will be treated as confidential.

**Please note:** Section 3 (including 3a and 3b) is obligatory. Sections 1, 2 and 4 are optional, but it will be helpful if you complete them also.

#### Section 1 – About the patient (optional section)

**Q1. Questionnaire completed by:**

*Patient*

*Parent*

*Other (e.g. relative, helper,  
patient association representative)*  Please specify: \_\_\_\_\_



**Patient community  
expressing needs and  
burdens based on  
their experiences  
orally**

**Not structured**



**Patient community  
expressing needs and  
burdens based on their  
experiences gathered in  
questionnaires that provide  
evidence**

**Structured**



### Patient feedback

*« I spend a lot of time in day unit for receiving transfusion. I loose time from school or work »*



### Patient Need

The provision of transfusions in a timely manner and convenient for the patients hours



### Data from the Patient Questionnaire

The majority of responders (63.84%) are transfused in **morning**

Of the patients transfused in the morning 30.7% are working full time, 13% are working part time and 21.6% are not working

Q18. Time of transfusion	Number of patients	Percentage
Morning	256	63.84%
Afternoon	117	29.18%
Evening	9	2.24%
Overnight	10	2.49%
Weekemd	8	1.99%
Other (hospitalised for 3-5 days)	1	0.25%



### Patient feedback

*"My center doesn't guarantee me a good adherence to treatments. I experience a lot of pain"*



### Patient Need

Adherence to lifelong treatment requires support from the healthcare providers



### Data from the Patient Questionnaire

27.77% of respondent are not treated in **expert centers**

31.45% were receiving Desferrioxamine monotherapy daily and 19.62% a combination of the subcutaneous drug and an oral chelator (Deferiprone)

➔ More than half still had to adhere to difficult and painful treatments

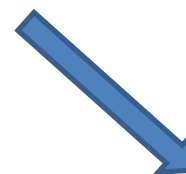
→ **Quantitative evidence provided by the questionnaire could support the shaping of Health Planning or Health Delivery centers**



**Determinate a way to provide clinical services as standard of care**



Hospitals with transfusion centers open in the late afternoon



**Formal recognition of disabilities, impairments and handicaps**



Having right to more days off from work

**- ERICA project –  
Tools to select the  
adequate PROMs**

## ERICA Patient-Centric Research WP3 team

**ERICA Objective:** To facilitate the Europe-wide implementation of standardized Patient-Centred Outcome Measures for rare diseases



**Ana Rath**

Director  
Orphanet  
ERICA WP3 Co-lead



**Mariangela Pellegrini**

Educational & Patients Program manager  
-EuroBloodNet  
Assistance Publique - Hôpitaux de Paris  
(APHP)



**Gavin McDonough**

Disability Project Manager  
Orphanet



**Céline Desvignes-Gleizes**

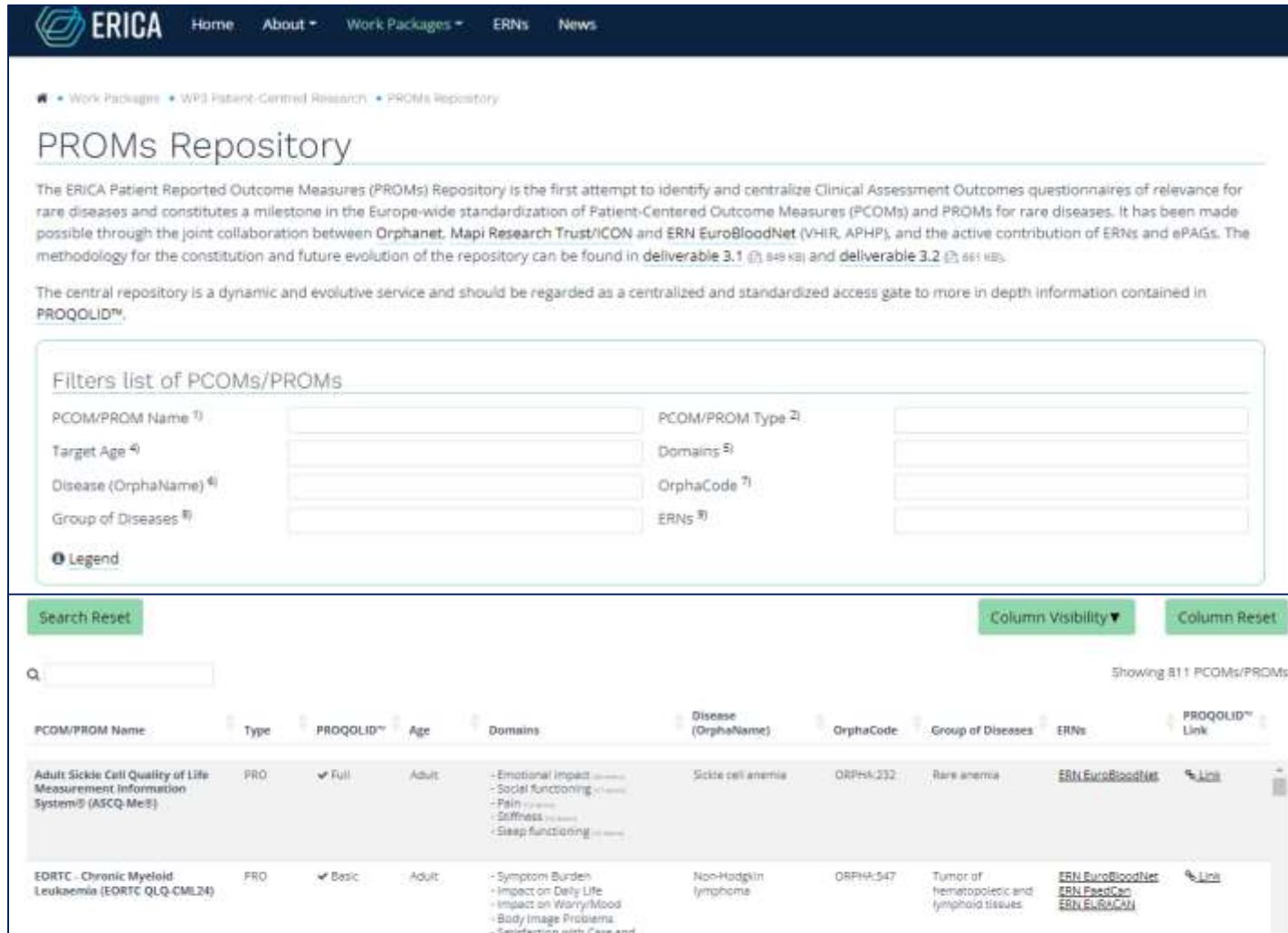
Knowledge management and  
partnerships  
Mapi Research Trust / ICON



**Mar Mañu Pereira**

Principal Investigator  
Vall d'Hebron Institute of  
Research (VHIR)  
ERICA WP3 Co-lead





The screenshot shows the ERICA website's 'PROMs Repository' page. At the top, there is a navigation bar with 'ERICA', 'Home', 'About', 'Work Packages', 'ERNs', and 'News'. Below the navigation bar, there are breadcrumb links: 'Work Packages' > 'WP3 Patient-Centred Research' > 'PROMs Repository'. The main heading is 'PROMs Repository'. A descriptive paragraph explains that the repository is the first attempt to identify and centralize Clinical Assessment Outcomes questionnaires for rare diseases. It mentions collaboration with Orphanet, Mapi Research Trust/ICON, and ERN EuroBloodNet. Below the text is a search and filter interface with a 'Filters list of PCOMs/PROMs' section containing input fields for PCOM/PROM Name, Target Age, Disease (OrphaName), Group of Diseases, PCOM/PROM Type, Domains, OrphaCode, and ERNs. There are also 'Search Reset', 'Column Visibility', and 'Column Reset' buttons. A search bar is present with the text 'Showing 811 PCOMs/PROMs'. Below the search bar is a table with columns: PCOM/PROM Name, Type, PROQOLID™, Age, Domains, Disease (OrphaName), OrphaCode, Group of Diseases, ERNs, and PROQOLID™ Link. Two rows are visible in the table:

PCOM/PROM Name	Type	PROQOLID™	Age	Domains	Disease (OrphaName)	OrphaCode	Group of Diseases	ERNs	PROQOLID™ Link
Adult Sickle Cell Quality of Life Measurement Information System® (ASQ-MeS)	PRO	Full	Adult	- Emotional Impact - Social functioning - Pain - Stiffness - Sleep functioning	Sickle cell anemia	ORPHA:232	Rare anemia	ERN EuroBloodNet	<a href="#">Link</a>
EORTC - Chronic Myeloid Leukaemia (EORTC QLQ-CML24)	PRO	Basic	Adult	- Symptom Burden - Impact on Daily Life - Impact on Worry/Mood - Body Image Problems - Satisfaction with Care and	Non-Hodgkin lymphoma	ORPHA:547	Tumor of hematopoietic and lymphoid tissues	ERN EuroBloodNet ERN EusCancer ERN EURACAN	<a href="#">Link</a>

### Repository v1.0 content:

- PROMs developed in Rare diseases (278)
- Generic Quality of life PROMs (5)
- PROMs measuring functional impacts (151)
- PROMs of interest for ERN (216)
- PROMs to assess health care management (161)

### Repository v2.0 content: ObsRO

## ERICA PROMs/PREMs repository

<https://erica-rd.eu/work-packages/patient-centred-research/proms-repository/>

## For complete information on PROMs

 PROQOLID™

### **PROMs Database**

<https://eprovide.mapi-trust.org/advanced-search?database=proqolid>

#### Database content:

- Author information
- Conditions of use
- Translations
- Population of development
- PROMs review copy
- Information on development and validation of PROMs

## 2 ERN Pilots on PROMs implementation

### ❖ ERN Transplantchild

- PETER Registry: Registry points for clinical data gathering (web)+ PROMS questionnaire (mobile app) for monitoring transplant phases for pediatric population

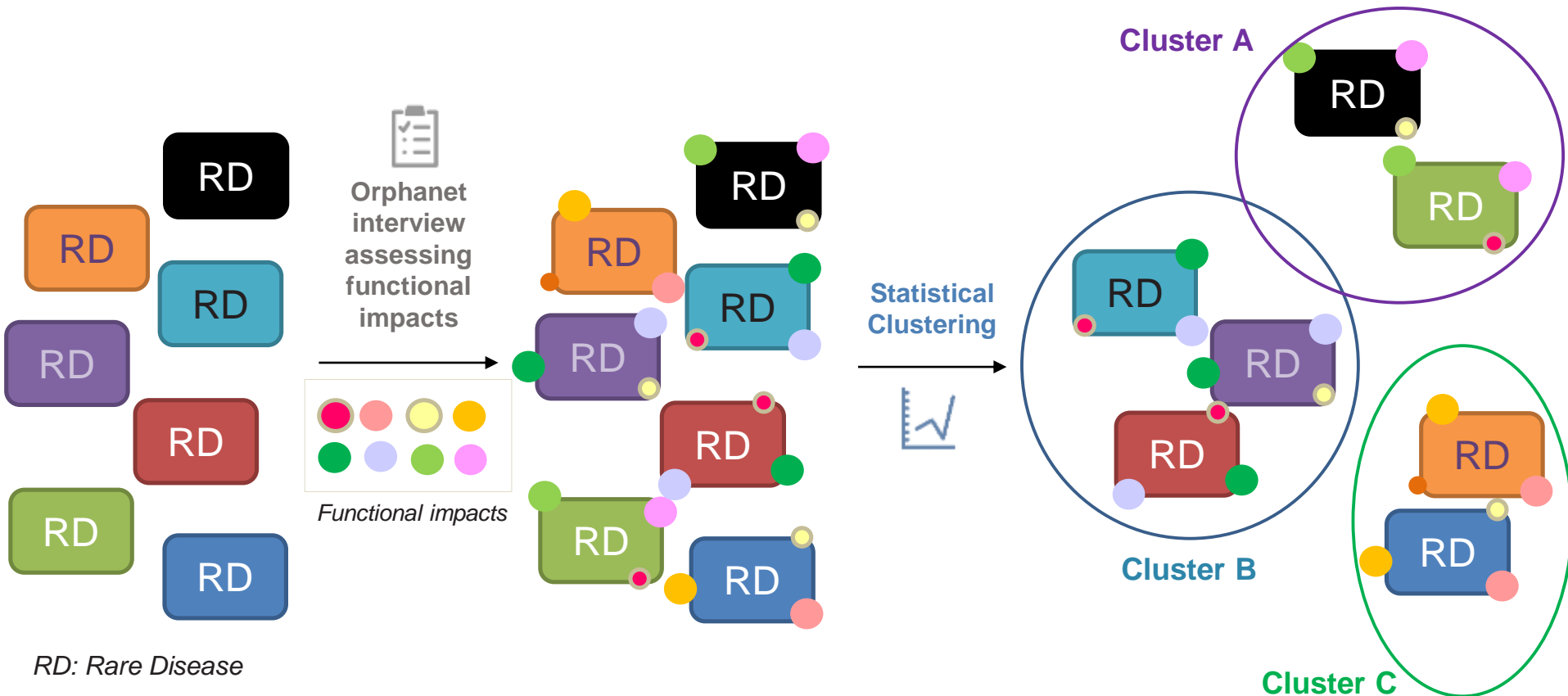
### ❖ ERN-EuroBloodNet

- SATISFY Trial: Associating PROMs for PKD in Clinical Trial in Danish Red Blood Cell centers

# How patient advocates can contribute?

- ❖ **Submit your PROMs of interest to be implemented in the repository** (Survey available in June 2023)
  
- ❖ **Annotate your disease**
  - What: To document the functional consequences of rd on daily life
  - How: 1 hour semi-structured interviews using the Orphanet Disability Thesarus (adapted from the WHO ICF)
  - Who: Please contact Gavin McDonough at [disability.orphanet@inserm.fr](mailto:disability.orphanet@inserm.fr)
  - Why:
    - To build upon the knowledge of clinicians & patient associations
    - To increase the number of rd/clinical entities indexed online
    - To strengthen our on-going ERICA WP3 clustering work

# Rare diseases clustering work



**→ A rare disease among your disease's cluster could have a questionnaire of interest for you**

# THANK YOU !



**Céline Desvignes-Gleizes**

Patient-Centred  
Outcomes specialist

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**Gavin McDonough**

Disability/Handicap  
Project Manager

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Development PROM Performance of Upper Limb

EURORDIS Masterclass 2023  
Elizabeth Vroom, World Duchenne Organization





Development PROM starting from the patients

About the role of a patient organisation in PROM development

# Duchenne Muscular Dystrophy

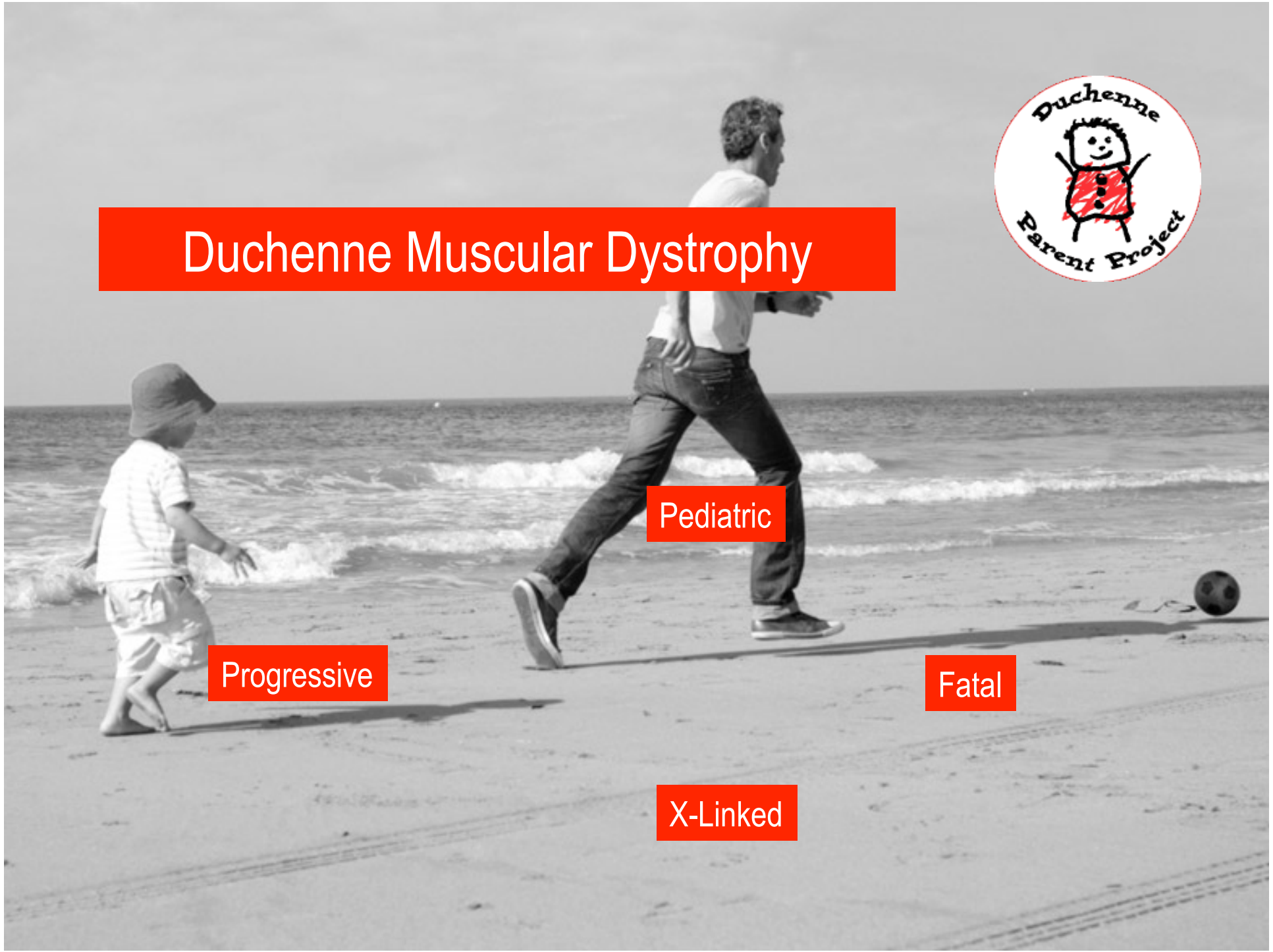


Pediatric

Progressive

Fatal

X-Linked



# Outcome measures in DMD



6 minute walk test

Fast majority of DMD patients is non ambulant

Need of outcome measures for non ambulant patients

'Walking is highly overrated'

Interviewed patients

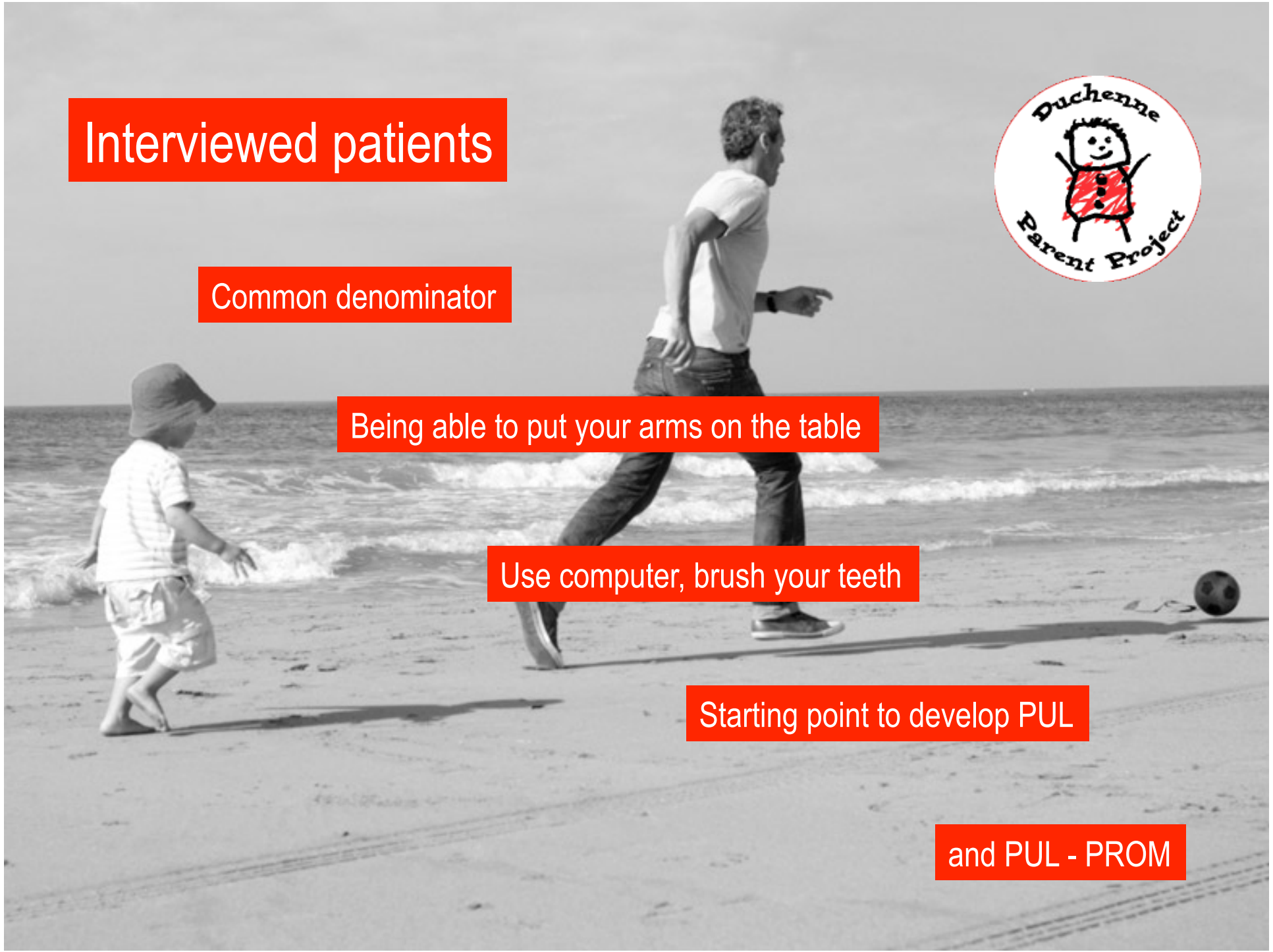
Common denominator

Being able to put your arms on the table

Use computer, brush your teeth

Starting point to develop PUL

and PUL - PROM



# Performance Upper Limb



Working group formed

Funded by Patient Organisation

PUL functional outcome measure

PROM-PUL

Validated

Owned by Patient Organization

Co-Creation





## AIM

To develop a patient-reported outcome measure (PROM) assessing upper limb function related to activities of daily living (ADL) that cannot be observed in a clinical setting, specifically for patients with Duchenne muscular dystrophy (DMD) across a wide age range, applicable in the different stages of the disease.



## METHOD

The developmental process was based on US Food and Drug Administration guidelines.

This included item generation from a systematic review of existing tools and expert opinion on task difficulty and relevance, involving individuals with DMD.

Cultural aspects affecting ADL were taken into consideration to make this tool applicable to the broad DMD community.

Items were selected in relation to a conceptual framework reflecting disease progression covering the full range of upper limb function across different ADL domains.





## RESULTS

After pilot testing and iterative Rasch analyses, redundant or clinically irrelevant items were removed.

The final questionnaire consists of 32 items covering four domains of ADL (food, self-care, household and environment, leisure and communication).

Test–retest reliability was excellent.



## INTERPRETATION

A DMD-specific upper limb PROM was developed on the basis of clinical relevance and psychometric robustness. Its main purpose is to document the patient selfreported natural history of DMD and assess the efficacy of interventions.



Dev Med Child Neurol. 2013 Nov;55(11):1038-45.  
Development of the Performance of the Upper Limb  
module for Duchenne muscular dystrophy

Mayhew A1, Mazzone ES, Eagle M, Duong T, Ash M, Decostre V, Vandenhauwe M, Klingels K, Florence J, Main M, Bianco F, Henrikson E, Servais L, Champion G, Vroom E, Ricotti V, Goemans N, McDonald C, Mercuri E; Performance of the Upper Limb Working Group.



Development of a patient-reported outcome measure for upper limb function in Duchenne muscular dystrophy: DMD Upper Limb PROM

Klingels K, Mayhew AG, Mazzone ES, Duong T, Decostre V, Werlauff U, **Vroom E**, Mercuri E, Goemans NM; Upper Limb Clinical Outcome Group.  
Dev Med Child Neurol. 2016 Sep 26

Development of a conceptual framework reflecting the disease progression and functional decline in DMD with input from a broad array of stakeholders



Systematic and critical review of the existing questionnaires that include items related to upper limb function



Selection and adaptation of existing items and integration of newly constructed items based on input from experts, patients, and families



Involvement of male children and adults with DMD and their families in an iterative process to establish the clinical meaningfulness and relevance of items to activities of daily living and validate the conceptual framework



Development of a pilot pro forma with a first selection of items suitable for ambulant and non-ambulant young males with DMD



Application of the questionnaire in a multi-centre setting and consecutive Rasch analyses



Discussion with experts, patients, and families to interpret the results of the Rasch analyses followed by adaptations of the questionnaire



Development of the final questionnaire



A new patient-reported outcome measure (PROM) for Duchenne muscular dystrophy (DMD) has been developed.

The DMD Upper Limb PROM targets upper limb function in daily life.

Psychometric techniques confirmed its unidimensionality, internal consistency, and test-retest reliability.

Involvement of different stakeholders guaranteed the clinical relevance of the tool.



Correlation outcomes PUL-PROM and PUL  
Paper will be submitted shortly



# Translation



Guidelines for the Translation of the 'DMD Upper Limb Patient Reported Outcome Measure'

Principles of Good Practice for the Translation and Cultural Adaptation Process for Patient-Reported Outcomes (PRO) Measures: report of the ISPOR Task Force for Translation and Cultural Adaptation





Ther Innov Regul Science. 2022 Jul;56(4):572-586.  
Unmet Therapeutic Needs of Non-Ambulatory Patients with Duchenne  
Muscular Dystrophy: A Mixed-Method Analysis

Anne L R Schuster 1, Norah L Crossnohere 2, Ryan Fischer 3, Patricia  
Furlong 3, John F P Bridges 2

Non-ambulatory Duchenne patients want new treatments that improve  
upper limb functioning and body system functioning, and not exclusively  
regaining ambulation. The PUL-PROM can be used as a patient-centric  
measure that accounts for the needs of later-stage Duchenne patients.



## Patient Reported Outcome Measures

- ✓ Standardisation
- ✓ Validation
- ✓ Translation
- Qualification
- Trademark
- ✓ Licensing



## Summary

Start from the patients perspective

Ability of Patient Organisation is relevant

Multistakeholder activity

C0-creation



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Thank you!

