



PROMs and PREMs in ENROL

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ENROL CONSORTIUM



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ENROL is endorsed by the European Hematology Association



Keys concepts from past webinars

- A registry is a platform that collects **patients data** like diseases, country, age, treatments
- ENROL maps **common data across existing registries in Europe** facilitating to monitor a disease across Europe and helping research and policy' development for rare haematological diseases.
- ENROL gathered **data** that in Europe would have been fragmented **in one central platform**. Enrol makes them **understandable, accessible** and **reusable** by researcher and policy makers and guarantying patients' privacy!
- Registries facilitate not only research but also **patient-centric research**

Learning objectives of the webinar

Main objective: The impact of PROMs and PREMs and the benefits of associating them to registries

Addressed questions

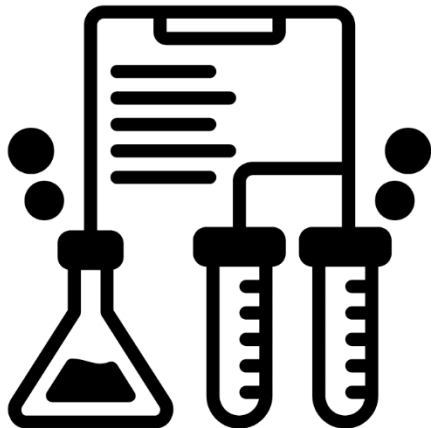
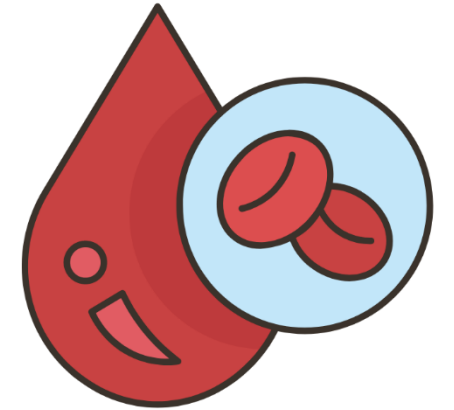
- What are PROMs & PREMs?
- What are the benefits of using them for patients and for scientific communities?
- What do I need to know about PROMs & PREMs before including them in a registry?
- Where can I find adequate PROMs & PREMs and who can I address to have more info on PROMs & PREMs?
- How to use PROMs & PREMs for Advocacy?



What are PROMs and PREMs?

Let's start from some examples

Let's imagine a Clinical Trial on a drug for increasing the hemoglobin



Let's start from some examples



- Fatigue.
- Weakness.
- Pale skin and gums.
- Shortness of breath.
- A fast or irregular heartbeat.



Hemoglobin 7

Let's start from some examples

With this new drug....



- Fatigue.
- Weakness.



Hemoglobin 14

Let's start from some examples

With this new drug....



- Fatigue.
- Weakness.

I feel still almost the same,
I don't know if the drug works!



Hemoglobin 14: the drug works!!!

Why Patient Perception Matters

Question: BT done in a room with other patients



...rassure les patients



20%

60%

7%

30%

...gêne les patients



10% 15%

3% 17%

...rend les patients
anxieux / stressés



5% 15%

3%



*Celgene study on BT Burden, 11/2020

- Strongly agree
- Agree somewhat



So: how to integrate the perception of the patient into the clinical outcomes of the trial?

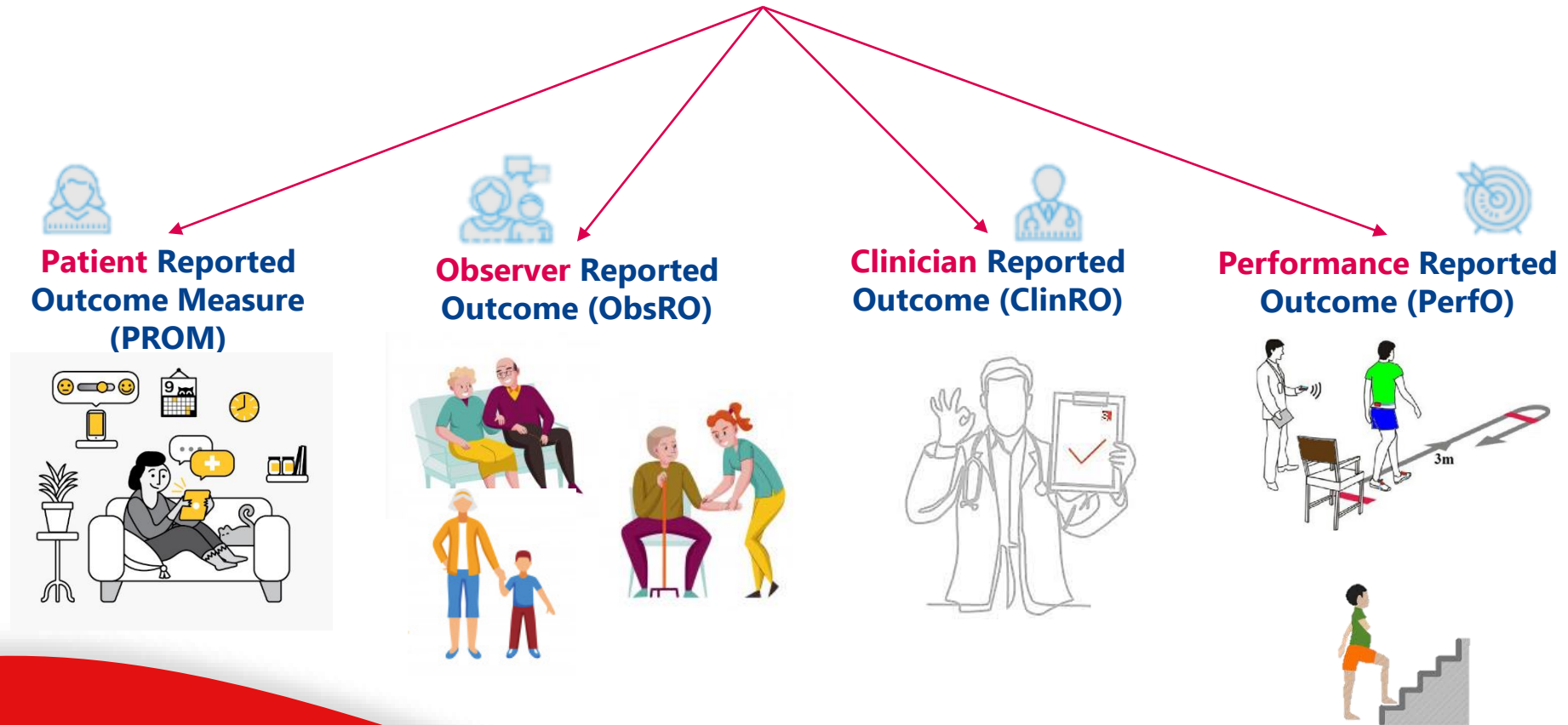
Patient-Reported Outcomes Measures (PROMs): Definition

“A measurement based on a report that **comes directly from the patient** (i.e., study subject) about the status of a patient’s health condition **without amendment or interpretation** of the patient’s response by a clinician or anyone else. A PRO can be measured by **self-report** or **by interview** provided that the interviewer records only the patient’s response”

FDA, 2009, "Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims"

PROMs belong to Clinical Outcome Assessment Measures

Clinical Outcome Assessment (COA)



To type of COA is determined by the perspective/point of view that is rated

PROMs structure

DERMATOLOGY LIFE QUALITY INDEX

The aim of this questionnaire is to measure how much your skin problem has affected your life OVER THE LAST WEEK. Please tick ✓ one box for each question.

- | | | | |
|----|---|--|--|
| 1. | Over the last week, how itchy, sore, painful or stinging has your skin been? | Very much
A lot
A little
Not at all | <input type="checkbox"/> 3
<input type="checkbox"/> 2
<input type="checkbox"/> 1
<input type="checkbox"/> 0 |
| 2. | Over the last week, how embarrassed or self-conscious have you been because of your skin? | Very much
A lot
A little
Not at all | <input type="checkbox"/> 3
<input type="checkbox"/> 2
<input type="checkbox"/> 1
<input type="checkbox"/> 0 |

Instructions

Item (= question) : it contains the measured concept and the recall period

Response options

Number of response options

Item Score

PROMs structure

The aim of this questionnaire is to measure how much your skin problem has affected your life OVER THE LAST WEEK. Please tick ✓ one box for each question.

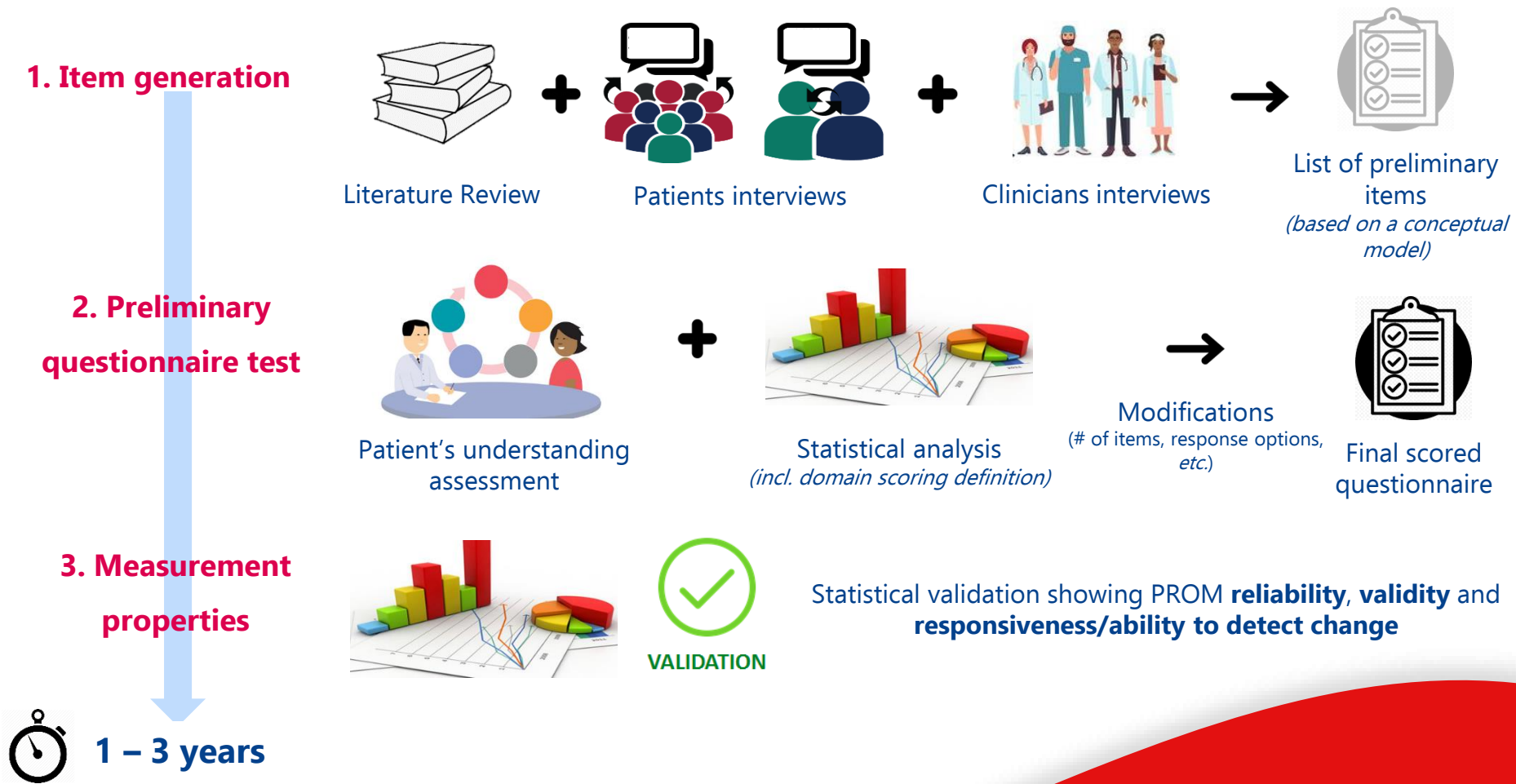
1.	Over the last week, how itchy, sore, painful or stinging has your skin been?	Very much <input type="checkbox"/>	A lot <input type="checkbox"/>	A little <input type="checkbox"/>	Not at all <input type="checkbox"/>
2.	Over the last week, how embarrassed or self-conscious have you been because of your skin?	Very much <input type="checkbox"/>	A lot <input type="checkbox"/>	A little <input type="checkbox"/>	Not at all <input type="checkbox"/>
3.	Over the last week, how much has your skin interfered with you going shopping or looking after your home or garden ?	Very much <input type="checkbox"/>	A lot <input type="checkbox"/>	A little <input type="checkbox"/>	Not at all <input type="checkbox"/> Not relevant <input type="checkbox"/>
4.	Over the last week, how much has your skin influenced the clothes you wear?	Very much <input type="checkbox"/>	A lot <input type="checkbox"/>	A little <input type="checkbox"/>	Not at all <input type="checkbox"/> Not relevant <input type="checkbox"/>
5.	Over the last week, how much has your skin affected any social or leisure activities?	Very much <input type="checkbox"/>	A lot <input type="checkbox"/>	A little <input type="checkbox"/>	Not at all <input type="checkbox"/> Not relevant <input type="checkbox"/>
6.	Over the last week, how much has your skin made it difficult for you to do any sport ?	Very much <input type="checkbox"/>	A lot <input type="checkbox"/>	A little <input type="checkbox"/>	Not at all <input type="checkbox"/> Not relevant <input type="checkbox"/>
7.	Over the last week, has your skin prevented you from working or studying ?	Yes <input type="checkbox"/>	No <input type="checkbox"/>	Not relevant <input type="checkbox"/>	
	If "No", over the last week how much has your skin been a problem at work or studying ?	A lot <input type="checkbox"/>	A little <input type="checkbox"/>	Not at all <input type="checkbox"/>	
8.	Over the last week, how much has your skin created problems with your partner or any of your close friends or relatives ?	Very much <input type="checkbox"/>	A lot <input type="checkbox"/>	A little <input type="checkbox"/>	Not at all <input type="checkbox"/> Not relevant <input type="checkbox"/>
9.	Over the last week, how much has your skin caused any sexual difficulties ?	Very much <input type="checkbox"/>	A lot <input type="checkbox"/>	A little <input type="checkbox"/>	Not at all <input type="checkbox"/> Not relevant <input type="checkbox"/>
10.	Over the last week, how much of a problem has the treatment for your skin been, for example by making your home messy, or by taking up time?	Very much <input type="checkbox"/>	A lot <input type="checkbox"/>	A little <input type="checkbox"/>	Not at all <input type="checkbox"/> Not relevant <input type="checkbox"/>

Domain(s) covered by the questionnaire

- Symptoms and feelings (2 items)
- Daily activities (2 items)
- Leisure (2 items)
- Work and school (1 item)
- Personal relationships (2 items)
- Treatment (1 item)

PROM development

(according to FDA 2009 PRO guidance*)



* *Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims - DECEMBER 2009*
Please note that the development of COA doesn't follow systematically the 2009 FDA Guidance requirements

Patient-Reported Experience Measures (PREMs): Definition

Patient-reported experience measures (PREMs) are questionnaires measuring **the patients' perceptions of their experience, the quality of care whilst receiving care**. In contrast to PROMs, PREMs do not look at the outcomes of care but the impact of the process of the care on the patient's experience e.g. communication, health care staff relationship and timeliness of assistance

Charlotte Kingsley, MBBS BSc FRCA, Sanjiv Patel, MBBS BMedSci FRCA, Patient-reported outcome measures and patient-reported experience measures, BJA Education, Volume 17, Issue 4, April 2017, Pages 137–144"

PREMs structure



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: _____

Q2. Patient’s age: _____

Q3. Patient’s gender Male Female

Q4. Patient’s marital status Married Single Cohabiting
 Divorced Children (number: ____)

Instructions

PREMs structure

Section 3 – The medical services you (the patient) are currently using

Q15. Where do you receive medical treatment for your condition? (tick all that apply)

- Specialised *haemoglobinopathy* centre
- General haematology department at a hospital
- General *paediatric* department of a hospital
- Private clinic/centre (non-specialist)
- Other (describe)
-
-

Q16. How long do you usually wait for a transfusion to be set up?

- Under 30 min 30-60 min
- 1-2 hours 2-3 hours Longer

Q17. Where are you transfused?

- Haematology day unit Adult Haematology ward
- Children's ward Accident & Emergency
- Specialised *Haemoglobinopathy* unit Home

Q18. When are you usually transfused?

- Morning Afternoon Evening Overnight Weekend

Q19. The treatment centre where you go for treatment is located:

- Local/near where I live
- Another region / city
- Another country If so, why do you go to another country treatment?

Q20. Is access to the treatment centre (in terms of distance, cost etc.):

- Very easy? Easy?
- Difficult? Very difficult?
- Not available? Available but too expensive

Q21. Who pays for your treatment? (Tick all that apply)

- Myself/my family
- Health insurance (private): Mine My employer's
- Health insurance (state)
- State-provided free healthcare State-provided, partly free
- Other model of payment

Please describe: _____

Q22. What specialist(s) do you visit, in addition to your main treating doctor?

- Internal medicine Frequency of visits: _____
- Haematologist Frequency of visits: _____
- Paediatrician Frequency of visits: _____
- Heart specialist Frequency of visits: _____
- Endocrinologist Frequency of visits: _____
(for checking growth and development, fertility, bone disease)
- Psychologist Frequency of visits: _____
- Liver specialist Frequency of visits: _____

Q23. Where do you see the specialist(s)? (Tick all that apply)

- Same hospital
- Other hospital

Score

Question

Response options



What do I need to know about
PROMs & PREMs before
associating them in a registry?

“Measuring What?”

What do you need to hear from
patients?



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



Describe disease burden

on patients quality of life (QOL) with PROMs&PREMs

Eg Generic and Disease-Specific QoL scales



Assess quality of care

with PREMs

Eg Generic and ERN-Specific PREMs scales



Evaluate treatment impact

on functioning with validated function-specific scales (ClinROs, PerfOs, PROMs)

Eg Mobility-specific scale



Capture patient perception of change in symptoms: with PROMs symptom-specific scales and ad-hoc diaries Eg Pruritus VAS & 5D-Itch

“What for?”

Clarification of PROMS/PREMs-related
objective is a prerequisite!



PROMs/PREMs, What for ?

- **Patient Management**

- Guidance / Education:

Dissemination of current knowledge to physicians

- Support medical decision-making

Improvement of diagnosis and treatment decisions

- Enhance communication

Support patient-and-doctor-interaction on description of symptoms, complaints about functional impact and expected treatment benefits

- **Health Care Management:**

Evaluation of Efficiency of Care in Real-World Practice

- **Clinical Research:**

Evaluation of intervention effects (care, drug, device, surgery, physiotherapy, *etc.*)

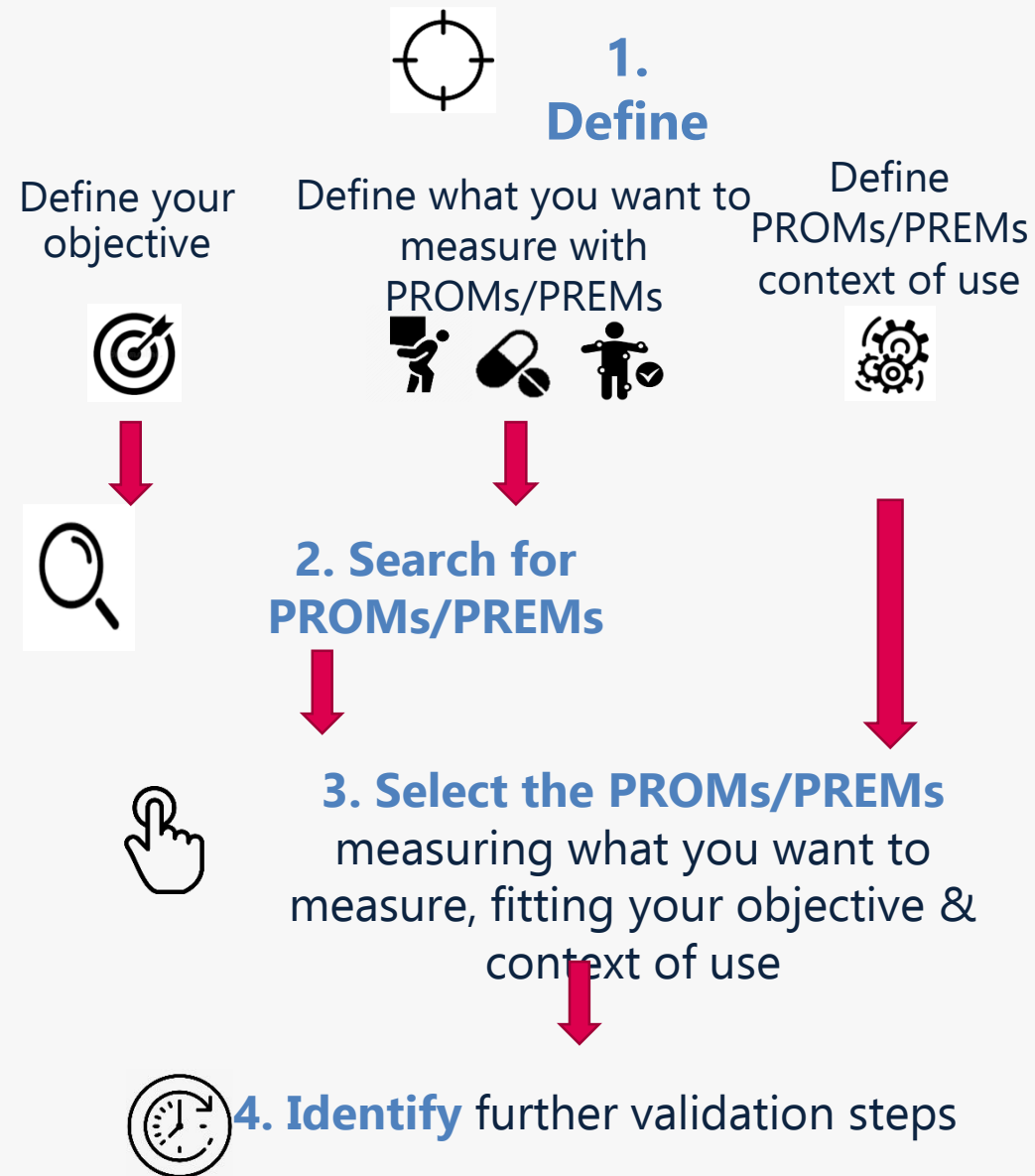
The context of use determines the PROMs/PREMs selection



The context of use determines your PROMs/PREMs 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 HRQoL:
 - 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



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

- **Feasible:** PROM/PREM should be **easy-to-use** (*e.g. NRS vs VAS*)
- **Actionable:** PROM/PREM score should easily lead to decision-making and action
- **In-Context:** PROM/PREM should be incorporated into the provider's **routine process** of care
- **Useful:** PROM/PREM collection and processing should contribute to **better healthcare**

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





IMPORTANCE OF EARLY PATIENTS ENGAGEMENTS OF ALL OF THOSE ACTIVITIES



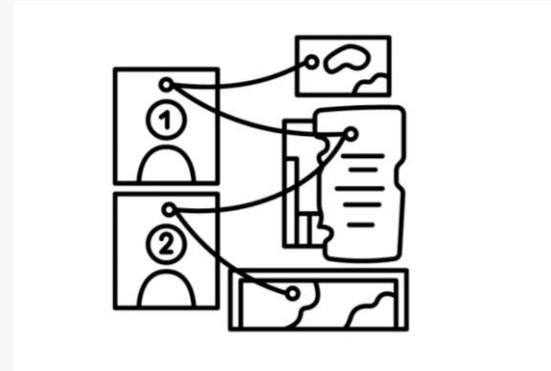


Why to have data issued from PROMs is important for advocacy?

PROMS/PREMs provide
evidences and not just
expressed needs or burdens



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



**Patient community
expressing needs and
burdens based on their
experiences gathered in a
questionnaires**



ENERCA Patient Questionnaire

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

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Section 1 – About the patient (optional section)

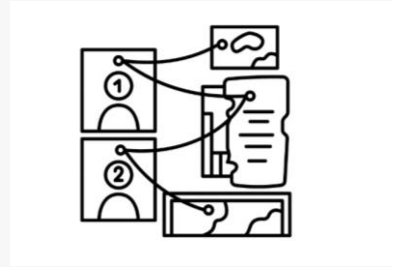
Q1. Questionnaire completed by:

Patient

Parent

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

Some examples
issued from this
questionnaire

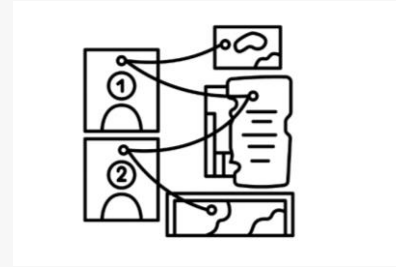


« I spend a lot of time in day unit for receiving transfusion. I loose time from school or work »»

Need “The provision of transfusions in a timely manner and at convenient for the patient hours”.

The majority of responders **256/401 (63.84%)** are transfused in morning

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%



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

Need “The provision of transfusions in a timely manner and at convenient for the patient hours”.

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

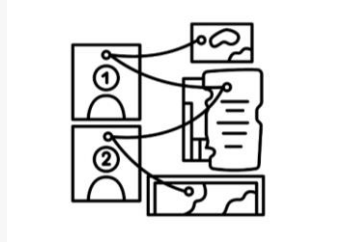


Need “The provision of transfusions in a timely manner and at convenient for the patient hours”.

Need: “The right to work”

The majority of responders 256/401 (63.84%) are transfused in morning, 13% are working part time and 21.6% are not working

Quantitative evidence (KPIs) → basis for shaping Health Planning or centre Health Delivery



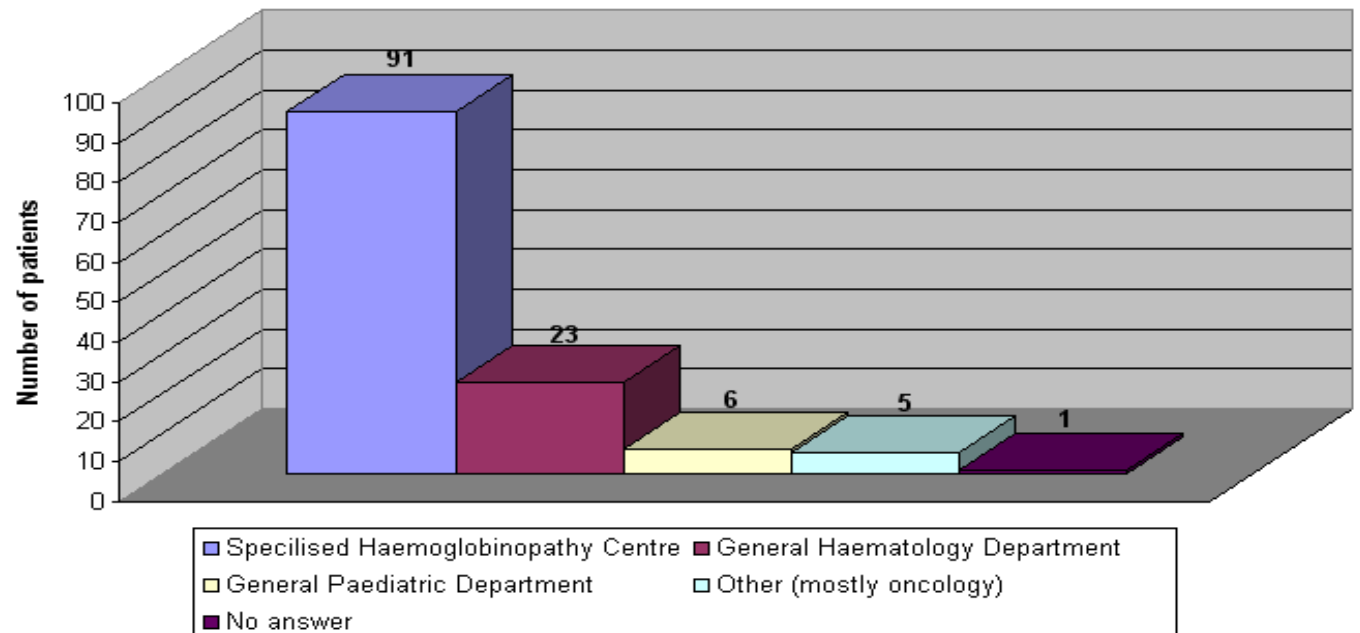
My center doesn't guarantee me a good adherence to treatments. I experience a lot of pain

of 126 patients who claimed regularity in taking medication 91 (72.22%) were being treated in specialised centres, while 35 (27.77%) in other departments.

In this cohort of patients 31.45% were receiving Desferrioxamine monotherapy daily while another 19.62% were on a combination of the subcutaneous drug and an oral chelator (Deferiprone)

so more than half still had to adhere to difficult and painful treatment.

Kind of centre where treated the patients who take drugs regularly





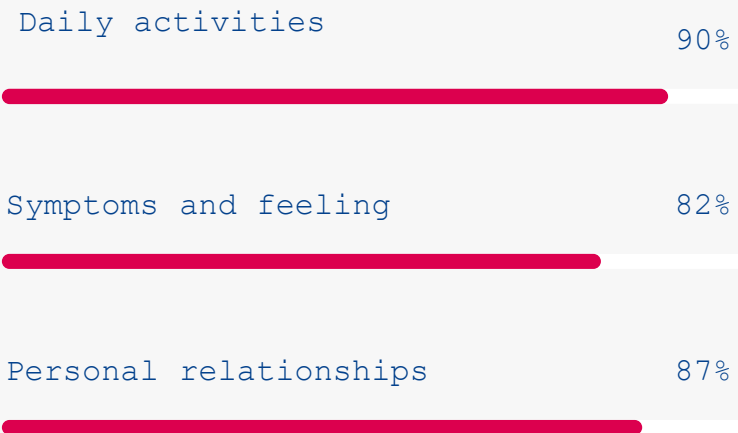
Need “Adherence to lifelong treatment requires support from the healthcare providers”

35 (27.77%) are not treated in expert centers .

In this cohort of patients 31.45% were receiving Desferrioxamine monotherapy daily while another 19.62% were on a combination of the subcutaneous drug and an oral chelator (Deferiprone)

Quantitative evidence (KPIs) → basis for shaping Health Planning or centre Health delivery

In the registries
PROMs and PREMs are
associated for
providing source of
data!



Systematic gathering of evidences via registries associating PROMs and PREMs

ACHIEVEMENTS

Using them for Advocacy

Involvement in the process of determinates baskets of benefits within a social health security rights



Determinate a way to provide clinical services as standard of care

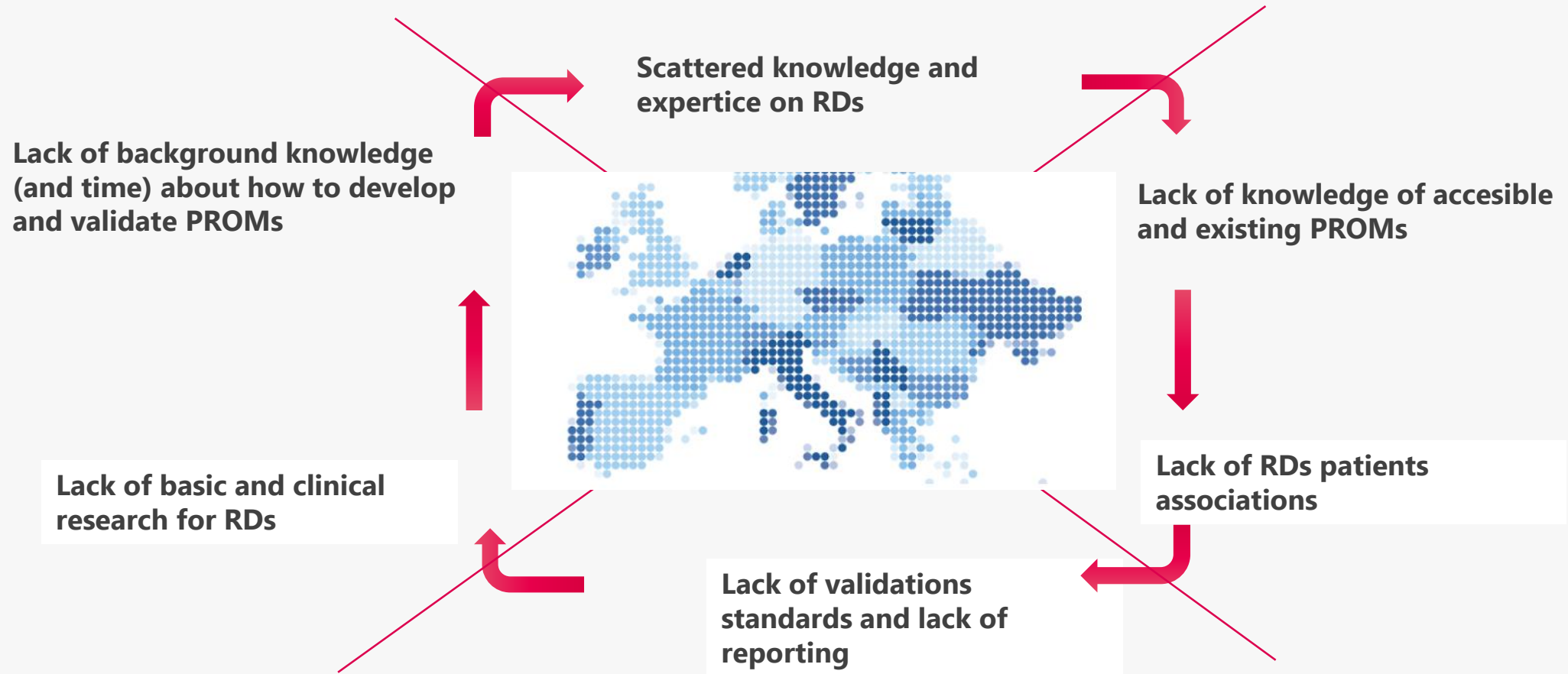
Hospital provided with transfusion canters opened in the late afternoon



Formal recognition of disabilities, impairments and handicaps

Having right to more days off from work

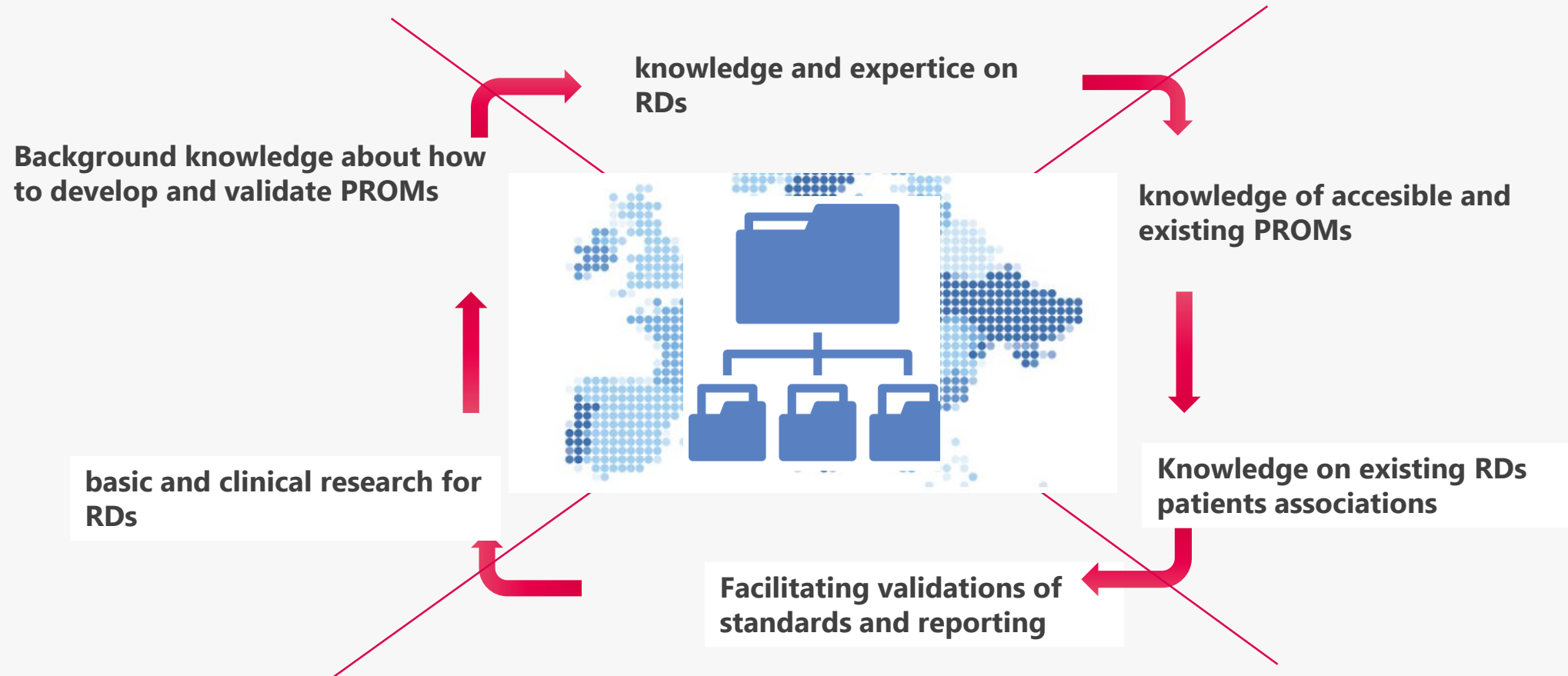
PROMs and RDs challenges





Where as patient representative I can find
adequate PROMs & PREMs?

PROMs and RDs challenges



WP3team

Patient-Centric Research



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Orphanet
ERICA WP3 Co-lead



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



Gavin McDonough

Position
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

PROMs selection strategy results

■ **PROQOLID™ search**

1. PROMs developed in Rare diseases (259 PROMs)
2. Generic Quality of life PROMs (160 PROMs)
3. PROMs measuring functional impacts (ex: Understanding, *etc.*) (151 PROMs)


• **ERN members survey** (211 PROMs)

➔ **Total PROMs identified:** 781 PROMs

➔ ObsRO will be included in the repository in upcoming month

➔ 132 ObsSRO including 15 developed in RD

RD PROMs/PREMs repository


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[PROMs Repository](#)

PROMs Repository

The ERICA Patient Reported Outcome Measures (PROMs) Repository is the first attempt to identify and centralize Clinical Assessment Outcomes questionnaires of relevance for rare diseases and constitutes a milestone in the Europe-wide standardization of Patient-Centered Outcome Measures (PCOMs) and PROMs for rare diseases. It has been made possible through the joint collaboration between [Orphanet](#), [Mapi Research Trust/ICON](#) and [ERN EuroBloodNet](#) (VHIR, APHP), and the active contribution of ERNs and ePAGs. The methodology for the constitution and future evolution of the repository can be found in [deliverable 3.1](#) (📄 849 KB) and [deliverable 3.2](#) (📄 561 KB).

The central repository is a dynamic and evolutive service and should be regarded as a centralized and standardized access gate to more in depth information contained in [PROQOLID™](#).

Filters list of PCOMs/PROMs

PCOM/PROM Name ¹⁾	<input type="text"/>	PCOM/PROM Type ²⁾	<input type="text"/>
Target Age ⁴⁾	<input type="text"/>	Domains ⁵⁾	<input type="text"/>
Disease (OrphaName) ⁶⁾	<input type="text"/>	OrphaCode ⁷⁾	<input type="text"/>
Group of Diseases ⁸⁾	<input type="text"/>	ERNs ⁹⁾	<input type="text"/>

[Legend](#)

Search Reset
Column Visibility ▾
Column Reset

Q

Showing 811 PCOMs/PROMs

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® (ASCQ-Me®)	PRO	✓ Full	Adult	<ul style="list-style-type: none"> - Emotional impact (20 items) - Social functioning (17 items) - Pain (13 items) - Stiffness (15 items) - Sleep functioning (12 items) 	Sickle cell anemia	ORPHA:232	Rare anemia	ERN EuroBloodNet	Link
EORTC - Chronic Myeloid Leukaemia (EORTC QLQ-CML24)	PRO	✓ Basic	Adult	<ul style="list-style-type: none"> - 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 PaedCan ERN EURACAN	Link

ERICA PROMs/PREMs repository

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

e.g. searching for transfusion related PROMs/PREMs

Search Reset

Column Visibility ▼

Column Reset

Q transfusion x

Showing 2 PCOMs/PROMs (of 811 total)

PCOM/PROM Name	Type	PROQOLID™	Age	Domains	Disease (OrphaName)	OrphaCode	Group of Diseases	ERNs	PROQOLID™ Link
Nontransfusion-dependent thalassemia - Patient-reported outcome (NTDT-PRO)	PRO	No	Adult	- Tiredness - Weakness - Shortness of Breath, with or without Physical Activity	Beta-thalassemia	ORPHA:848	Rare anemia	ERN EuroBloodNet	
Specific Thalassemia Quality of Life Instrument (STQOLI)	PRO	No	Adult	- Disease and symptoms (12 items) - Chelation therapy (13 items: (5 items per os chelation therapy users, 5 items for subcutaneous chelation therapy users, and 3 common items)) - Psychosocial impact (10 items) - Transfusion impact (5 items)	Beta-thalassemia	ORPHA:848	Rare anemia	ERN EuroBloodNet	

ERICA PROMs/PREMs repository

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



ERICA

European Rare Disease Research
Coordination and Support Action

For complete information on

PROMs/PREMs

The logo for PROQOLID™ features the word 'PROQOLID' in a bold, sans-serif font. The 'PRO' is in dark blue, and 'QOLID' is in green. The 'Q' is stylized with a white dot in the center. There are three small green dots above the 'Q' and three small white dots above the 'O'. A small 'TM' trademark symbol is at the top right of the 'D'.

PROMs /PREMs Database

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

- Free of access for ERICA members upon request to **Mari Murel** (m.murel@lumc.nl)
- Database content:
 - Author information
 - Conditions of use
 - Translations
 - Population of development
 - PROMs/PREMs Review copy
 - Information on development and validation of PROMs/PREMs

Systematic gathering of evidences via registries associating PROMs and PREMs

ACHIEVEMENTS

Using them for Research

Let's take a concrete example... of
connection among PROMs,
registry, research & increasing
patients quality of life...



European
Reference
Network

for rare or low prevalence
complex diseases

 Network
Hematological
Diseases (ERN EuroBloodNet)

EXAMPLE FROM THE ERN-EUROBLOODNET

How could Researchers benefit from the data collected by the ERNs and the ERN registries

SCD QoL is similar to Cancer: on treatment Vaso-occlusive Pain crises are the Primary reason in emergency departments.

SCD QoL & Pain Crisis

many vaso-occlusive pain crises are managed **at home** with oral medication that includes **OPIOIDS** and supportive care

Differences in output pain management and opioids prescription

How does a new drug / genetic modifier affect on vaso-occlusive pain crisis? What are the **eligibility criteria** for prescribing the drug?

We need PROMs in ERNs Registries

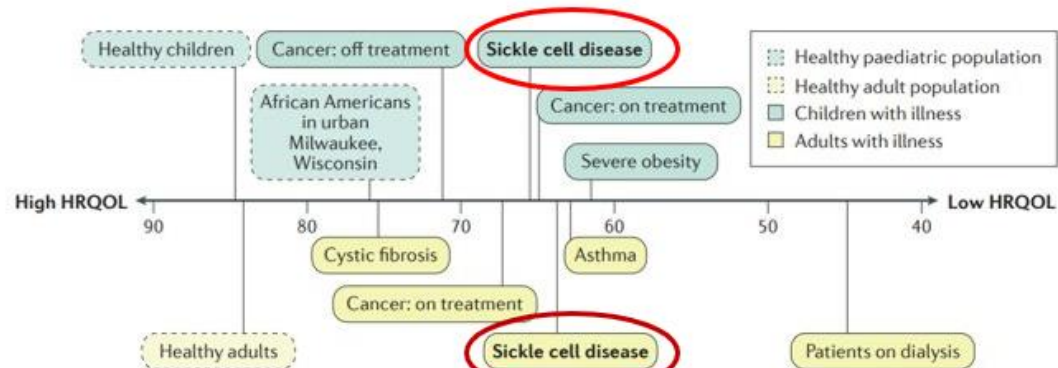


Figure 7 | **Health-related quality of life.** Physical functioning scores measured using the 36-Item Short Form Health Survey (SF-36) and the Pediatric Quality of Life Inventory (PedsQL) generic core scales in healthy individuals and individuals with chronic disease^{237,272}. Scores range from 100, representing the best health-related quality of life (HRQOL), to 0. Specific areas represented in physical functioning scores include the ability to perform all types of physical activities, such as running, walking for a short distance, lifting heavy objects and bathing without help.

Search Reset

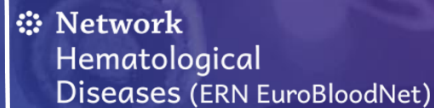
ERICA

Column Visibility ▼ Column Reset

Showing 18 PCOMs/PROMs (of 811 total)

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® (ASCQ-Me®)	PRO	✓ Full	Adult	- Emotional impact (20 items) - Social functioning (17 items) - Pain (13 items) - Stiffness (15 items) - Sleep functioning (12 items)	Sickle cell anemia	ORPHA:232	Rare anemia	ERN_EuroBloodNet	Link
Sickle Cell Pain Diary	PRO	✓ Basic	Adolescent Adult	- Pain	Sickle cell anemia	ORPHA:232	Rare anemia	ERN_EuroBloodNet	Link
Pediatric Quality of Life Inventory™ Sickle Cell Disease Module	PRO or ObsRO	✓ Full	Pediatric Adolescent Adult	- Pain and Hurt (9 items) - Pain Impact (9)	Sickle cell anemia	ORPHA:232	Rare anemia	ERN_EuroBloodNet	Link

Thank You



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