



**EMPOWERING  
MYELOMA ADVOCACY  
ACROSS EUROPE**

# Understanding the challenges around implementing PROMs for patients receiving novel immunotherapies in haematology

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1. Myeloma Patients Europe; 2. Netherlands Comprehensive Cancer Organisation (IKNL); 3. University College London Hospitals NHS Trust; 4. Acute Leukemia Advocates Network; 5. Applied Patient Experience

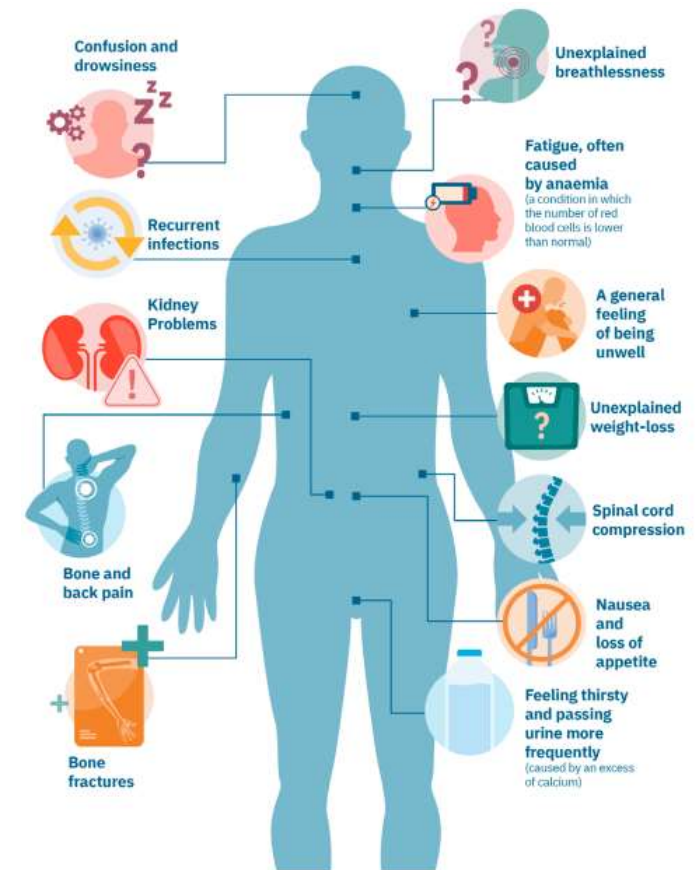
# Financial Disclosures

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## Haematological Malignancies

- Myeloma, Lymphoma, Leukaemia.
- **40-50%** patients relapse and/or develop refractory disease.
- Novel immunotherapies offer innovative treatments
  - **Chimeric Antigen Receptor T cell (CAR-T) therapy.**
  - **Bispecific Antibody treatments.**
- Promising results but potentially serious side-effects.
  - Cytokine Release Syndrome 87% CAR-T, 67% Bispecific antibody treatments
- Expensive medicines ➡ important to evaluate impact on patients in terms of safety, efficacy and patients' quality of life.

## Symptoms of Myeloma



# Patient Reported Outcome Measures (PROMs)

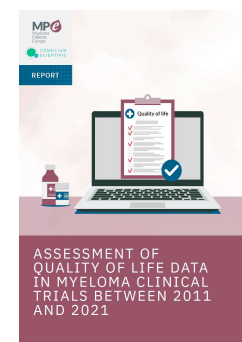
Capture patients' perspectives on their health, symptoms and quality of life

## Commonly used PROMs in myeloma clinical trials (2011-2021)

- EORTC QLQ-C30
- PRO-CTCAE
- EORTC QLQ-MY20
- EQ-5D-3L
- EQ-5D-5L

**Only 33% of global trials, and 41% of European trials intended to collect PRO data.**

**No current validated PROMs for use among people with haematological cancers receiving CAR-T and bispecific antibody treatments.**



**Myeloma Patients Europe (MPE): (2023). *Assessment of quality of life data in myeloma clinical trials between 2011-2021.***

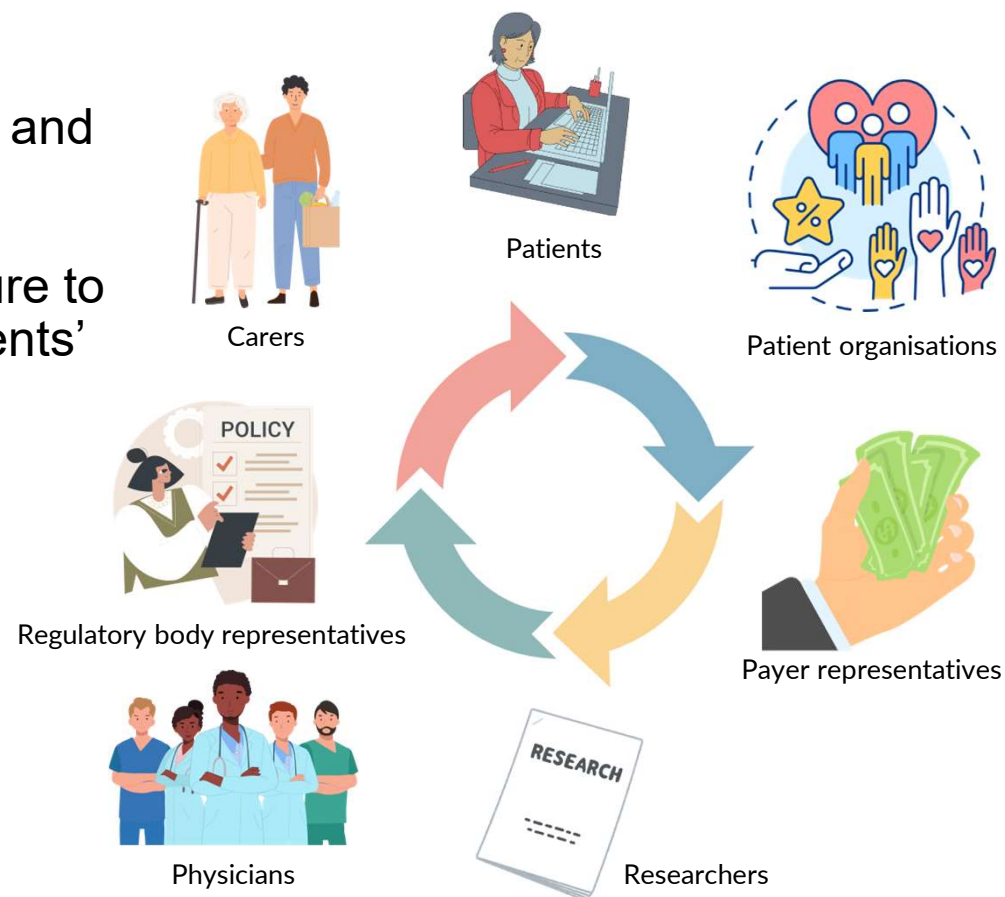
# Understanding the challenges in PROM for Bispecific Antibody and CAR-T treatments in haematology

## Project Aims

1. Understand current experiences, perspectives and challenges.
2. Explore what can be done now and in the future to improve our understanding of haematology patients' experiences of novel immunotherapies.

## Methods

Online workshops x 2 March/April 2024 (n=30)  
Online interviews x13 (n=15)



# Workshop insights

## Patients' experiences of side-effects

### CAR-T

low blood pressure, difficulty concentrating, depression.

Fear and anxiety.



"I lost my sense of smell which I have not regained since."

### CRS

Fatigue, fever, chills, sweating, loss of appetite, taste, loss of smell.



"There were multiple hospitalisations, I was often on steroids. It was a pretty miserable seven weeks."

### Bispecific antibody treatments



"I couldn't open boxes when I was cooking... I always had to ask someone else just because your nails are so frail, that's very painful."

Dizziness, cough, loss of appetite and taste, skin and nails.

Immunocompromised state → social and work impacts.

### No single PROM captures all the domains important to patients treated with novel immunotherapies

No clear guidance on which PROM is best suited leading to disagreement and inconsistency.

Selection of PROMs can be challenging – some regulators and payers may prefer specific PROMs.

Use of multiple PROMs throughout development and during patients' treatment may make assessing QoL difficult across treatments and time.



# Workshop insights

## Challenges on when to collect PROMs

### Timing of PROM collection

#### Baseline assessments

1<sup>st</sup> day in hospital or treatment day 1

And/or when treatment first offered

#### First follow-up

One week after infusion/initiation

#### Subsequent follow-up

First 2 months - weekly

#### Long-term follow-up

CAR-T: every 6 months then annual assessment of broader impact

Bispecific antibodies treatments: longer-term administration context

### Patient burden and feasibility constraints





# Workshop insights

## Improving PROM data collection

### Efficiency

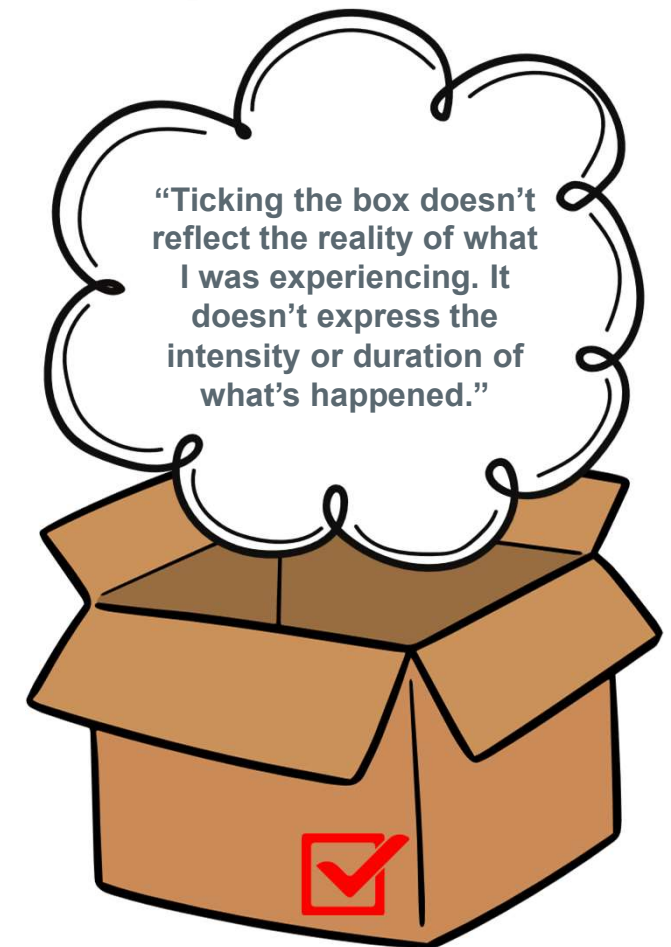
- Integrating data capture into routine clinic visits
- Electronic capture of PROs, apps, websites or a 'bring your own device' but with paper as back-up

### "Whole patient" perspective

- Capturing nuances of patients and carers' experiences and broader impacts including emotional, social and mental health
- Interviews and open-ended responses

### Specific tailored and validated PROM for novel immunotherapies

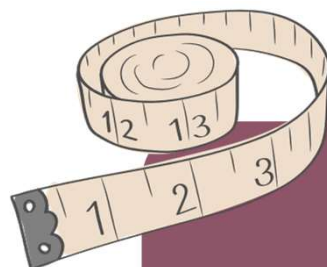
- Multi-stakeholder collaboration essential to develop and refine



## Three key takeaways



**PROMs for CAR-T  
and Bispecifics  
are currently  
inadequate**



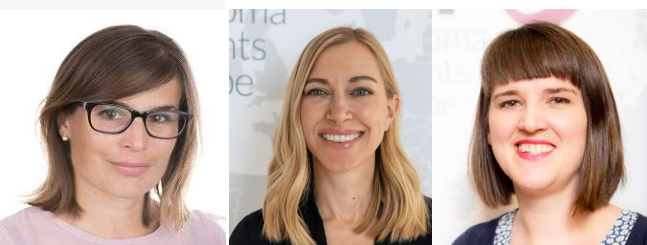
**Tailored and  
timely PROMs  
are essential**



**Collaboration  
and technology  
can improve  
PROMs**

# THANK YOU!

To the participants of  
our workshops and  
interviews, and to our  
collaboration team



## References

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