

INTRODUCTION

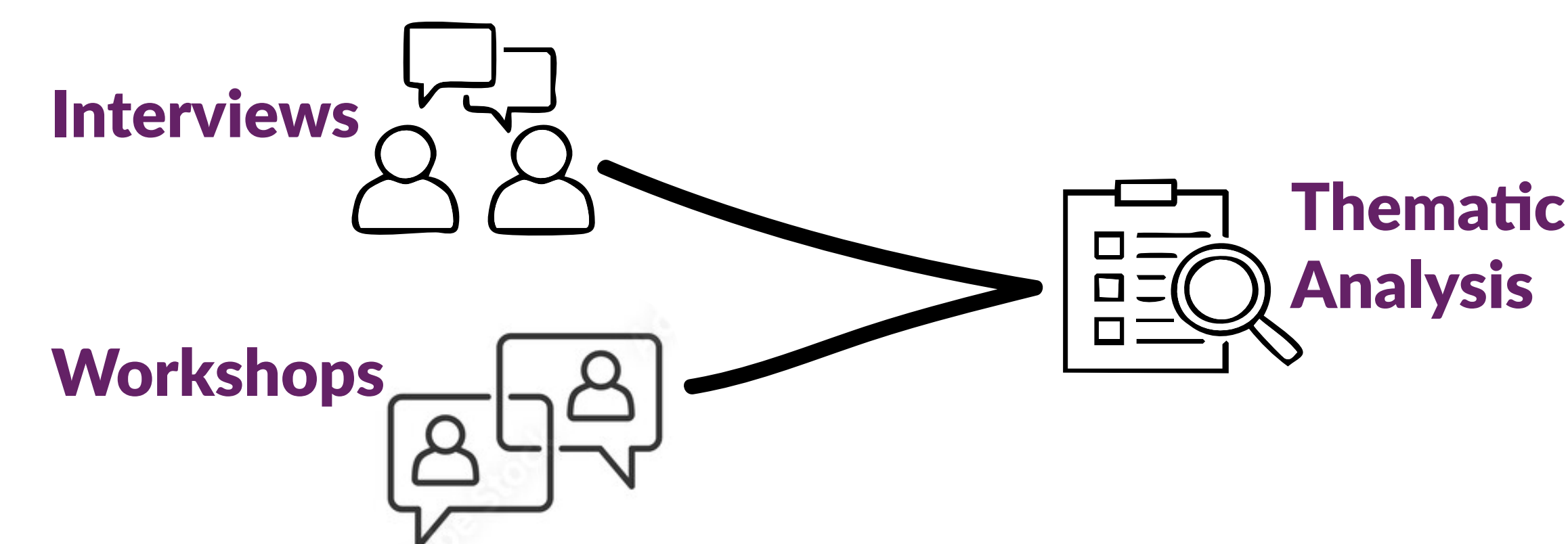
Chimeric antigen receptor T-cell therapy (CAR-T) and T-cell engaging bispecific antibodies (BsAbs) are promising treatments for myeloma, leukemia and lymphoma^{1,2,3}. However, they are associated with specific toxicities, high symptom burden⁴ and have an impact on patients' quality of life (QoL). Adequately capturing patient experiences and the impact of side effects on QoL is both crucial and challenging. Generic Patient Reported Outcome Measures (PROMs) are widely used bringing uncertainty about how well patients' experiences of treatments are captured.

AIM

To understand the challenges in PROMs for BsAbs and CAR-T treatments in haematology from a multi-stakeholder lens and identify opportunities to optimise PROM use in CAR-T and BsAb trials.

METHOD

Myeloma Patients Europe (MPE) conducted online workshops and interviews with patients, carers, clinicians, researchers, patient organisations, industry, regulatory and payer representatives. Thematic analysis was conducted to identify key themes.

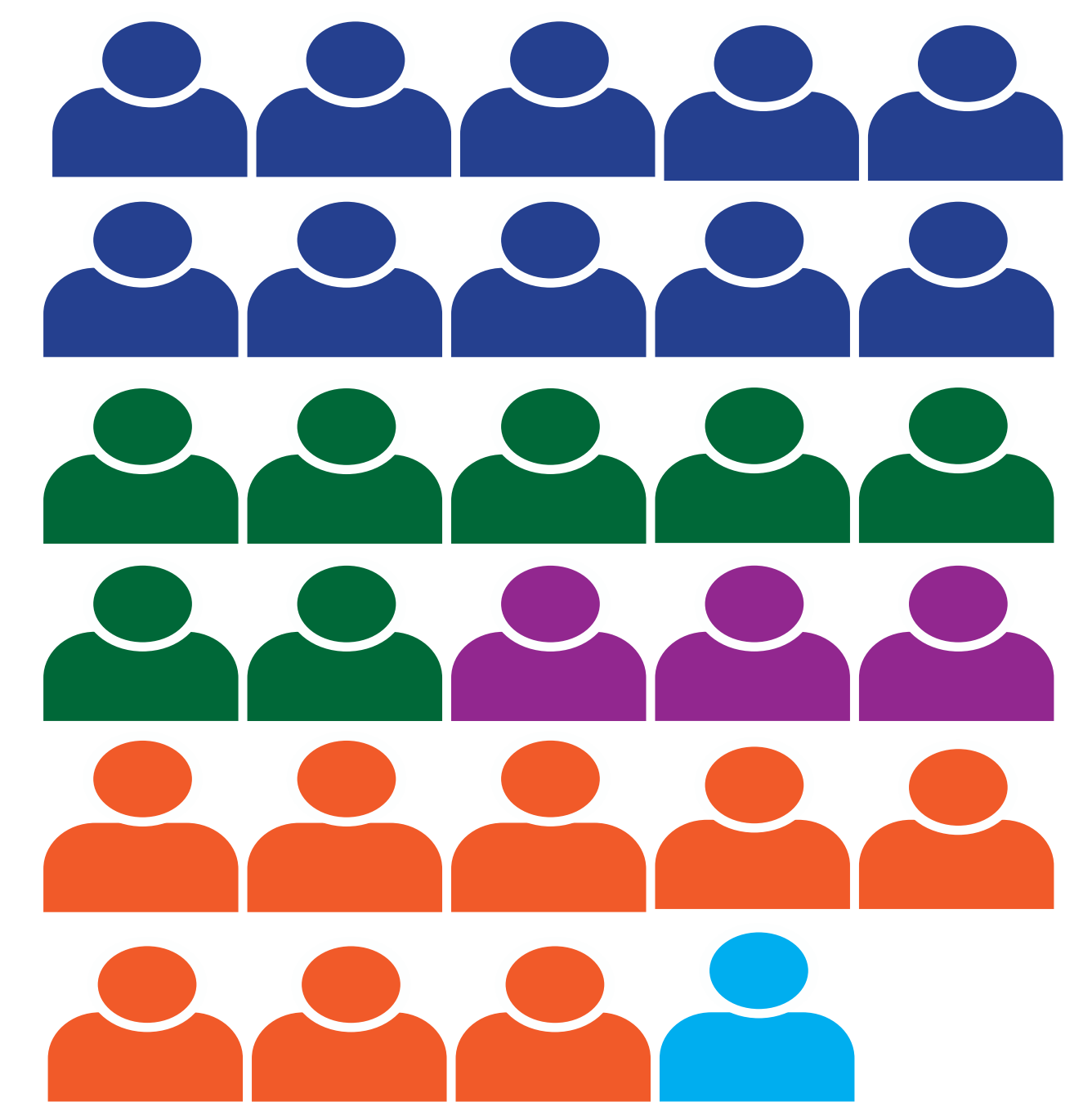


PARTICIPANTS OVERVIEW

29
stakeholders participated in

2
workshops and

16
virtual interviews



Patients, patient organisation representatives
academic researcher, clinician, trialists
HTA representative and regulators
industry representatives and carers

KEY STAKEHOLDER VOICES

“There’s all of these [adverse events] that don’t get captured in existing measures. I think being able to update those measures ... keeping in mind the adverse event profile of contemporary treatments I think is important.”

Academic Researcher

“The doctors and nurses were more interested in the physical side effects more than my mental health. And I think all these measurements that they did, ... about how my physical health worked, and if I can walk or run, if I can be around other people, and if I have the energy to do things, but never about the mental part about it, which was the most difficult part. And my doctor basically told me that we can help you with your body and your, your cancer, but the mental part you’re gonna have to do by yourself.”

Myeloma Patient

“One of the fundamental problems that exists in measuring patient reported outcomes, is when we get comfortable and the regulators and the HTA agencies get comfortable with one particular measure, [it] gets embedded in people’s mind, into the bureaucratic system of the regulatory agencies and HTA bodies, [the PROM] becomes the norm despite their huge limitations”

Industry Representative

INSIGHTS

- PROM selection for CAR-T and BsAb therapies is challenging, and no single PROM captures all the domains that are important to patients.
- Older general established QoL tools dominate despite their limitations.
- Capturing real-world patients’ specific treatment-related experiences and the broader impacts on emotional, social and mental health is missing.

CONCLUSIONS

WHAT DOES THIS MEAN?

New PROMs tailored to CAR-T and BsAbs are required and are in development⁵. However, harmonisation of measures and follow-up timepoints is needed to optimise current PROM use in clinical trials in the interim. Continuing to capture patient-centred data on quality of life is invaluable to help build the evidence base whilst new tools are in development.

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CONTACT INFORMATION

SCAN ME



Myeloma Patients Europe online
 Facebook: mpeurope
 Twitter: @mpeurope
 LinkedIn: Myeloma Patients Europe
 Instagram: MyelomaPatientsEurope
 Email: research@mpeurope.org
 Website: www.mpeurope.org