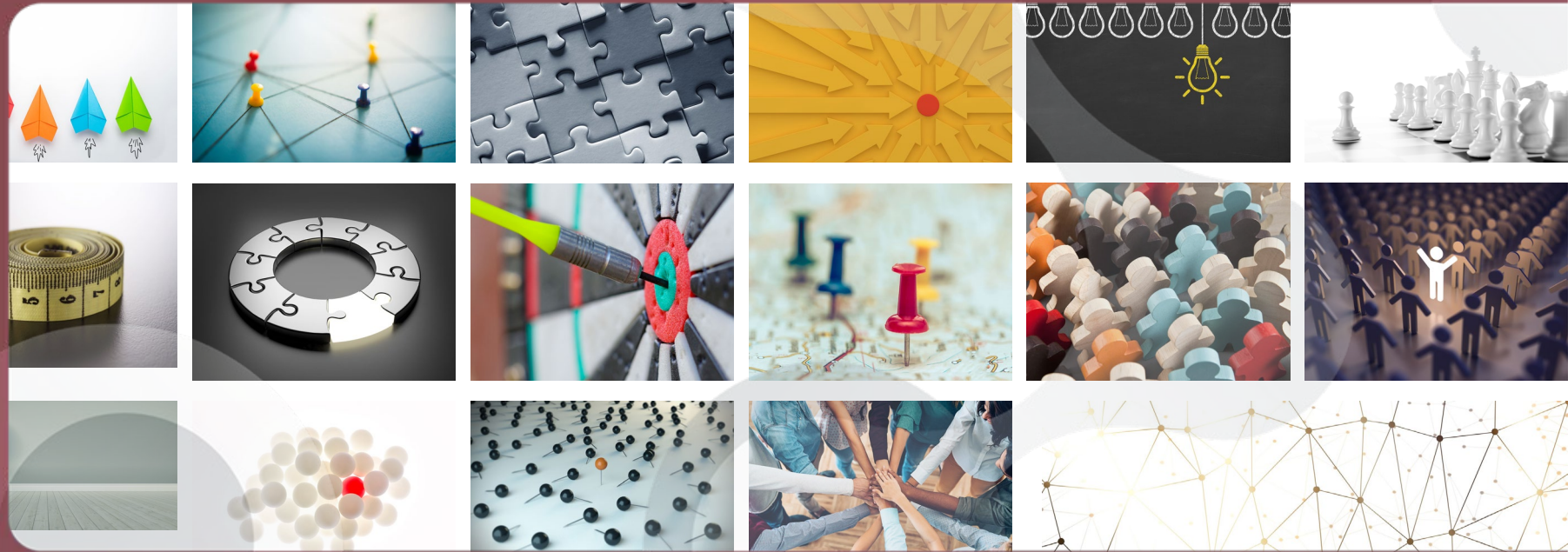




EMPOWERING  
MYELOMA ADVOCACY  
ACROSS EUROPE



# Patient Preference Research

*Kate Morgan, Head of Policy and Access, Myeloma Patients Europe*

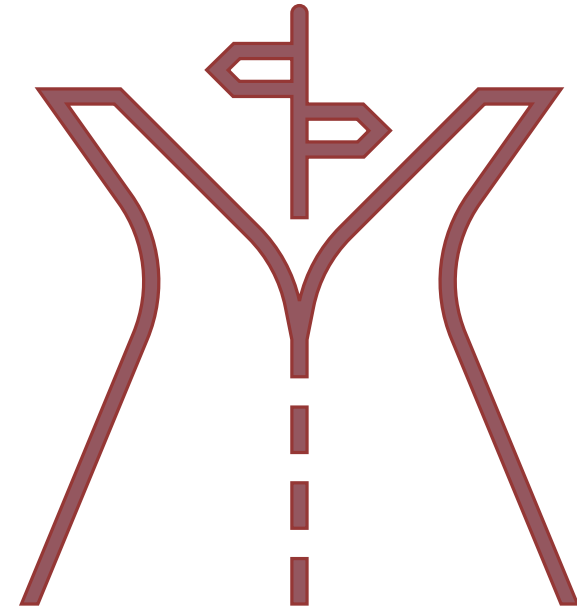
# Definition of patient preferences

## *A patient group perspective*

- Patient preferences relate to data generated directly from patients on how they make **choices** on different treatment options and attributes.
- Patient preference research uses specific methodologies that present patients with real or hypothetical treatment choices. They seek to understand their preferences on specific **treatment and attributes** and to explore the **trade-offs** made on the benefits and risks of a treatment.
- Patient preference data **should not be seen in isolation** of other forms of patient evidence (such as PRO data and patient interviews/surveys).
- Agreeing the definition of types and role of patient-based evidence is important.

Treatment A  
attributes

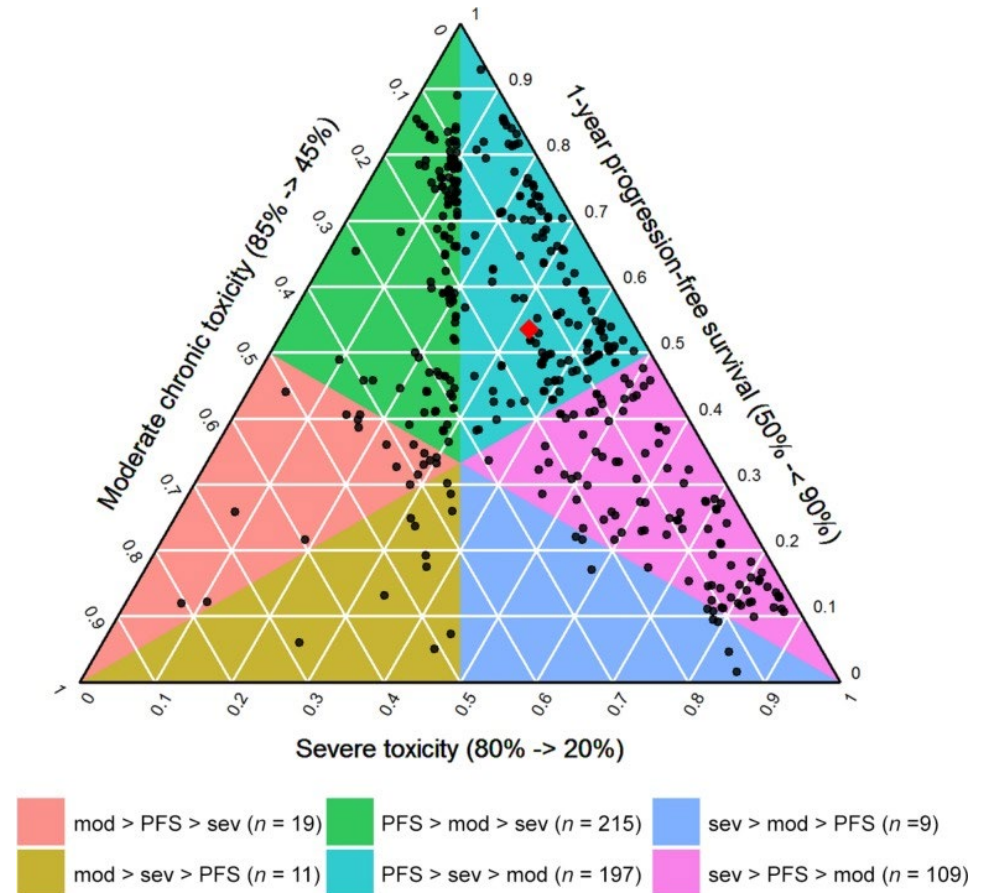
Treatment B  
attributes



# Importance of understanding patient preferences

*Strengthens the patient “demand-side”*

- **Patients are the end users of medicines.** Preference research and data helps us better understand what patients want from treatment.
- Patients have **different experiences**, perspectives and wants from their treatment. It cannot be assumed all patients want the same thing.
- For example, Myeloma UK used multi-criteria decision-making analysis to elicit the preferences of **560 patients** with myeloma regarding the possible benefits and risks of treatments.
- Participants who gave a higher weight to severe or life-threatening toxicity were more **frequently younger**, working, and looking after **dependent family members** and had **more frequently experienced severe or life-threatening side effects**.



# Importance of understanding patient preferences

## Differences across diseases

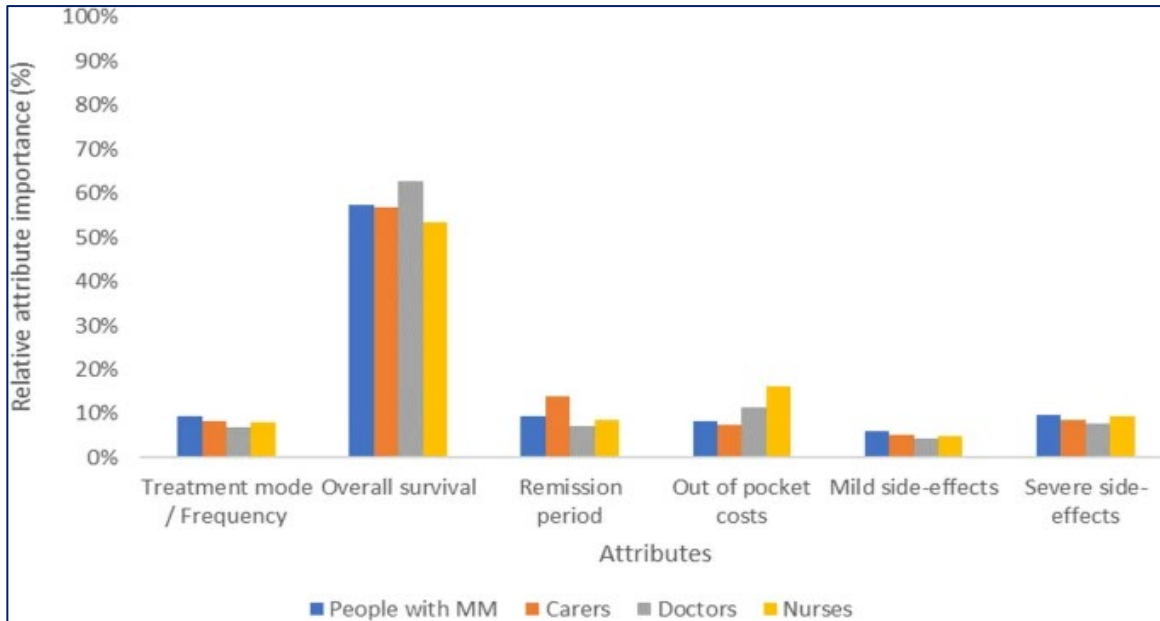
- Patient preference elicitation can be important for diseases like myeloma where:
  - Many treatments are combinations.
  - Many different forms of administration.
  - Different treatment options, at different disease stages.
- A discrete choice experiment run by Myeloma UK with 475 patients found that patients who were diagnosed in the last 5 years placed a greater emphasis on survival.
- Patients who had been on treatment for longer (i.e., not diagnosed recently) placed more importance on the mode/frequency of treatment.
- **Suggests that patients have different preferences depending on the stage of disease and prior experience and that preferences may change over time.**

Factors	Treatment A	Treatment B	Neither of these treatments
Average overall survival	7 years 	3 years 	
Average remission period	5 years 3 months 	9 months 	
Mild or Moderate side-effects	60 out of 100 (60%) risk 	20 out of 100 (20%) risk 	
Severe side-effects	5 out of 100 (5%) risk 	10 out of 100 (10%) risk 	
How treatment is taken	Intravenous drip (Hospital / clinic) Time: 2-3 hours	Subcutaneous Injection (Hospital / clinic) Time: 15 mins	
Frequency of taking treatment			
Average out of pocket costs to you over a year	£0	£0	
I would choose	<input type="radio"/> Treatment A	<input type="radio"/> Treatment B	<input type="radio"/> Neither



# Importance of understanding patient preferences


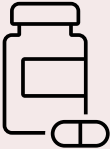

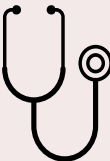
## Overcoming assumptions



- Assumptions are often made on what patients prefer.
- Another patient preference study in myeloma compared treatment preferences of myeloma patients, to carers, haematologists, and nurses.
- 124 patients, 44 carers, 28 haematologists, and 34 nurses.
- Overall survival was the most important attribute across all participant groups. However, physicians placed more importance on overall survival than other groups.
- **If we want to know what patients think, we need to ask them!**

# How can patient preference data be used?

From clinical development through to the clinic

Stage	Idea for use
<b>Clinical development</b> 	<ul style="list-style-type: none"><li>• Appropriate selection of endpoints in clinical trials (e.g. PFS vs OS vs QoL).</li><li>• Is a treatment acceptable to patients? (e.g. CAR-T).</li></ul>
<b>Regulation</b> 	<ul style="list-style-type: none"><li>• Benefit risk assessments by EMA.</li><li>• Frames and provides context for decision-making (in a more robust way).</li></ul>
<b>Reimbursement</b> 	<ul style="list-style-type: none"><li>• Answer specific questions from committees (such as value patients place on administration, survival gains or QoL).</li><li>• Is the treatment acceptable to patients?</li></ul>
<b>Clinical practice</b> 	<ul style="list-style-type: none"><li>• Doctors ensure they discuss relevant questions with patients in their decision-making.</li><li>• Inform and interpret clinical guidelines (e.g. EHA – ESMO myeloma guidelines).</li></ul>

**Listening to patients at each stage of drug development is extremely important!**

# Challenges for patient preference research and data

What we have learned from our experience at MPE....

## Understandability

- Difficult for patients to understand and potentially emotive.
- Some methodologies are not patient friendly.
- Sometimes patient friendly is not methodologically correct.
- Recruitment challenges in different countries.

**Need to explain to patients correctly and involve them!**

## Interpretability and applicability

- What constitutes a robust sample?
- Can you apply data gathered in one country to patients in others?
- Do patient preferences hold true at different time points?

**Partnerships are important!**

## Responsibility and strategy

- Patient preference data has happened in small pockets to-date – no clear strategy.
- Who has responsibility for these types of study?
- What data and questions do industry, EMA and HTA need to know?

**Clear guidance and collaboration is needed!**

**Conclusion:  
Patient preference data is an important  
part of the puzzle of patient  
involvement.**

**Most issues can be addressed through  
effective research design,  
collaboration and dialogue between  
different stakeholders.**