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SCIENCE MEDICINES HEALTH

## Patient Involvement in Patient Preference Studies: a Case Study in Multiple Myeloma

CDDF multi-stakeholder workshop on involving patients in oncology drug development  
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### **Rosanne Janssens**

PhD researcher, Regulatory Sciences and Pharmaco-Economics, KU Leuven  
Seconded national expert, Oncology, Haematology and Diagnostics, EMA

### **Nicole Wicki**

Patient advocacy manager, Myeloma Patients Europe (MPE)

**KU LEUVEN**

**prefer.**  
PATIENT PREFERENCES

**MPe**  
Myeloma  
Patients  
Europe

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# Patient preferences: what's in a name?

“Qualitative or quantitative statements of the **relative** desirability or acceptability to patients of specified **alternatives or choices** among outcomes or other attributes that differ among alternative health interventions”



**Patient preferences**, measured through **patient preference studies** indicate:

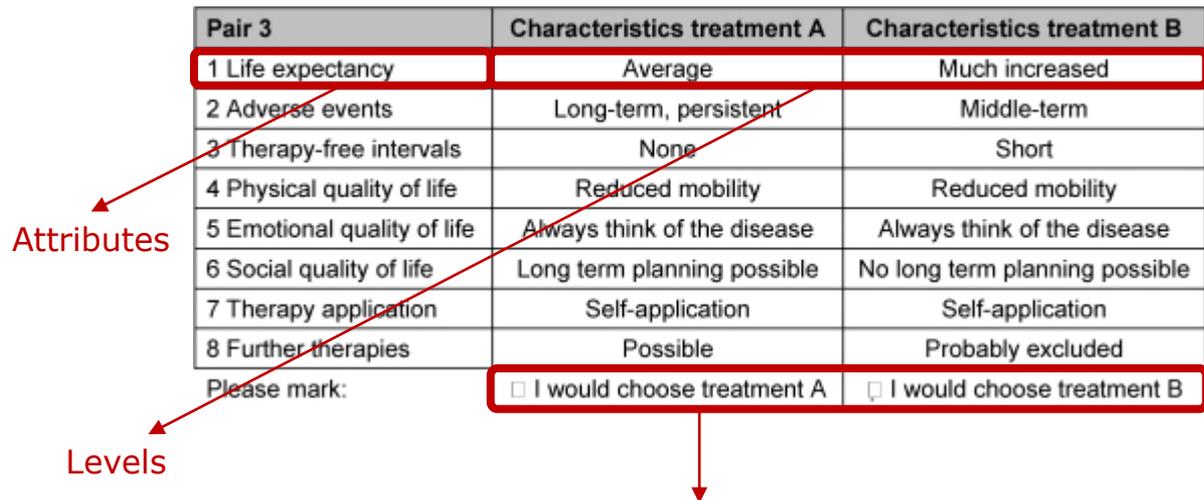
1. WHAT patients find important (**clinical**) **outcomes** of a medical product
2. HOW MUCH **patients value these outcomes**
3. DIFFERENCES in patient preferences according to patient characteristics (**heterogeneity**)

# Patient preference study: an example

| Pair 3                      | Characteristics treatment A                         | Characteristics treatment B                         |
|-----------------------------|---|---|
| 1 Life expectancy           | Average   | Much increased                                      |
| 2 Adverse events            | Long-term, persistent                               | Middle-term   |
| 3 Therapy-free intervals    | None  | Short   |
| 4 Physical quality of life  | Reduced mobility                                    | Reduced mobility                                    |
| 5 Emotional quality of life | Always think of the disease                         | Always think of the disease                         |
| 6 Social quality of life    | Long term planning possible                         | No long term planning possible                      |
| 7 Therapy application       | Self-application                                    | Self-application                                    |
| 8 Further therapies         | Possible  | Probably excluded                                   |
| Please mark:                | <input type="checkbox"/> I would choose treatment A | <input type="checkbox"/> I would choose treatment B |

Attributes

Levels



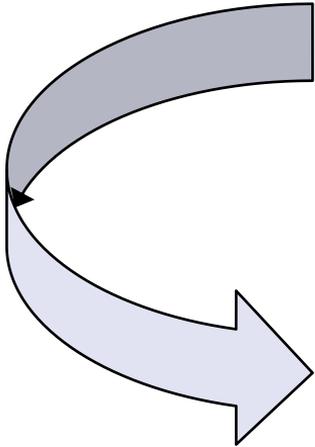
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## Multi-stakeholder barriers hamper formal use of preference study results

### **A lack of guidance on how to design patient preference studies aiming to inform decision-making:**

1. How to select a preference method?
2. How to select attributes?
3. How to select a representative sample?
4. How to measure and deal with preference heterogeneity?
5. How to reduce the cognitive burden of preference surveys?
6. How to ensure patients understood the questions?
7. How to avoid bias in preference studies?

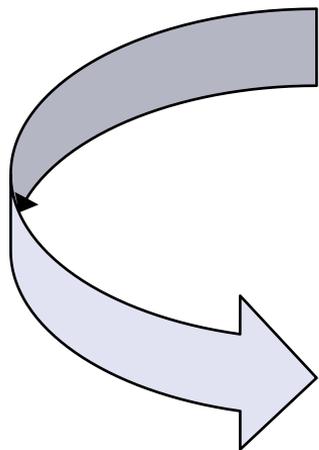


### **Questions on the validity, reliability, reproducibility and generalizability of preference study results:**

*"Can decision-makers trust the results?"*

## Multi-stakeholder barriers require a multi-stakeholder approach

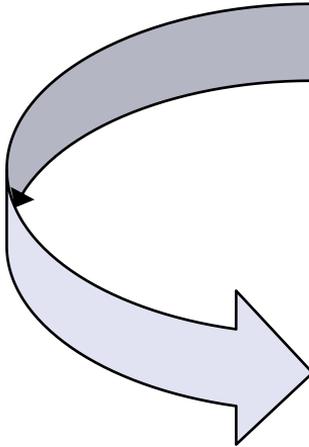
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**Involve patients (organizations), academics, clinicians and decision-makers to increase the validity, reliability, reproducibility and generalizability of preference study results**

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# Preference study in Multiple Myeloma: research objectives

## Methodological

1. To compare preferences elicited by 2 different preference methods (DCE vs SW)
2. To understand how preferences vary with patient and disease characteristics

Multiple Myeloma (MM) is characterized by the development of novel (combination) therapies with **complex benefit-risk profiles**

Uncertainty on the **value of these treatments according to patients:**

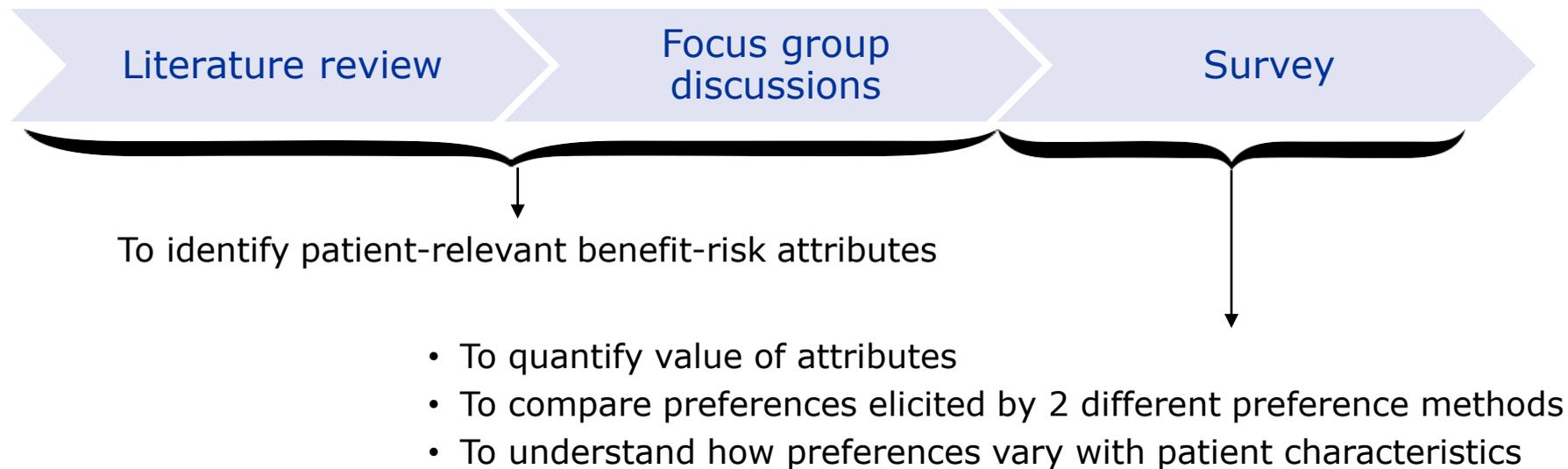
- Importance of clinical outcomes
- Patients' views on acceptable benefit-risk ratios

## Clinical

1. To identify and quantify patient-relevant benefit-risk attributes of MM treatments
2. To quantify the value of benefit-risk attributes of MM treatment



## Overview of steps in preference study



# Opportunities for patient involvement in preference study



- **Increasing the quality of the study protocol:**
  - Understanding the clinical context of the disease
  - Identification of patient-relevant attributes
  - Feasibility of qualitative and quantitative method
  - Amount and type of information given to patients
  - Understandability of questions and attributes
- **Organizational:**
  - Patient recruitment
  - Identifying clinical partners
- **Increasing the quality of the interpretation of the results:**
  - Interpretation of the results (workshop): what do they mean, why are they there?
  - Disseminating study results back to patient community

## Challenges and learnings



- **Ensure understanding of the aims and nature of the study**
  - Preference methods and related documents are complex!
- **Involve patients at regular time points of the study**
  - To avoid an overload of information at once
- **Ask for specific input**
  - E.g. on 'translating' the documents and information to patient-friendly language
- **Recognize their efforts and compensate for these efforts**
  - E.g. via increased visibility: presentations, workshops

# Myeloma Patients Europe Perspective

# Patient perspective: the importance of preference studies

- Patient advocates routinely asked for perspectives on patient values (e.g. to support regulatory and reimbursement decisions)
- Move away from anecdotal evidence and evidence based on the views of a handful of patients – **robust data is important**
- Need to understand emphasis patients put different factors (survival, QoL, administration methods; psychosocial issues)
- In myeloma, the treatment pathway is crowded and resources are becoming increasingly limited – prioritisation is important, as is more personalised care

What do patients value?

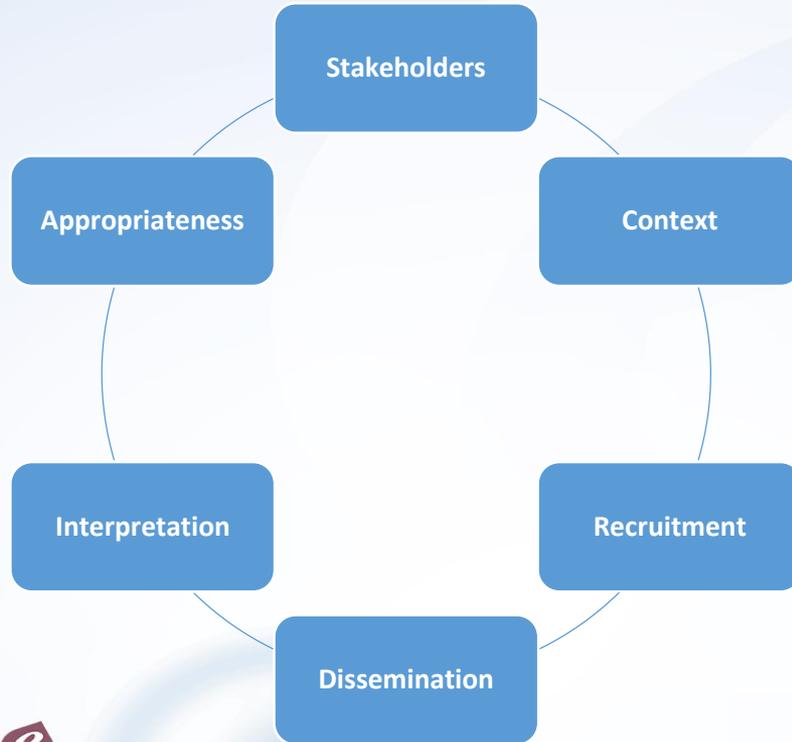


# Patient advocates are increasingly taking the lead in developing preference studies Collaborations are very important for scientific rigour and utility

- Academics can assist on selecting the best methodology for patient preference studies
  - Discrete choice vs. multi-criteria decision-making analysis (MCDA)
  - Quantitative vs. qualitative analysis vs. both?
- Scientific rigour is often difficult to navigate for patient advocates, so academic support on this is crucial
  - What constitutes a “robust” sample or results?
  - How do you ask questions in the right way?
  - When does a survey need to go through ethics approval? Why, where and how?
  - Eligibility criteria
  - Peer review and publication
- Data analysis, storage and ownership is complex



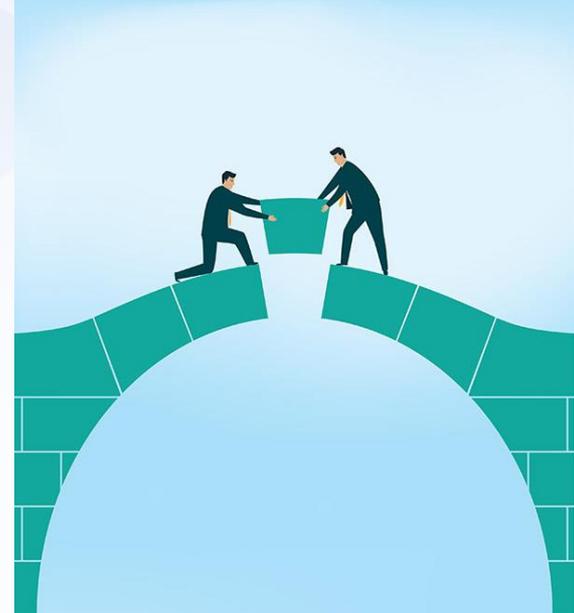
# Role of MPE: ensuring content is accurate from a disease perspective, appropriate for patients, and data generated is useful to the myeloma and patient advocacy community



- Well placed given knowledge of the disease pathway and direct interaction with patients
- Utilise prior knowledge and experience (e.g. pilot)
- Review and guide on what is acceptable to patients (e.g. work on protocol and focus groups)
  - Survey acceptability (length, complexity)
  - Sensitivity (e.g. survival questions)
- Access to networks
- Utilise findings external engagement of MPE with industry, regulators, payers and policy-makers
- Inform the patient preference strategy of MPE moving forward

# Reflections...

- From MPE experience, collaboration with academia and patient groups has been important as each partner has different expertise to bring to the table
- A partnership between the different stakeholders bridges the gaps in each others knowledge and allows for the development of robust and useful data on patient preferences





## Study contributors

- Ana Vallejo (Myeloma Patients Europe)
- Ananda Plate (Myeloma Patients Europe)
- Anneleen Vanhellemont (KU/UZ Leuven)
- Douwe Postmus (University of Groningen)
- Hilde Stevens (I3h, Université libre de Bruxelles)
- Isabelle Huys (KU Leuven)
- Kate Morgan (Myeloma Patients Europe)
- Martina Vandebroek (KU Leuven)
- Michel Delforge (KU/UZ Leuven)
- Steven Simoens (KU Leuven)
- Rosanne Janssens (KU Leuven)



# Any questions, comments or ideas?

Email at [rosanne.janssens@ema.europa.eu](mailto:rosanne.janssens@ema.europa.eu)