Patient Preference Research

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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.
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**.

Postmus D et al. Oncologist 2018;23(1):44-51
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.
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

<table>
<thead>
<tr>
<th>Stage</th>
<th>Idea for use</th>
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<tbody>
<tr>
<td><strong>Clinical development</strong></td>
<td>• Appropriate selection of endpoints in clinical trials (e.g. PFS vs OS vs QoL).</td>
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<td></td>
<td>• Is a treatment acceptable to patients? (e.g. CAR-T).</td>
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<td><strong>Regulation</strong></td>
<td>• Benefit risk assessments by EMA.</td>
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<td>• Frames and provides context for decision-making (in a more robust way).</td>
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<td><strong>Reimbursement</strong></td>
<td>• Answer specific questions from committees (such as value patients place on administration, survival gains or QoL).</td>
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<tr>
<td></td>
<td>• Is the treatment acceptable to patients?</td>
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<td><strong>Clinical practice</strong></td>
<td>• Doctors ensure they discuss relevant questions with patients in their decision-making.</td>
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<td>• Inform and interpret clinical guidelines (e.g. EHA – ESMO myeloma guidelines).</td>
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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.