How patient preferences can contribute to development and regulation of medicines

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Involving patients versus patients’ preferences

- Every patient has wishes, needs, values and preferences

- Involving 1-2 or small group of patients versus perspective of representative sample?

- Likert-scale survey?

<table>
<thead>
<tr>
<th></th>
<th>Very important</th>
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<td>Effectiveness</td>
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- Pairwise comparisons!
  - Quantifying the relative importance of different aspects of a good or service

FDA guideline for the evaluation of devices for market approval
Methods for measuring patient preferences

- **Preference elicitation**
  - **Discrete Choice Based related techniques**
    - Discrete Choice Experiment/Best-Worst scaling Type 3
    - Adaptive Conjoint Analysis
    - Self-explicated conjoint
    - Measure of Value
  - **Threshold related techniques**
    - Standard Gamble
    - Time Trade-off
    - Person Trade-off
    - Starting Known Efficacy
    - Test Trade-off
    - (Probabilistic) Threshold Technique
    - Contingent valuation
  - **Rating related techniques**
    - Constant Sum Scaling
    - Repertory Grid Method
    - Analytic Hierarchy Process
    - Starting Known Efficacy
    - Swing Weighting
  - **Ranking related techniques**
    - Qualitative Discriminant Process
    - Q-methodology
    - Control Preferences Scale
    - Best-Worst scaling Type 1, 2

Soekhai et al., 2019, Value in Health
The medical product lifecycle: when and how patient preferences can inform decisions

<table>
<thead>
<tr>
<th>MPLIC</th>
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<td>Pre-discovery</td>
<td>Select &amp; prioritise targets and leads</td>
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Whichello et al., 2020, Health Policy
Guidance needed on how to elicit preferences

- ISPOR task forces and special interest groups
- Erasmus Choice Modelling Centre
- Smarter Choice Better Health
- International Academy for Health Preference Research

- PREFER project: 5-year project focusing aiming to
  - A five-year project that has received funding from the Innovative Medicines Initiative 2 Joint Undertaking which received support from the European Union's Horizon 2020 research and innovation programme and the European Federation of Pharmaceutical Industries and Associations (EFPIA). A total of 31 partners from academia and industry working together.
PREFER recommendations: framework

Framework Components

1. Study purpose
2. Study objectives
3. Team expertise
4. Study timing
5. Organisation
6. Ethics & good practice
7. Study population
8. Method selection & analysis planning
9. Sample size
10. Preference question design
11. Conduct
12. Pilot study
13. Recruitment
14. Data collection
15. Analysis, interpretation
16. Write-up in study report
17. Return results to patients and researchers
18. Applying patient preference data to inform decision-making

Classified as public by the European Medicines Agency
PREFER recommendations: key messages

• Involve patients, HTA and regulators in preference studies as early as possible
• Decide on a preference elicitation method based on research question, MPLC stage, population size and resources
• Include evidence-based or theoretically sound psychological characteristics in a preference study (consider at least health literacy or numeracy)
• Select educational materials based on the specific educational needs of the participants and develop this material using a systematic and evidence-based approach
Concluding remarks and future directions

• Methodological development of preference methods
  o Evidence-based guidance
  o External validity
  o Internal validity & data quality

• Continued dialogue with decision makers in MPLC
  o Ongoing assessment of trust in methods and outcomes
  o Moving beyond current decision frameworks

• Building foundation for full integration of patients’ preferences in decision-making along the medical product lifecycle
Thank you

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- www.eur.nl/ecmc
- www.imi-prefer.eu