Patient Preferences in Benefit-Risk Assessments during the Drug Life Cycle

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Health preference research

1. What matters to patients?
2. How much does it matter?
3. What matters most?

At different decision points in the medical product life cycle.

Well informed and reflected if used in regulatory decisions.
Stepwise approach

- Assess methods
- Conduct clinical case studies
- Develop recommendations
Results

- Through literature review, 143 semi-structured interviews and focus groups with stakeholders:
  - lack of consensus:
  - on definition and role of patient preferences
  - on the study design, and conduct of patient preference studies
  - Patients want to be well-informed and that heterogeneity is acknowledged
- 32 preference exploration and elicitation methods identified that have been applied to the development of medicinal products and medical devices.
- Although the use of patient preferences is desired by stakeholders, their concerns and requirements need to be addressed before patient preferences can be integrated throughout the medical product life cycle
## Example Questions for Case Studies

<table>
<thead>
<tr>
<th>Concerns</th>
<th>Research question</th>
<th>Case Studies</th>
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<tbody>
<tr>
<td><strong>Industry</strong></td>
<td>To what degree does repeat application of a method on different samples from the same population give similar results?</td>
<td>12 high potential PP methods to use</td>
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<tr>
<td>Patient sample</td>
<td></td>
<td>At least 3 disease case study teams to fit PREFER methodological questions with clinical questions (lung cancer, reuma arthritis, and neuromuscular disorders)</td>
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<tr>
<td><strong>Regulator</strong></td>
<td>How generalizable are preferences from one specific population in a disease to different populations in that or related diseases?</td>
<td>Simulation studies</td>
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<tr>
<td>Generalizability of the results</td>
<td></td>
<td>--------------------------------------------------------------------------------------------------------</td>
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<tr>
<td><strong>HTA</strong></td>
<td>To what degree do small changes in the number, type and definitions of attributes impact results for a given method</td>
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<td>Study design (e.g. risk of influencing PP research)</td>
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<tr>
<td><strong>Patient</strong></td>
<td>Does a serious game improve the understandability of questions in preference studies significantly without impacting the results?</td>
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<tr>
<td>Lack of patient knowledge and education</td>
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An RA (side) study (Karin Schölin Bywall)

- What do RA-patients prefer when stepping up RA treatment with (another) biologic or JAK-inhibitor?
- Will interactive information affect preferences?
- Literature and expert reviews, focus groups:
  - Attributes: route of administration, frequency of administration, reduction of disease activity, improvement in functional capacity, seriousness of side effect, chance of getting a side-effect, monthly costs for society and number of sick days.
- Patient partners
- DCE based on Swedish RA-registry (N=5000)