Patient Preference Information – What It Is and What It Is Not

A webinar in preparation for the FDA-ISPOR Summit on Using Patient Preference Information in Medical Device Regulatory Decisions

March 4, 2020
Background

• This webinar is intended to provide background information about patient preference information as both:

  – a stand-alone educational event

    AND

  – as an introduction to (but not requirement for) the upcoming ISPOR-FDA Summit 2020
ISPOR-FDA Summit 2020

• Using Patient-Preference Information in Medical Device Regulatory Decisions: Benefit-Risk and Beyond
• March 31, 2020
• FDA White Oak Campus or Via Livestream
• https://www.ispor.org/conferences-education/conferences/upcoming-conferences/isporsfda-summit-2020
<table>
<thead>
<tr>
<th>Time</th>
<th>Topic</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Introduction</strong></td>
<td></td>
</tr>
<tr>
<td>12:00 – 12:05</td>
<td>Introduction in context of upcoming FDA/ISPOR PPI Summit. Introduction of speakers</td>
</tr>
<tr>
<td></td>
<td>Learning objectives</td>
</tr>
<tr>
<td><strong>Overview of Patient Preference Methods</strong></td>
<td></td>
</tr>
<tr>
<td>12:05 – 12:25</td>
<td>What PPI and what PPI is not</td>
</tr>
<tr>
<td></td>
<td>Overview of stated-preference methods</td>
</tr>
<tr>
<td><strong>Developing a Patient Preference Study</strong></td>
<td></td>
</tr>
<tr>
<td>12:25 – 12:45</td>
<td>Overview of core components in designing a stated-preference study</td>
</tr>
<tr>
<td></td>
<td>Example of findings and interpretation from a stated-preference study</td>
</tr>
<tr>
<td></td>
<td>Recommended qualities of patient preference studies (from PPI Guidance)</td>
</tr>
<tr>
<td><strong>Wrap up</strong></td>
<td></td>
</tr>
<tr>
<td>12:45 – 1:00PM</td>
<td>Q&amp;A</td>
</tr>
<tr>
<td></td>
<td>Additional Resources</td>
</tr>
</tbody>
</table>
Welcome and Introductions

• Moderator

Michelle Tarver
Director, Patient Science & Engagement Program
Center for Devices and Radiological Health (CDRH)
US Food and Drug Administration

• Presenters

Brett Hauber
Senior Economist and Vice President, Health Preference Assessment, RTI Health Solutions
Affiliate Associate Professor, CHOICE Institute, University of Washington School of Pharmacy

Deborah A Marshall
Canada Research Chair, Health Services and Systems Research Professor, Department of Community Health Sciences, Cumming School of Medicine, University of Calgary
Learning Objectives

- Become familiar with the range of methods that are included among stated-preference methods
  - Recognize the difference between patient-preference information and other types of input
  - Identify the core components in designing and implementing a stated-preference project
  - Become familiar with the recommended qualities of patient preference studies considered when determining whether PPI constitutes valid scientific evidence
Overview of Patient Preference Methods
Patient Preference Information (PPI)

• qualitative or quantitative assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions

  Relative: Preferences are not absolute measures in a vacuum. How much something is preferred can only be understood relative to something else (e.g., I prefer this set of outcomes to that set of outcomes).

  Desirability: how much to I want the good outcomes

  Acceptability: how much of a bad outcome am I willing to accept

  Attributes that differ: One option can only be preferred to another if it differs from the other somehow

Thinking About Patient Preferences

- Guidance from CDRH discusses patient input\(^1\)

Thinking About Patient Preferences

- Guidance from CDRH discusses patient input

Patient Input

Patient Perspectives

Patient preference

Patient-reported outcomes

Patient Perspectives in the CDRH Guidance is analogous to Patient Experience Data in 21st Century Cures

Thinking About Patient Preferences

- Guidance from CDRH discusses patient input\(^1\)

Patient preference and patient reported outcomes are two types of patient input

---

\(^1\) FDA Final Patient Preference Guidance Document. August 24, 2016. [https://www.fda.gov/media/92593/download](https://www.fda.gov/media/92593/download)
PPI is Different than Patient Reported Outcomes

- **PPI**
  qualitative or quantitative assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions.

- PPI is an assessment of desirability or acceptability (i.e., what a patient wants).

- **PRO**
  any report of the status of a patient’s health condition that comes directly from the patient, without interpretation of the patient’s response by a clinician or anyone else.

- PRO is a measure of a realized outcome (i.e., what it is or what it is like).

---

PPI is Different than Multi-Criteria Decision Analysis

• **PPI**
  qualitative or quantitative assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions

• **MCDA**
  A methodology for appraising alternatives on individual, often conflicting criteria, and combining them into one overall appraisal
  It is a process for decision making
  PPI can be an input into MCDA used to provide weights for the outcomes

PPI is Different than the Quality-Adjusted Life Years

- **PPI**
  qualitative or quantitative assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions

- **QALY**
  A measure of health outcome which assigns to each period of time a weight, ranging from 0 to 1 corresponding to the quality of life during that period…
  Weights, or health-state utilities, are a form of stated preference.
  PPI can be used to provide weights

1 FDA Final Patient Preference Guidance Document. August 24, 2016. [https://www.fda.gov/media/92593/download](https://www.fda.gov/media/92593/download); 2 Garber et al., in Cost-Effectiveness in Health and Medicine (Gold et al., eds.) 1996.
3 Health-state utilities most often reflect public preferences rather than patient preferences.
Overview of Stated Preference Methods
There are multiple methods to elicit patient preference information

MDIC Catalog of Methods

<table>
<thead>
<tr>
<th>Method</th>
</tr>
</thead>
<tbody>
<tr>
<td>Structured-weighting</td>
</tr>
<tr>
<td>Simple direct weighting</td>
</tr>
<tr>
<td>Ranking exercises</td>
</tr>
<tr>
<td>Swing weighting</td>
</tr>
<tr>
<td>Point allocation</td>
</tr>
<tr>
<td>Analytic hierarchy process</td>
</tr>
<tr>
<td>Outranking methods</td>
</tr>
<tr>
<td>Health-state utility</td>
</tr>
<tr>
<td>Time tradeoff</td>
</tr>
<tr>
<td>Standard gamble</td>
</tr>
<tr>
<td>Stated-preference</td>
</tr>
<tr>
<td>Direct-assessment questions</td>
</tr>
<tr>
<td>Threshold technique</td>
</tr>
<tr>
<td>Conjoint analysis and discrete-choice experiments</td>
</tr>
<tr>
<td>Best-worst scaling exercises</td>
</tr>
<tr>
<td>Revealed-preference</td>
</tr>
<tr>
<td>Patient-preference trials</td>
</tr>
<tr>
<td>Direct questions in clinical trials</td>
</tr>
</tbody>
</table>

IMI-PREFER Compendium of Methods


### Discrete-Choice Experiments

- Patients are asked to make choices

<table>
<thead>
<tr>
<th>Medicine Features</th>
<th>Medicine A</th>
<th>Medicine B</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pain while moving around one hour after taking the medicine</td>
<td><img src="#" alt="Graph" /></td>
<td><img src="#" alt="Graph" /></td>
</tr>
<tr>
<td>Pain while sitting, lying down, or sleeping one hour after taking the medicine</td>
<td><img src="#" alt="Graph" /></td>
<td><img src="#" alt="Graph" /></td>
</tr>
<tr>
<td>Stiffness one hour after taking the medicine</td>
<td><img src="#" alt="Graph" /></td>
<td><img src="#" alt="Graph" /></td>
</tr>
<tr>
<td>Difficulty doing your daily activities one hour after taking the medicine</td>
<td><img src="#" alt="Graph" /></td>
<td><img src="#" alt="Graph" /></td>
</tr>
<tr>
<td>Chance of a bleeding ulcer requiring an operation within the next year because of the medicine</td>
<td><img src="#" alt="Graph" /></td>
<td><img src="#" alt="Graph" /></td>
</tr>
<tr>
<td>Additional chance of a stroke within the next 5 years because of the medicine</td>
<td><img src="#" alt="Graph" /></td>
<td><img src="#" alt="Graph" /></td>
</tr>
</tbody>
</table>

**Which medicine would you choose if these were the only medicines available?**

- **Medicine A**
- **Medicine B**

Discrete-Choice Experiments

- Patients are asked to make choices between different hypothetical profiles

Discrete-Choice Experiments

- Patients are asked to make choices between different hypothetical profiles
- Each profile is defined by attributes

<table>
<thead>
<tr>
<th>Medicine Features</th>
<th>Medicine A</th>
<th>Medicine B</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pain while moving around one hour after taking the medicine</td>
<td>![Image]</td>
<td>![Image]</td>
</tr>
<tr>
<td>Pain while sitting, lying down, or sleeping one hour after taking the medicine</td>
<td>![Image]</td>
<td>![Image]</td>
</tr>
<tr>
<td>Stiffness one hour after taking the medicine</td>
<td>![Image]</td>
<td>![Image]</td>
</tr>
<tr>
<td>Difficulty doing your daily activities one hour after taking the medicine</td>
<td>![Image]</td>
<td>![Image]</td>
</tr>
<tr>
<td>Chance of a bleeding ulcer requiring an operation within the next year because of the medicine</td>
<td>![Image]</td>
<td>![Image]</td>
</tr>
<tr>
<td>Additional chance of a stroke within the next 5 years because of the medicine</td>
<td>![Image]</td>
<td>![Image]</td>
</tr>
</tbody>
</table>

Which medicine would you choose if these were the only medicines available? Medicine A Medicine B

Discrete-Choice Experiments

- Patients are asked to make choices between different hypothetical profiles.
- Each profile is defined by attributes that can take on different levels.

Discrete-Choice Experiments

Patients are asked to make choices between different hypothetical profiles

Each profile is defined by attributes that can take on different levels

Experimental design determines the profiles

Discrete-Choice Experiments

- Patients are asked to make choices between different hypothetical profiles.
- Each profile is defined by attributes that can take on different levels.
- Experimental design determines the profiles and profile pairs.

Discrete-Choice Experiments

- Patients are asked to make choices between different hypothetical profiles.
- Each profile is defined by attributes that can take on different levels.
- Experimental design determines the profiles, profile pairs, and series of choice questions.
Discrete-Choice Experiments

- Pattern of choices to the series of choice questions allows us to estimate a relative preference weight for each attribute level.
- Differences in preference weights reveal the impact of a change in levels on utility.

Discrete-Choice Experiments

- Pattern of choices to the series of choice questions allows us to estimate a relative preference weight for each attribute level
- Differences in preference weights reveal the impact of a change in levels on utility
- The effect on utility of a change in one attribute can be compared to the change in utility in another attribute

Discrete-Choice Experiments

- Pattern of choices to the series of choice questions allows us to estimate a relative preference weight for each attribute level.
- Differences in preference weights reveal the impact of a change in levels on utility.
- The effect on utility of a change in one attribute can be compared to the change in utility in another attribute.
- This comparison allows us to estimate things like overall attribute importance.

### Discrete-Choice Experiments

<table>
<thead>
<tr>
<th>Improvement in Pain</th>
<th>Maximum Acceptable Bleeding-Ulcer Risk</th>
<th>Maximum Acceptable Heart-Attack Risk</th>
<th>Maximum Acceptable Stroke Risk</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>75 mm to 50 mm</td>
<td>50 mm to 25 mm</td>
<td>25 mm to 0 mm</td>
</tr>
<tr>
<td>Pain while moving around 1 hour after taking the medicine (ambulatory pain)</td>
<td>0.74% (0.32%, 2.61%)</td>
<td>0.43% (0.18%, 0.91%)</td>
<td>0.73% (0.29%, 1.19%)</td>
</tr>
<tr>
<td>Pain while sitting, lying down, or sleeping 1 hour after taking the medicine (resting pain)</td>
<td>0.00% (N/A)</td>
<td>0.00% (N/A)</td>
<td>0.00% (N/A)</td>
</tr>
</tbody>
</table>

- Pattern of choices to the series of choice questions allows us to estimate a relative preference weight for each attribute level.
- Differences in preference weights reveal the impact of a change in levels on utility.
- The effect on utility of a change in one attribute can be compared to the change in utility in another attribute.
- This comparison allows us to estimate things like overall attribute importance and risk tolerance.

Threshold Technique

• Compares a reference option (no treatment or standard of care)

Threshold Technique

- Compares a reference option (no treatment or standard of care) to an alternative (target option) offering additional benefit and additional risk

Threshold Technique

- Compares a reference option (no treatment or standard of care) to an alternative (target option) offering additional benefit and additional risk.
- Risk in the target option is varied until the patient is indifferent between the two options (MAR).

Threshold Technique

- Compares a reference option (no treatment or standard of care) to an alternative (target option) offering additional benefit and additional risk
- Risk in the target option is varied until the patient is indifferent between the two options (MAR)
- Or benefit in the target option is varied until the patient is indifferent between the two options (MAB)

Threshold Technique

- The item for which the threshold will be estimated must have numeric values.
- Values can be adjusted until the specific threshold is identified for each patient until an interval of threshold values if identified for each patient.

Threshold Technique

- Results can be presented as histograms (as above) or as mean values.
- Thresholds can be regressed in respondent characteristics to identify factors associated with higher or lower risk tolerance.

Modified Swing Weighting

- A swing is the range of levels of an attribute (change in level from the highest to the lowest)

<table>
<thead>
<tr>
<th>Swings</th>
</tr>
</thead>
<tbody>
<tr>
<td>Increasing the probability of surviving 12 months from 45% to 65%</td>
</tr>
<tr>
<td>Decreasing probability of experiencing long-lasting symptoms of moderate severity from 20% to 5%</td>
</tr>
<tr>
<td>Decreasing probability of experiencing severe symptoms or events requiring medical intervention from 35% to 15%</td>
</tr>
</tbody>
</table>

Modified Swing Weighting

• Ranking swings

Part 1, Question 1

Imagine that you are currently on a treatment that has all of the following effects:
• Probability of surviving at 12 months = 45%
• Probability of experiencing long-lasting symptoms of moderate severity = 20%
• Probability of experiencing severe symptoms or events requiring medical intervention = 35%

You are given the opportunity to upgrade the performance of this treatment on one of these outcomes. Which of the following options would you prefer:

- Increasing the probability of surviving 12 months from 45% to 65%
- Decreasing probability of experiencing long-lasting symptoms of moderate severity from 20% to 5%
- Decreasing probability of experiencing severe symptoms or events requiring medical intervention from 35% to 15%

Modified Swing Weighting

• Swing in one attribute is adjusted until the patient is indifferent between the swing

Part 2, Question 1

Consider the following two options:

Treatment A
• Probability of surviving 12 months = 45%
• Probability of experiencing severe symptoms or events requiring medical intervention = 15%

Treatment B
• Probability of 12 months = 55%
• Probability of experiencing severe symptoms or events requiring medical intervention = 35%

Which of these options would you prefer:
○ Treatment A
○ Treatment B

Part 2, Question 2

Consider the following two options:

Treatment A
• Probability of surviving 12 months = 45%
• Probability of experiencing severe symptoms or events requiring medical intervention = 15%

Treatment B
• Probability of 12 months = 65%
• Probability of experiencing severe symptoms or events requiring medical intervention = 35%

Which of these options would you prefer:
○ Treatment A
○ Treatment B

Modified Swing Weighting

- Swing in one attribute is adjusted until the patient is indifferent between the swing.

**Part 2, Question 1**

Consider the following two options:

**Treatment A**
- Probability of surviving 12 months = 45%
- Probability of experiencing severe symptoms or events requiring medical intervention = 15%

**Treatment B**
- Probability of 12 months = 55%
- Probability of experiencing severe symptoms or events requiring medical intervention = 35%

Which of these options would you prefer:
- Treatment A
- Treatment B

**Part 2, Question 2**

Consider the following two options:

**Treatment A**
- Probability of surviving 12 months = 45%
- Probability of experiencing severe symptoms or events requiring medical intervention = 15%

**Treatment B**
- Probability of 12 months = 65%
- Probability of experiencing severe symptoms or events requiring medical intervention = 35%

Which of these options would you prefer:
- Treatment A
- Treatment B

Modified Swing Weighting


Best-Worst Scaling (object case)

- Items define outcomes or attributes of treatment
- Unlike DCE, Threshold Technique, and Modified Swing Weighting, items do not have levels that vary

Signs and Symptoms in Psoriasis and Psoriatic Arthritis

<table>
<thead>
<tr>
<th>Skin symptoms</th>
<th>Joint symptoms</th>
<th>Impacts of daily activities</th>
</tr>
</thead>
<tbody>
<tr>
<td>Itching skin</td>
<td>Joint pain, soreness, or tenderness</td>
<td>Difficulty with work or school activities</td>
</tr>
<tr>
<td>Redness of skin</td>
<td>Swelling of fingers or toes</td>
<td>Difficulty with social or leisure activities</td>
</tr>
<tr>
<td>Flaking skin</td>
<td>Fatigue</td>
<td>Difficulty going shopping or doing housework or yard work</td>
</tr>
<tr>
<td>Nail Problems</td>
<td>Morning stiffness</td>
<td>Difficulty sleeping</td>
</tr>
<tr>
<td>Difficulty choosing clothing</td>
<td>Eye problems</td>
<td>Discomfort while doing everyday tasks</td>
</tr>
<tr>
<td>Embarrassment</td>
<td>Difficulty dressing</td>
<td>Problems with relations</td>
</tr>
<tr>
<td></td>
<td>Difficulty walking</td>
<td></td>
</tr>
</tbody>
</table>

Best-Worst Scaling (object case)

- Items are divided into sets according to an experimental design
- Respondents indicate which item is “best” and which” is “worst” within each set in a series of questions

Best-Worst Scaling (object case)

- Pattern of choices provides data to estimate relative importance weights for all items

Summary

• Patient preference information (PPI) is different than other measures of patient input:
  – Patient reported outcomes (PRO)

• Patient preference information (PPI) can be an input into other processes or measures
  – Multi-criteria decision analysis (MCDA)
  – Quality-adjusted life years (QALY)

• There are many methods for eliciting and quantifying patient preferences
  – Four common methods are, discrete-choice experiments (DCE), threshold technique, modified swing weighting, and best-worst scaling (BWS)
Developing a Patient Preference Study

How do we get there?
Core components in designing a stated-preference study

Core components in designing a stated-preference study

- Objective
- Relevant
- Scientifically valid
- Feasible

1. Research question
2. Attributes and levels
3. Construction of tasks
4. Experimental design
5. Preference elicitation
6. Instrument design
7. Data collection
8. Statistical analyses
9. Results and conclusions
10. Study presentation

Define Objective
Attributes and levels
Design experiment
Design and implement survey instrument
Analyze data
Report Results

Some of these categories require multiple steps
Well-defined research question for which a stated preferences study is an appropriate method to answer it

- What aspects of health outcomes or attributes of health interventions are most important to patients?
- Assessing a new device or drug – what are the benefit-risk trade-offs?
- What is an acceptable benefit-risk threshold?
- What is the value of non-health outcomes (e.g. mode of administration)?
- Do preferences differ amongst subgroups of patients?
• Were all the important and relevant attributes and attribute levels included and supported by evidence?

Using Qualitative Methods for Attribute Identification

- What attributes are important to people
- Number of attributes relevant to research question
  - Omitted attributes adversely affect study quality
- Understand how people discuss attributes
  - What words or phrases do they use?
- Understand relationships between the attributes (e.g. route of drug administration and drug regimen; pain and function)

Task #2 Attributes and Levels

- Were all the important and relevant attributes and attribute levels included and supported by evidence?

**Selection of Attributes and Attribute Levels**

- **Attributes** - Consider all potential attributes, but balance:
  - Relevant to research question
  - What is important to respondents
  - What is important in the decision making context
  - Plausibility and feasibility

- **Levels** - Encompass salient range of values
Task #4 Experimental Design

Was the choice of experimental design justified and evaluated?

Principle of experiment: Systematically vary attributes and levels to investigate the determinants of choice behaviour

Example: 2 attributes each with 2 levels

Effectiveness (low vs high) and Side Effects (mild vs severe) for treatment of rheumatoid arthritis

<table>
<thead>
<tr>
<th>Treatment</th>
<th>Effectiveness</th>
<th>Side Effects</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Low</td>
<td>Mild</td>
</tr>
<tr>
<td>2</td>
<td>Low</td>
<td>Severe</td>
</tr>
</tbody>
</table>

Task #4 Experimental Design

Was the choice of experimental design justified and evaluated?

**Principle of experiment:** Systematically vary attributes and levels to investigate the determinants of choice behaviour

**Identification**

Unbiased parameter estimates for all model parameters

Must include sufficient numbers of attribute-level combinations to determine independent effects

Example: 2 attributes each with 2 levels

Effectiveness (low vs high) and Side Effects (mild vs severe) for treatment of rheumatoid arthritis

<table>
<thead>
<tr>
<th>Treatment</th>
<th>Effectiveness</th>
<th>Side Effects</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Low</td>
<td>Mild</td>
</tr>
<tr>
<td>2</td>
<td>Low</td>
<td>Severe</td>
</tr>
<tr>
<td>3</td>
<td>High</td>
<td>Mild</td>
</tr>
<tr>
<td>4</td>
<td>High</td>
<td>Severe</td>
</tr>
</tbody>
</table>
Task #4 Experimental Design

- Was the choice of experimental design justified and evaluated?

**Principle of experiment:** Systematically vary attributes and levels to investigate the determinants of choice behaviour

- **Identification**
  - Unbiased parameter estimates for all model parameters

- **Efficiency**
  - Maximise the precision of parameter estimates

- **Statistical Efficiency**
  - Minimise confidence intervals around parameter estimates

- **Response Efficiency**
  - Minimise measurement error
Steps in Survey Development

- Objective
- Relevant
- Scientifically valid
- Feasible

Patient and clinician involvement in developing survey

- Literature search & focused review
- Qualitative research e.g. focus groups
- Research team input
- Survey pre-test
- Survey pilot test
- Main survey to Field

- Clinician involvement with recruitment
- Patient involvement completing pre-tests

Testing survey format and method of administration (e.g. online tests if doing survey online)

Evidence + Engagement to develop attributes and levels

Cognitive feasibility

Administrative feasibility

Administering survey
### Components of a Preferences Survey

<table>
<thead>
<tr>
<th>Component</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Screening</strong></td>
<td>• Confirming inclusion and exclusion criteria</td>
</tr>
<tr>
<td><strong>Informed consent</strong></td>
<td>• Informed consent</td>
</tr>
<tr>
<td><strong>Background questions</strong></td>
<td>• Experience with disease, treatment and management</td>
</tr>
<tr>
<td><strong>Information treatment</strong></td>
<td>• Descriptions of each attribute and level in the choice tasks</td>
</tr>
<tr>
<td></td>
<td>• Warm-up questions</td>
</tr>
<tr>
<td><strong>Choice Task questions</strong></td>
<td>• 8-16 choice Task questions depending on complexity determined by experimental design</td>
</tr>
<tr>
<td><strong>Validity and Reliability</strong></td>
<td>• Validity and Reliability of measurement and choice</td>
</tr>
<tr>
<td><strong>Demographic questions</strong></td>
<td>• Age, gender, martial status, education, etc.</td>
</tr>
<tr>
<td><strong>Attention questions</strong></td>
<td>• Easy to understand, easy to answer, answered consistent with preferences</td>
</tr>
</tbody>
</table>
Steps in Survey Development

- Objective
- Relevant
- Scientifically valid
- Feasible

Literature search & focused review

Qualitative Research e.g. focus groups

Research team input

Survey pre-test

Survey pilot test

Main survey to Field

Patient and clinician involvement in developing survey

Clinician involvement with recruitment

Patient involvement completing pre-tests

Testing survey format and method of administration (e.g., online tests if doing survey online)

Evidence + Engagement to develop attributes and levels

Cognitive feasibility

Administrative feasibility

Administering survey
## Validity and Reliability

<table>
<thead>
<tr>
<th>Measurement</th>
<th>Validity</th>
<th>Reliability</th>
</tr>
</thead>
<tbody>
<tr>
<td>Measurement</td>
<td>How accurately an instrument measures outcome of interest</td>
<td>How consistently an instrument measures the outcome</td>
</tr>
<tr>
<td>Choice</td>
<td>Do measures of relative importance or tradeoffs reflect true preferences?</td>
<td>Do measures of relative importance or tradeoffs measure preferences consistently?</td>
</tr>
</tbody>
</table>

Indirect utility (value) function: \( V = \alpha + \beta_1 X_1 + \beta_2 X_2 + \varepsilon \ldots \)

<table>
<thead>
<tr>
<th>Treatment</th>
<th>Effectiveness</th>
<th>Side Effects</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Lowest</td>
<td>Mild</td>
</tr>
<tr>
<td>2</td>
<td>Lowest</td>
<td>Severe</td>
</tr>
<tr>
<td>3</td>
<td>Highest</td>
<td>Mild</td>
</tr>
<tr>
<td>4</td>
<td>Highest</td>
<td>Severe</td>
</tr>
</tbody>
</table>

- Ordering and magnitude of attribute parameters
- Relative importance of attributes
- Value of changes in attribute levels
- Compare change in utility in one attribute to the change in utility in another attribute (marginal rates of substitution)
### Interpretation of Preferences Results

<table>
<thead>
<tr>
<th>Attribute</th>
<th>Level</th>
<th>Coefficient</th>
<th>P value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Efficacy</td>
<td>10 (highest)</td>
<td>0.55</td>
<td>&lt;0.01</td>
</tr>
<tr>
<td></td>
<td>5 (medium)</td>
<td>0.30</td>
<td>&lt;0.01</td>
</tr>
<tr>
<td></td>
<td>3 (lowest)</td>
<td>-</td>
<td>-</td>
</tr>
<tr>
<td>Side Effect</td>
<td>Mild</td>
<td>0.66</td>
<td>&lt;0.01</td>
</tr>
<tr>
<td></td>
<td>Moderate</td>
<td>0.36</td>
<td>&lt;0.01</td>
</tr>
<tr>
<td></td>
<td>Severe</td>
<td>-</td>
<td>-</td>
</tr>
</tbody>
</table>

- **Ordering:** Higher efficacy (0.55) is preferred to lower efficacy (0.31); Mild side effects (0.66) are preferred to severe side effects (constrained to 0).

- **Relative importance:** Marginal utility of reducing side effects from moderate to mild = 0.66 – 0.36 = 0.30; Marginal utility of increasing efficacy from medium to high (5 points) = 0.55 – 0.30 = 0.25 = 0.05 per point.

- **Compare changes in Benefits (efficacy) and Risks (side effects):** Willing to give up 6 points of efficacy to reduce side effects from moderate to mild.
Recommended Qualities Patient Preference Studies

“For quantitative patient preference studies in particular, the Agency considers the study qualities outlined here, among other things, when deciding whether a given quantitative dataset of PPI constitutes valid scientific evidence.”

1. Patient Centeredness
2. Representativeness of sample and generalizability of results
3. Capturing Heterogeneity of Patients’ Preferences
4. Good Research Practices by Recognized Professional Organizations
5. Effective Communication of Benefit, Harm, Risk and Uncertainty
6. Minimal cognitive bias
7. Logical soundness
8. Relevance
9. Robustness of analysis of results
10. Study conduct
11. Comprehension by Study Participants

Recommended Qualities

- Patient Centeredness
- Relevance
- Good Research Practices

- Representativeness
- Logical Soundness
- Effective communication of benefit, risk
- Minimal cognitive bias

- Heterogeneity
- Robustness of analysis

- Study Conduct
- Comprehension by Participants

General Principals

Study Design

Technical

Practical
Summary

• Designing patient preference study is different than your everyday survey!
  – Objective, Relevant, Scientifically valid and Feasible

• Qualitative research is important to identify and select attributes and levels

• Experimental design to systematically vary attributes and levels to investigate the determinants of choice behaviour
  – Identification - unbiased parameter estimates for all model parameters
  – Efficiency - Maximise the precision of parameter estimates balancing statistical and response efficiency

• 11 qualities recommended for a scientifically valid patient preferences study that consider: general principals, study design, technical and practical aspects
Questions?
Additional Resources


Additional Resources