ELICITING PATIENT PREFERENCES: APPLYING DECISION THEORY TO HEALTH RESEARCH

Andrea Beyer, PhD
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Contents

• Why collect patient preferences?
• Decision Analysis
• Visualize Sub-study: eliciting patient preferences
• Study design
• Building Value Function
• Eliciting Weights
• Planned analysis
• Summary
Training Objectives

• To demonstrate the application of the MACBETH approach for eliciting preferences
  – To show how benefits and risks can be decomposed
  – To show how qualitative data can be converted to quantitative data

• To highlight the steps needed to collect preference data using this approach
Importance of Patients’ Perception for Treatment Decisions

Regulators’ view:
An increased cure rate in cancer, a potentially life-saving treatment will always outweigh a grade 1 or 2 AE (e.g. (permanent hair loss) - positive regulatory decision

Some patients’ view:
This permanent hair loss is important, severe enough for me to decline the potentially curative and life-saving adjuvant therapy – negative treatment decision

“The mastectomy and loss of breast are NOTHING compared to the loss of my hair.”

“Not a day goes by that I don’t regret doing the NN (therapy). Oh, if we could only turn back the hands of time!”

“I never, never, never would have agreed to take NN if I was informed of this 6.3% risk; even a 3% risk...or any risk...”
How to bring patient preferences/values into BR decisions?

- Patients with the specific disease condition know which outcomes and symptoms matter most to them.
- Patients enrolled in regulatory drug trial are (ideally) the target group for treatment once a drug is licensed, yet we do not explore their values and preferences in a systematic way.
- In terms of listening to the patients’ voice, trial patients are an underutilized source.

G. Rasi, AIFA, 2013
Can Decision Analysis Help?

“The spirit of decision analysis is divide and conquer:

decompose a complex problem into simpler problems, get one’s thinking straight on these simpler problems, paste these analyses together with logical glue, and come out with a program of action for the complex problem”

(Howard Raiffa 1968, p. 271)
Preference Elicitation Sub-study

Evaluate the use of the MACBETH approach for eliciting patient preferences using a simple pair-wise comparison between treatment outcomes

− determine value functions for treatment outcomes
− assess weights between treatment outcomes (trade-offs)
Applying the MACBETH approach to preference elicitation*

• Determine the treatment outcomes of interest
• Determine the levels for each outcome, ranging from best case to worst case
• Create the value elicitation section of the questionnaire
• Create the weighting elicitation section
• Collect data from patients and convert the qualitative responses of patients to quantitative scores
• * Seek patient input/confirmation for steps 1-4
## Example for Atrial Fibrillation

<table>
<thead>
<tr>
<th>Treatment outcome</th>
<th>Levels</th>
</tr>
</thead>
<tbody>
<tr>
<td>Ischemic Stroke</td>
<td>No patients developing ischemic stroke</td>
</tr>
<tr>
<td></td>
<td>1% of patients developing ischemic stroke</td>
</tr>
<tr>
<td></td>
<td>2% of patients developing ischemic stroke</td>
</tr>
<tr>
<td></td>
<td>3% of patients developing ischemic stroke</td>
</tr>
<tr>
<td></td>
<td>4% of patients developing ischemic stroke</td>
</tr>
<tr>
<td>Myocardial Infarction</td>
<td>No patients developing myocardial infarction</td>
</tr>
<tr>
<td></td>
<td>1% of patients developing myocardial infarction</td>
</tr>
<tr>
<td></td>
<td>2% of patients developing myocardial infarction</td>
</tr>
<tr>
<td></td>
<td>3% of patients developing myocardial infarction</td>
</tr>
<tr>
<td></td>
<td>4% of patients developing myocardial infarction</td>
</tr>
<tr>
<td>Major bleeding</td>
<td>No patients developing a major bleed</td>
</tr>
<tr>
<td></td>
<td>2% of patients developing a major bleed</td>
</tr>
<tr>
<td></td>
<td>4% of patients developing a major bleed</td>
</tr>
<tr>
<td></td>
<td>6% of patients developing a major bleed</td>
</tr>
<tr>
<td></td>
<td>8% of patients developing a major bleed</td>
</tr>
<tr>
<td>Minor bleeding</td>
<td>15% of patients developing a minor bleed</td>
</tr>
<tr>
<td></td>
<td>20% of patients developing a minor bleed</td>
</tr>
<tr>
<td></td>
<td>25% of patients developing a minor bleed</td>
</tr>
<tr>
<td></td>
<td>30% of patients developing a minor bleed</td>
</tr>
<tr>
<td></td>
<td>35% of patients developing a minor bleed</td>
</tr>
</tbody>
</table>
Eliciting Values

Building a value scale for “Minor bleeding”

15 % of patients with minor bleeding
20 % of patients with minor bleeding
25 % of patients with minor bleeding
30 % of patients with minor bleeding
35 % of patients with minor bleeding

What is the difference in value between

15% of patients and 20% of patients
with a minor bleeding?

extreme
v. strong
strong
moderate
weak
very weak
no
Building a value scale for “Minor bleeding”

What is the difference in value between

15% of patients and 20% of patients
with a minor bleeding?
Building a value scale for “Minor bleeding”

Very Strong

Strong

Weak

Very Weak

15%

20%

25%

30%

35%
Number of patients with linear or non-linear curves

**Linear Value Function**

**Concave Value Function**

Local impacts and scores for both references and options on the selected criterion

Mathematical expression:

\[-33.33/1 \times + 100\]
\[-27.78/1 \times + 94.45/1\]
\[Y = \{-22.22/1 \times + 83.33/1\}
\[-16.67/1 \times + 66.68/1\]

Scores: 32, 30, 25, 6, 8
Qualitative responses will be converted to quantitative scores (0-100) then mapped to one of the 10 value function profiles below.

**Value Function Profiles**

1. $\text{Jud}_1 = \text{Jud}_2 = \text{Jud}_3$
2. $\text{Jud}_1 < \text{Jud}_2 < \text{Jud}_3$
3. $\text{Jud}_1 > \text{Jud}_2 > \text{Jud}_3$
4. $\text{Jud}_1 > \text{Jud}_2 < \text{Jud}_3$
5. $\text{Jud}_1 < \text{Jud}_2 > \text{Jud}_3$
6. $\text{Jud}_1 = \text{Jud}_2 = \text{Jud}_3$
7. $\text{Jud}_1 < \text{Jud}_2 < \text{Jud}_3$
8. $\text{Jud}_1 > \text{Jud}_2 > \text{Jud}_3$
9. $\text{Jud}_1 > \text{Jud}_2 < \text{Jud}_3$
10. $\text{Jud}_1 < \text{Jud}_2 > \text{Jud}_3$
If you could increase one treatment effect from its worst value (on the bottom) to its best value (on the top), which one would you increase?

<table>
<thead>
<tr>
<th>Treatment</th>
<th>Worst Value</th>
<th>Best Value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Ischemic stroke</td>
<td>0%</td>
<td>4%</td>
</tr>
<tr>
<td>Myocardial infarction</td>
<td>0%</td>
<td>4%</td>
</tr>
<tr>
<td>Pulmonary embolism</td>
<td>0%</td>
<td>4%</td>
</tr>
<tr>
<td>Fatal bleeding</td>
<td>0%</td>
<td>4%</td>
</tr>
<tr>
<td>Major bleeding</td>
<td>0%</td>
<td>8%</td>
</tr>
<tr>
<td>Minor bleeding</td>
<td>0%</td>
<td>35%</td>
</tr>
</tbody>
</table>

- Ischemic stroke: 0% of the patients getting an ischemic stroke vs. 4% of the patients getting an ischemic stroke
- Myocardial infarction: 0% of the patients getting a myocardial infarction vs. 4% of the patients getting a myocardial infarction
- Pulmonary embolism: 0% of the patients getting a pulmonary embolism vs. 4% of the patients getting a pulmonary embolism
- Fatal bleeding: 0% of the patients getting a fatal bleeding vs. 4% of the patients getting a fatal bleeding
- Major bleeding: 0% of the patients getting a major bleeding vs. 8% of the patients getting a major bleeding
- Minor bleeding: 0% of the patients getting a minor bleeding vs. 35% of the patients getting a minor bleeding
<table>
<thead>
<tr>
<th>Condition</th>
<th>0% of the patients getting</th>
<th>4% of the patients getting</th>
<th>8% of the patients getting</th>
<th>15% of the patients getting</th>
</tr>
</thead>
<tbody>
<tr>
<td>Ischemic stroke</td>
<td>an ischemic stroke</td>
<td>a myocardial infarction</td>
<td>a major bleeding</td>
<td>a minor bleeding</td>
</tr>
<tr>
<td>Myocardial infarction</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Pulmonary embolism</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Fatal bleeding</td>
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<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Major bleeding</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Minor bleeding</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
If you could increase one treatment effect from its worst value (on the bottom) to its best value (on the top), which one would you increase?
How desirable is this improvement?

0% of the patients getting an ischemic stroke

0% of the patients getting a myocardial infarction

0% of the patients getting a pulmonary embolism

0% of the patients getting a fatal bleeding

0% of the patients getting a major bleeding

15% of the patients getting a minor bleeding

Ischemic stroke

Myocardial infarction

Pulmonary embolism

Fatal bleeding

Major bleeding

Minor bleeding

How desirable is this improvement?

extreme

very strong

strong

moderate

weak

very weak
How much more desirable is the improvement on the right when compared to the one on the left?

- **Myocardial Infarction**: 4% of the patients getting a myocardial infarction
- **Pulmonary Embolism**: 0% of the patients getting a pulmonary embolism

**Comparison**:
- Strong: 4% of patients
- Very Strong: 0% of patients

**Legend**:
- Very weak
- Weak
- Moderate
- Strong
- Very strong
- Extreme
Assessing the weights

<table>
<thead>
<tr>
<th>Condition</th>
<th>Ischemic stroke</th>
<th>Myocardial Infarction</th>
<th>Major bleeding</th>
<th>Minor bleeding</th>
</tr>
</thead>
<tbody>
<tr>
<td>Percentage</td>
<td>30%</td>
<td>25%</td>
<td>40%</td>
<td>10%</td>
</tr>
</tbody>
</table>
Evaluation of clinical data using patient values
## Building a Decision Model

### Global Results

Table of global and partial scores for each option in each criteria

<table>
<thead>
<tr>
<th></th>
<th>Number of relapses</th>
<th>Time to disease prog</th>
<th>Disease progress</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>Good</td>
<td>100</td>
<td>100</td>
<td>100</td>
<td>100</td>
</tr>
<tr>
<td>Treat A</td>
<td>50</td>
<td>92</td>
<td>86</td>
<td>72</td>
</tr>
<tr>
<td>Treat B</td>
<td>6</td>
<td>89</td>
<td>100</td>
<td>52</td>
</tr>
<tr>
<td>Treat C</td>
<td>-6</td>
<td>11</td>
<td>29</td>
<td>6</td>
</tr>
<tr>
<td>Neutral</td>
<td>0</td>
<td>0</td>
<td>0</td>
<td>0</td>
</tr>
</tbody>
</table>

### Weights

- Good: 46%
- Treat A: 36%
- Treat B: 15%
Summary

- Method can be used to collect patient preferences in a remote setting
- Can be easily extended to patients within clinical trials (advanced PRO)
- Complies with decision theoretic principles
- Further research is needed to assess aggregation of the data