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ELICITING PATIENT PREFERENCES: APPLYING DECISION THEORY TO HEALTH RESEARCH

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Disclaimer

The views and opinions expressed in this presentation are those of the presenter, and should not be attributed to the FDA, EMA or any other regulatory body.

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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."

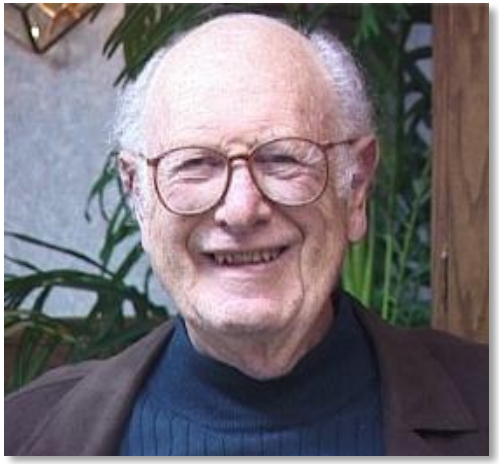
"Not a day goes by that I don't regret doing the NN (therapy). Oh, if we could only turn back the hands of time!"

"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

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"

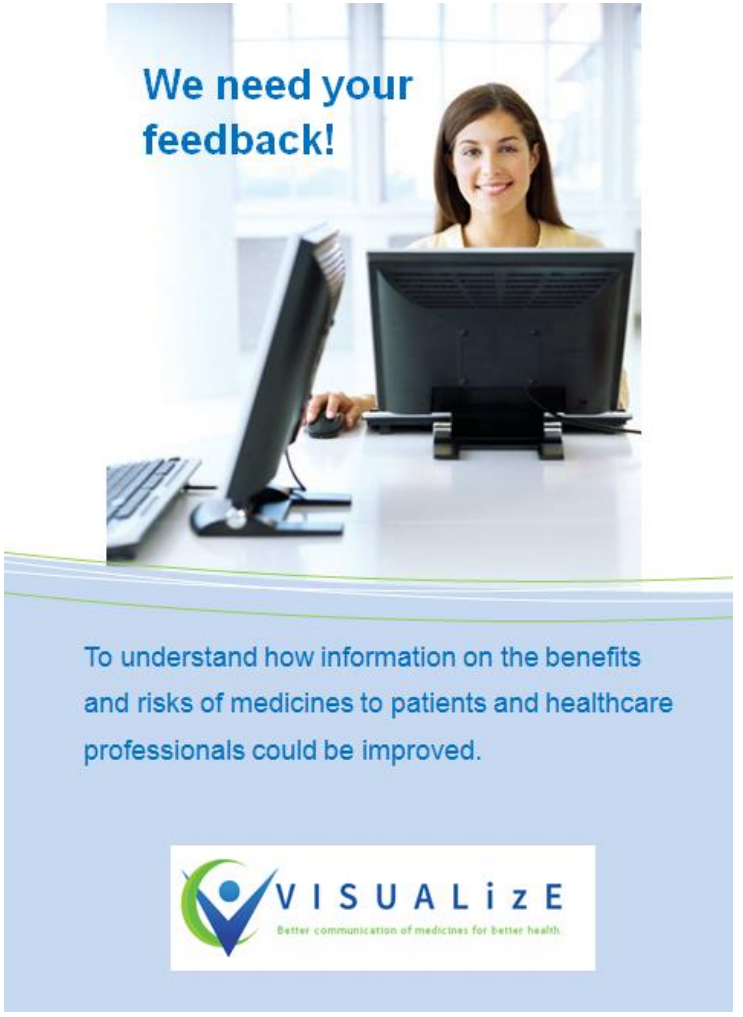
(Howard Raiffa 1968, p. 271)

Visualizing Uncertainty Among Laypersons and Experts

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)



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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

Treatment Outcomes and Levels

Example for Atrial Fibrillation

Treatment outcome	Levels
Ischemic Stroke	No patients developing ischemic stroke
	1% of patients developing ischemic stroke
	2% of patients developing ischemic stroke
	3% of patients developing ischemic stroke
	4% of patients developing ischemic stroke
Myocardial Infarction	No patients developing myocardial infarction
	1% of patients developing myocardial infarction
	2% of patients developing myocardial infarction
	3% of patients developing myocardial infarction
	4% of patients developing myocardial infarction
Major bleeding	No patients developing a major bleed
	2% of patients developing a major bleed
	4% of patients developing a major bleed
	6% of patients developing a major bleed
	8% of patients developing a major bleed
Minor bleeding	15% of patients developing a minor bleed
	20% of patients developing a minor bleed
	25% of patients developing a minor bleed
	30% of patients developing a minor bleed
	35% of patients developing a minor bleed

Eliciting Values

Building a value scale for "Minor bleeding"

15 % of patients with minor bleeding

20 % of patients with minor bleeding

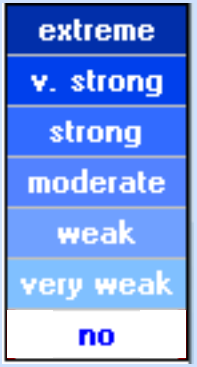
25 % of patients with minor bleeding

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?



Building a value scale for “Minor bleeding”

Very Strong

15%

20%

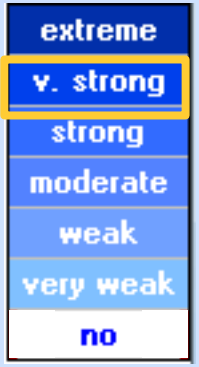
25%

30%

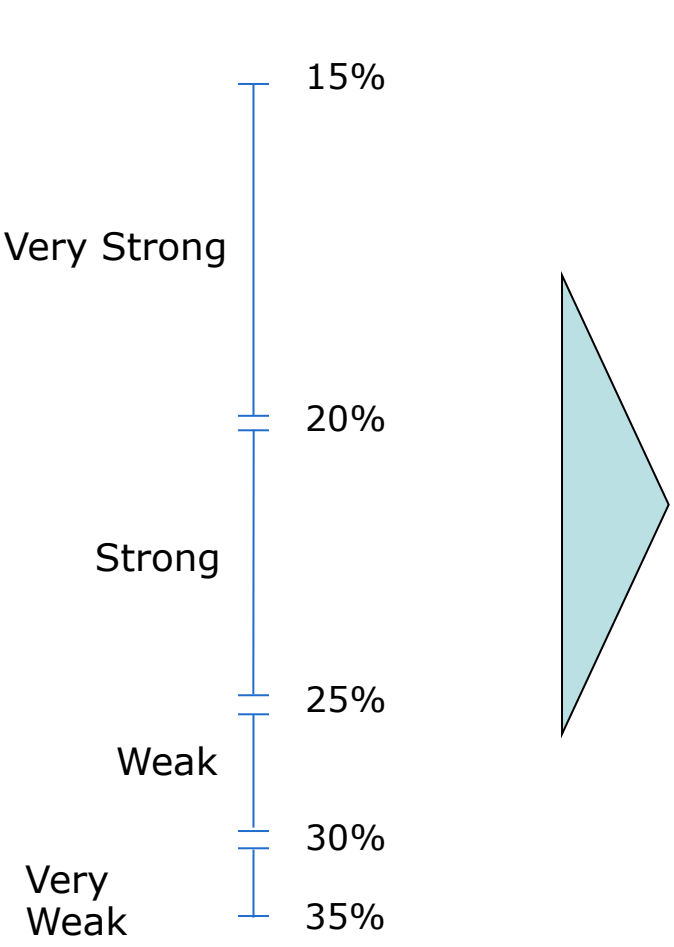
35%

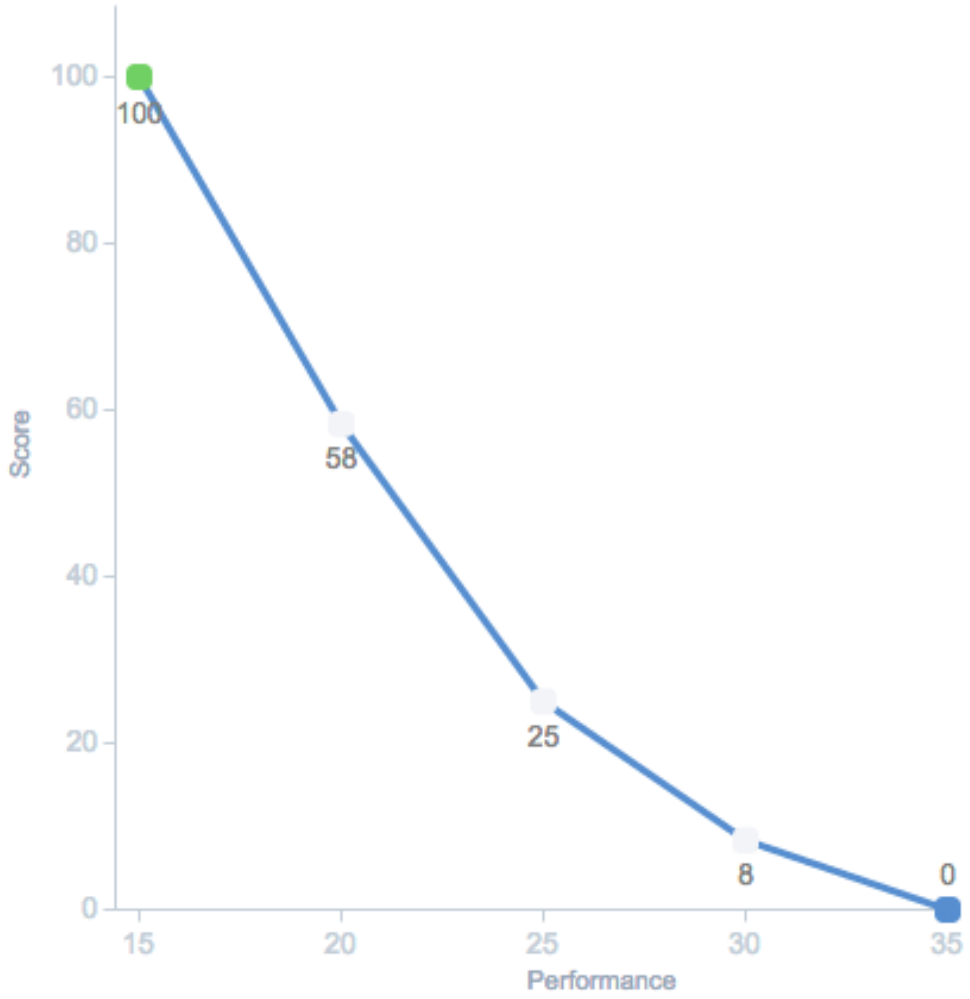
What is the difference in value between

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



Building a value scale for “Minor bleeding”





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wiseD

Define Criteria | Define Scales | Weight Criteria | Define Options | Evaluate Options | Analize Results

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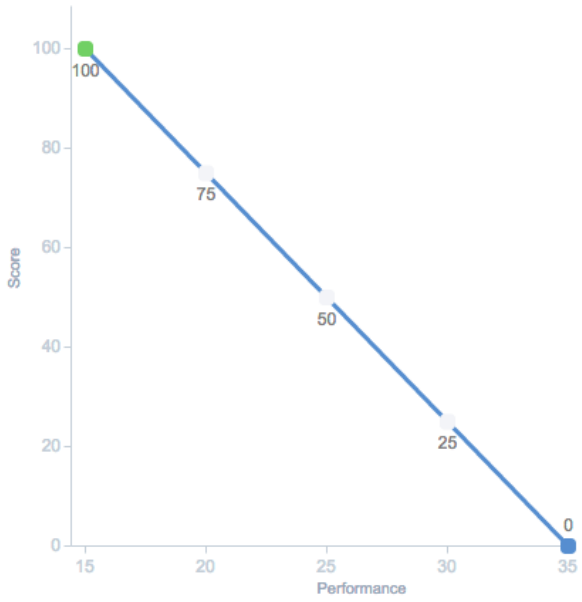
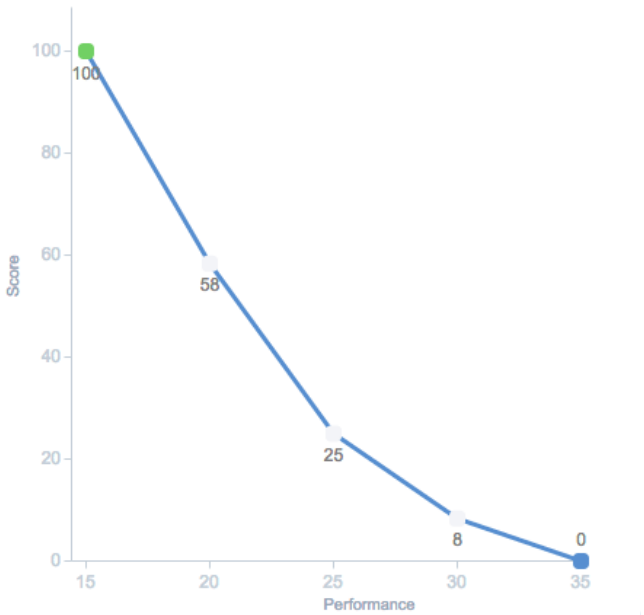
Add Reference | Edit Reference | Delete Reference | Set Good | Set Neutral | Get Scale | Edit Scale | Propose Scale

Linear Value Function



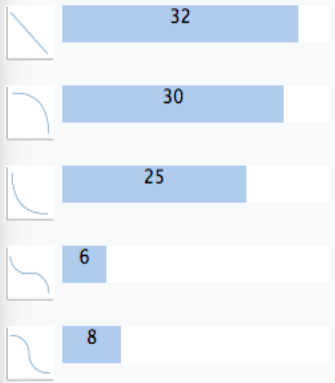
Concave Value Function

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



Mathematical expression

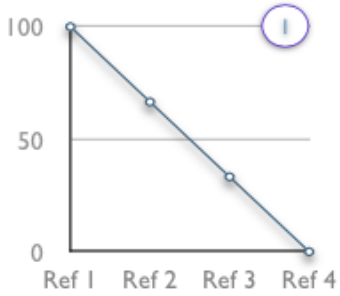
$$Y = \begin{cases} -33.33/1\,X + 100 & <= 0 < x < 1 \\ -27.78/1\,X + 94.45/1 & <= 1 < x < 2 \\ -22.22/1\,X + 83.33/1 & <= 2 < x < 3 \\ -16.67/1\,X + 66.68/1 & <= 3 < x < 4 \end{cases}$$



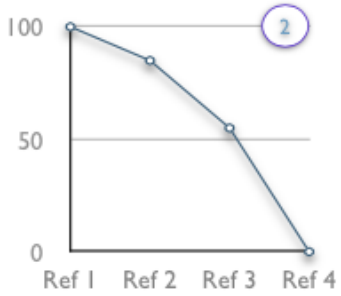
Number of patients with linear or non-linear curves

Value Function Profiles

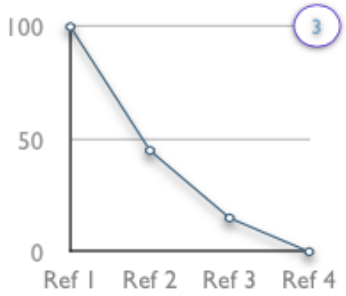
Qualitative responses will be converted to quantitative scores (0-100) then mapped to one of the 10 value function profiles below



Jud1 = Jud2 = Jud3



Jud1 < Jud2 < Jud3



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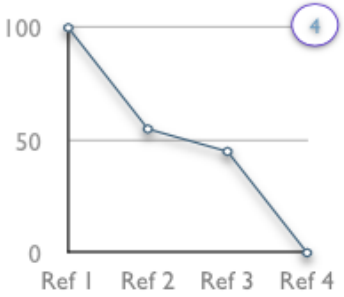

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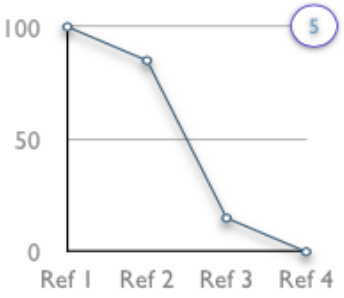

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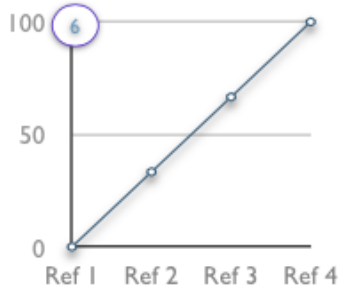

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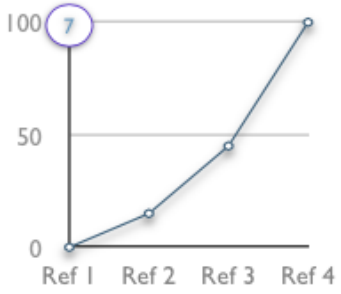

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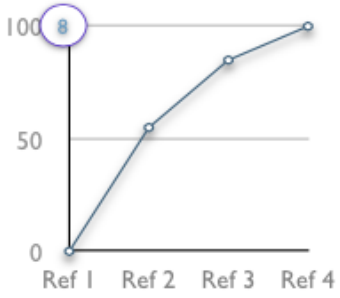

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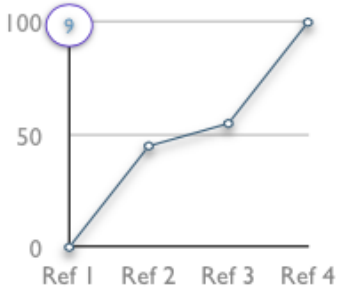

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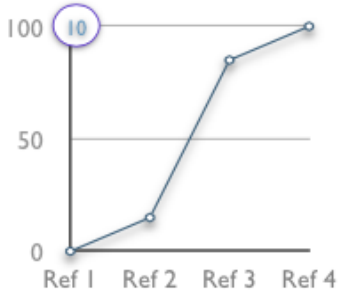


Jud1 < Jud2 > Jud3

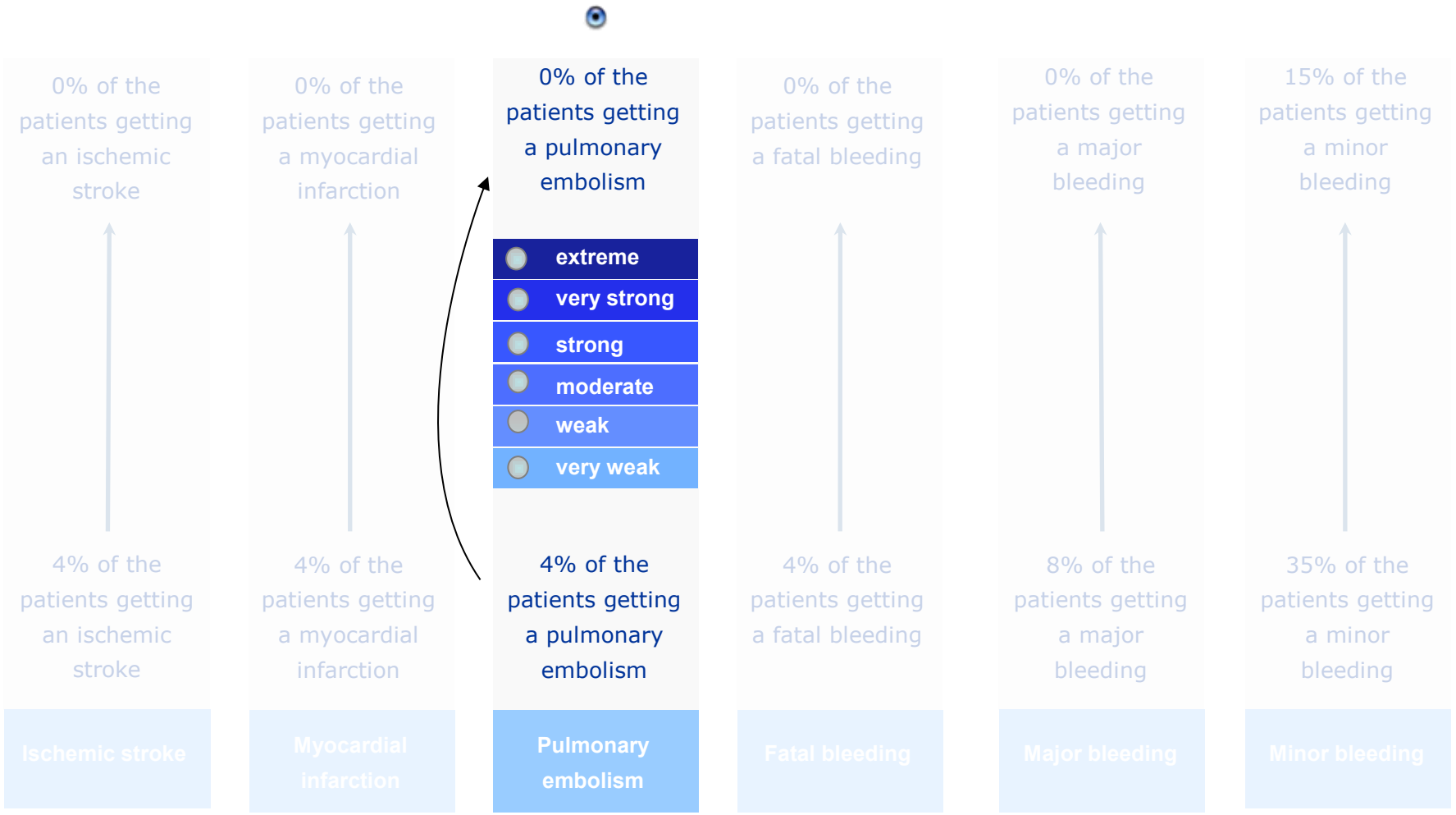
Eliciting Weights

If you could increase one treatment effect from it's worst value (on the bottom) to it's best value (on the top), which one would you increase?

Ischemic stroke	Myocardial infarction	Pulmonary embolism	Fatal bleeding	Major bleeding	Minor bleeding
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
4% of the patients getting an ischemic stroke	4% of the patients getting a myocardial infarction	4% of the patients getting a pulmonary embolism	4% of the patients getting a fatal bleeding	8% of the patients getting a major bleeding	35% of the patients getting a minor bleeding

Qualitative swing weighting

How desirable is this improvement?



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- extreme
- very strong
- strong
- moderate
- weak
- very weak

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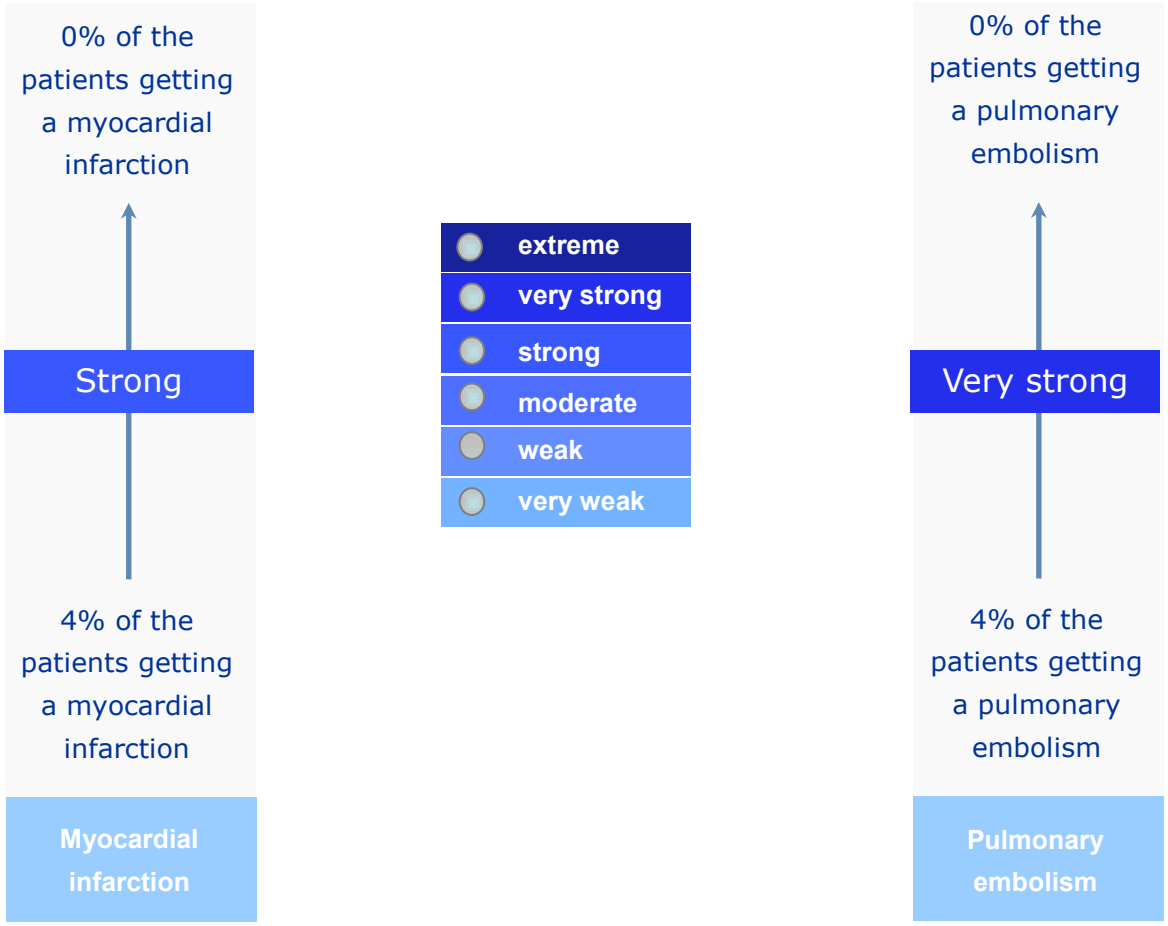
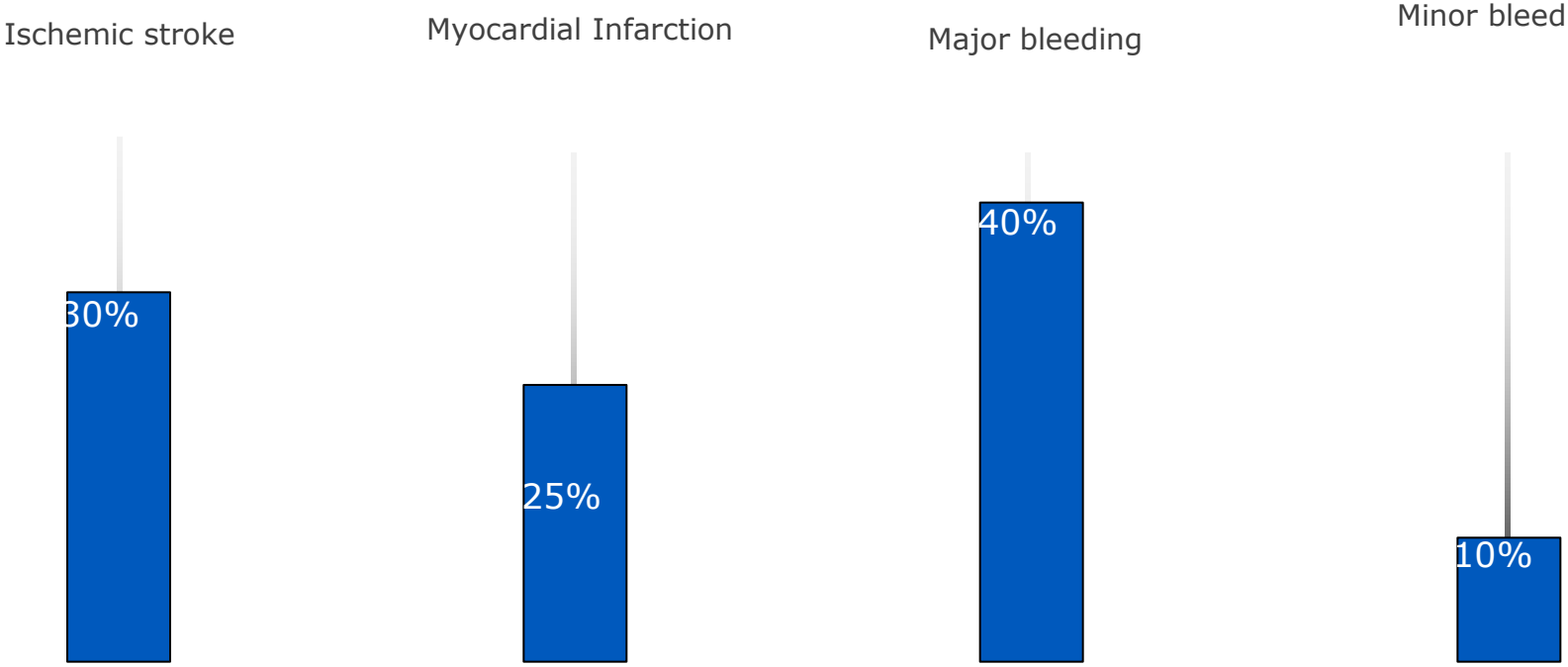
How much more desirable is the improvement on the right when compared to the one on the left?



Assessing the weights



Evaluation of clinical data using patient values

Building a Decision Model

Filter

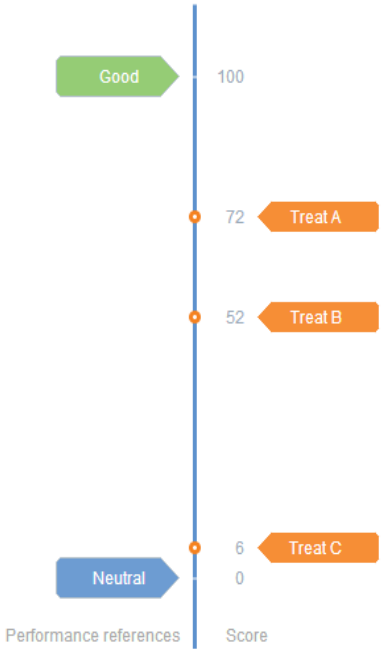
All Intersection

Tree

Global Results

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

	Number of relapses	Time to disease prog	Disease progr...	Total
Good	100	100	100	100
Treat A	50	92	86	72
Treat B	6	89	100	52
Treat C	-6	11	29	6
Neutral	0	0	0	0
Weigths	46%	38%	15%	



Results

Global results
Tabela de pontuações globais e parciais para cada opção em cada fator de avaliação

Analysis

Profile Analysis
Pontuações das opções em todos os fatores. Selecione a opção pretendida para ver o seu perfil. A seleção de duas opções permite ver a comparação entre as duas

Sensitivity Analysis
Análise da sensibilidade dos resultados a variações nos pesos dos fatores

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