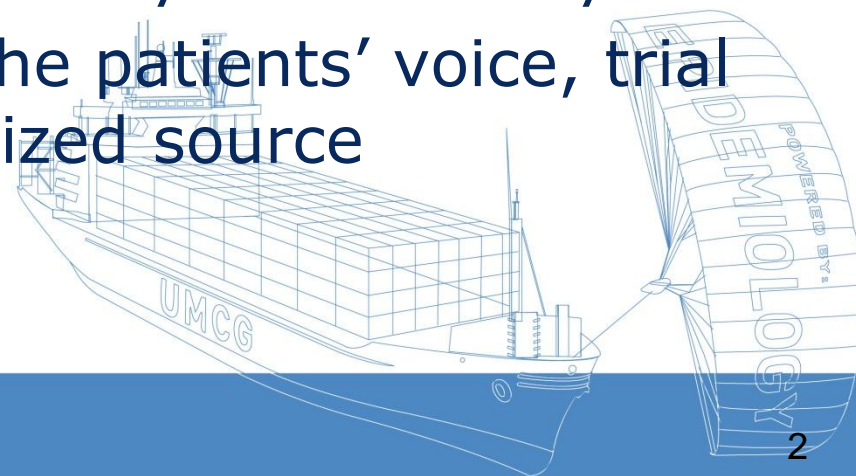


The Patient's Understanding of Benefit-Risk



European Medicines Agency: 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, EMA: AIFA Conference, February 2013



umcg

DEPARTMENT OF EPIDEMIOLOGY

The **VALUE** Study in Multiple Sclerosis

- Method allows for design of questionnaire using simple pair-wise comparisons written in plain language supported by the MACBETH software
- Data was easily collected via a web-based user interface and can be use to collect patient preferences in remote settings, e.g., multi-site clinical trials
- Qualitative data transformed to quantitative scores and can be used to build a treatment decision model
- Method complies with decision theoretic principles



Building a decision model

wisEd

Home Settings

Define Criteria Define Scales Weight Criteria Define Options Evaluate Options

Add Criterion Edit Criterion Delete Criterion Add Task Duplicate Criterion

MS Study

- Number of relapses
- Time to Disease Progression
- Disability due to Disease Progression
- Number of deaths by liver failure in 10 years
- Number of deaths or severe disability by PML
- Number of deaths by Leukemia

Collect responses to pair-wise comparisons of treatment levels for Number of Relapses

The VALUE Study - Value and Utilities in European Patients

EMA\UMCG Collaboration

Question number:

1 2 3 4 5 6 7 8 9 10

Previous

Next

Pause

Quit

What is the difference in attractiveness between:

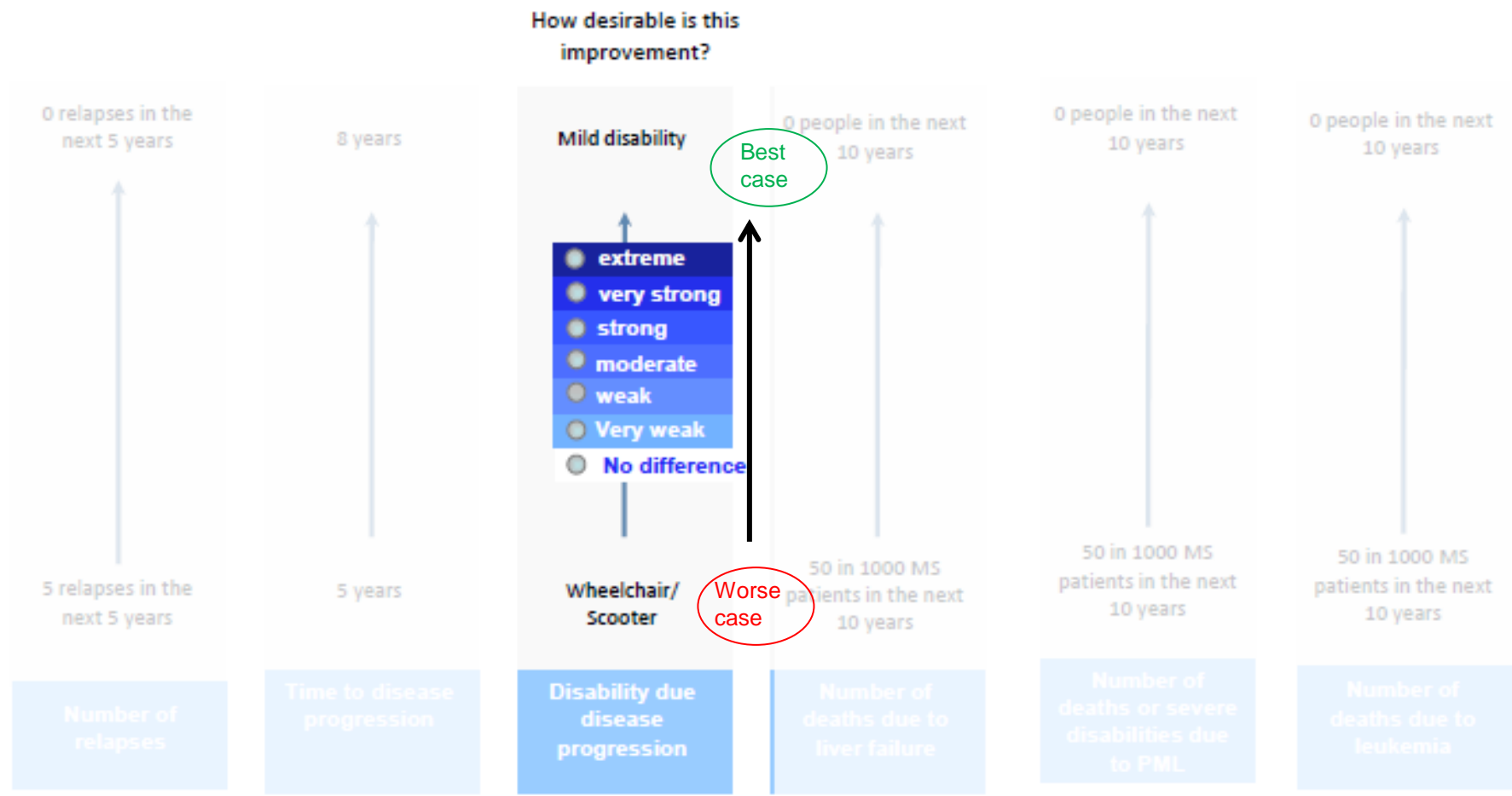
“having no relapses in the next 5 years compared to 1 relapse in the next 5 years?”

User interface of MACBETH collects qualitative data

- ☐ extreme
- ☐ very strong
- ☐ strong
- ☐ moderate
- ☐ weak
- ☐ Very weak
- ☐ no



Example of swing weighting questions posed to patients



Building a Treatment Decision Model

Global Results

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

	Number of relapses	Time to Disease Prog	Disability due to DI	Number of deaths by	Number of deaths or	Number of deaths by	Total
Good	100	100	100	100	100	100	100
Treat A	97	-20	85	97	99	100	68
Treat B	98	0	30	94	100	98	55
Neutral	0	0	0	0	0	0	0
Weights	18%	21%	32%	11%	14%	4%	



Based on hypothetical clinical trial data, one patient's values and judgments indicated that Treatment A outperforms Treatment B

Results

Global results

Table of local and global scores for each option in each criterion

Analysis

Profile Analysis

Options' scores on all criteria. Select an option to see it's profile. Selecting two options will also show their comparison

Sensitivity Analysis

Sensitivity analysis of the results to changes on the weights of the criteria

Main Study Results

- Qualitative pair-wise questions were easy for patients to understand/respond
- Value function curves indicate differing risk attitudes
- Majority of patients assigned the highest weight to treatment outcome reduction of 'disability due to disease progression'
- Ability to walk was a strong predictor for values (risk attitude) towards treatment side effects

