Helping Patients Understand Benefits and Risks of Medicines: How Decision Theory Can Help

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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.

European Medicines Agency

- Standing EMA Working party with patients and consumers
- Permanent patient representatives on some committees and Advisory groups, but not the CHMP
- Patients effectively excluded from key decisions on licensing
- Direct involvement of patients with the disease under discussion extremely rare

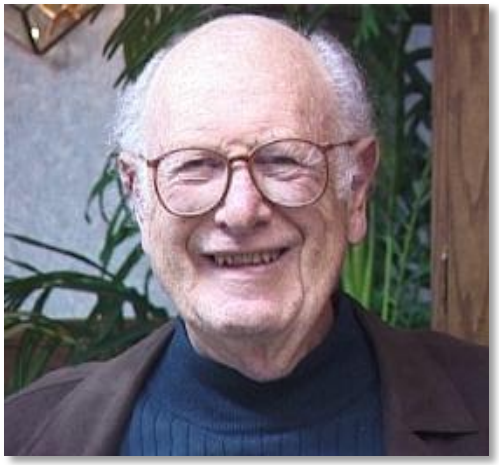
G. Rasi, EMA: AIFA Conference, February 2013

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

Decision Analysis – A New Pathway for Patient Voice?



"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)

VALue and Utilities among European Patients: The VALUE Study

- Objective:
  - to evaluate the use of the MACBETH (**M**easuring **A**ttractiveness through a **C**ategorical **B**ased **E**valuation) software for the elicitation of patient preferences using a simple pair-wise comparison between treatment outcomes
    - determine patients' value functions for MS treatment outcomes
    - assess weights patients assign to treatment outcomes
    - User acceptance of the questionnaire design and user interface
- Design
  - Web-based study among 62 Multiple Sclerosis (MS) patients evaluating several MS treatment outcomes
  - Supported by the EMA Patient and Healthcare Professionals and the UK MS Society whose members (patients) were invited to participate

Bana e Costa C, De Corte J-M, Vansnick JC. MACBETH. International Journal of Information Technology and Decision Making. 2012;11(2):359-87

Steps for eliciting preferences using MACBETH

- Identify the important treatment outcomes
- Determine the levels of within each outcome
- Elicit the preferences for the within outcome levels
- Use swing weighting method to collect weights

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The VALUE Study - Value and Utilities in European Patients

- Introduction ✓
- Disease History ✓
- MS Favorable Effects: ✓
- - Number of Relapses
- - Time to Disease Progression
- - Disease Progression
- MS Unfavorable Effects:
- - Number of deaths due to Liver Failure
- - Number of deaths due to PML
- - Number of deaths due to Leukemia
- Weighting
- B/R Tradeoff
- HRQoL

Question number 1 of 4
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Imagine there is a treatment where patients could experience one of the two outcomes below:

0 Relapses in the next 5 years	1 Relapse in the next 5 years

What is the difference in attractiveness between the two outcomes?

Extreme
Very Strong
Strong
Moderate
Weak
Very Weak
Indifferent

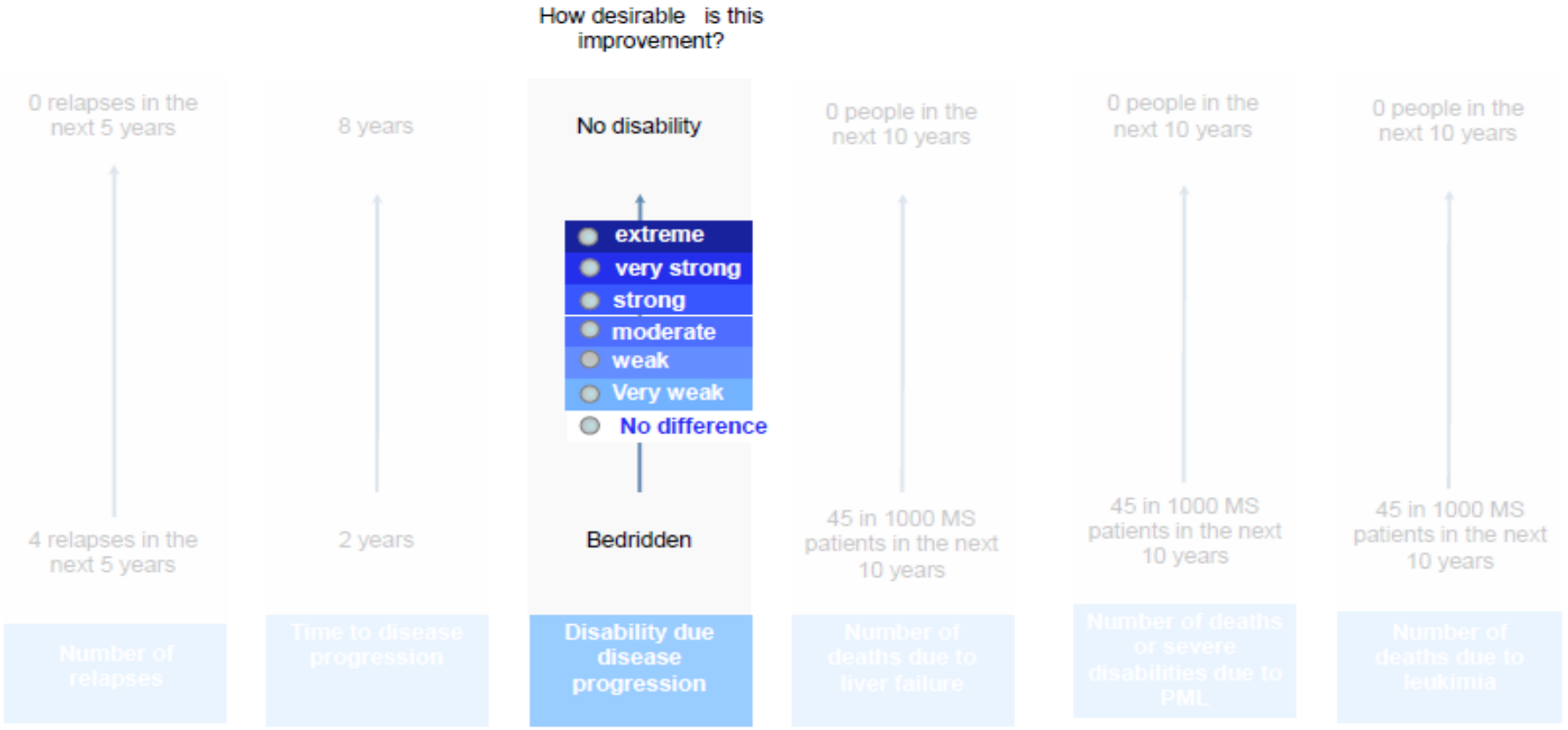
Question number: 1 2 3 4 5 6 7 8 9 10 11 12 13

Previous | Next | Pause | Quit

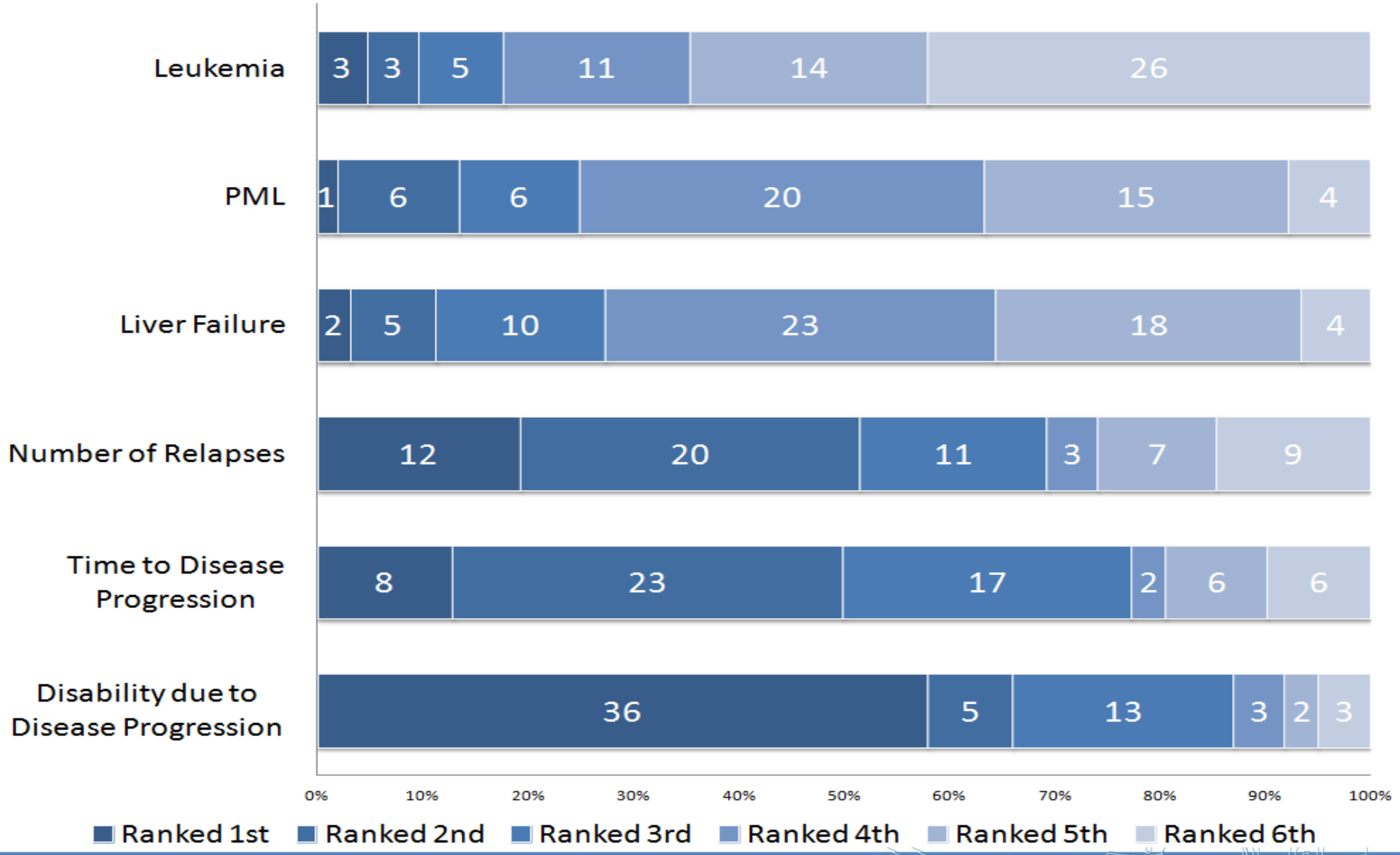
Which of the folowing improvements is the most important?

Number of relapses	Time to disease progression	Disability due to disease progression	Number of deaths due to liver failure	Number of deaths or severe disabilities due to PML	Number of deaths due to leukimia
0 relapses in the next 5 years	8 years	No disability	0 people in the next 10 years	0 people in the next 10 years	0 people in the next 10 years
4 relapses in the next 5 years	2 years	Bedridden	45 in 1000 MS patients in the next 10 years	45 in 1000 MS patients in the next 10 years	45 in 1000 MS patients in the next 10 years

The VALUE Study - Value and Utilities in European Patients



Distribution of Patients' Weights



Summary

- Method allows design of questionnaire using simple pair-wise comparisons written in plain language
- Qualitative data converted to quantitative scores and can used to build a treatment decision model
- Data was easily collected via a web-based user interface and can be use to collect patient preferences in a remote setting, e.g., clinical trial
- These data help regulators gain better understanding of patients values and can be used as inputs to the current regulatory decision making process