

EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

# Eliciting patient preferences about the benefits and risks of medicinal products

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20<sup>th</sup> Congress of the European Hematology Association  
Patient Advocacy Session 1, Saturday 13 June 2015

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# Benefit-Risk Assessment – Working Definition

The balance between benefits (and uncertainty) against risks (and uncertainty)

Benefits	“Good things”; clinical efficacy
Risks	“Bad things”; identified harms (toxicity) and potential harms
Uncertainty	<ul style="list-style-type: none"><li>• Measurable: Statistical uncertainty, measurement error,...</li><li>• Things we could know but don't, because, e.g., poor quality of the data, population not studied, ...</li></ul>



# How do regulators decide? By...

## Discussing



## Voting



No systematic elicitation of preferences



# EMA activities involving patients

## Involvement as experts

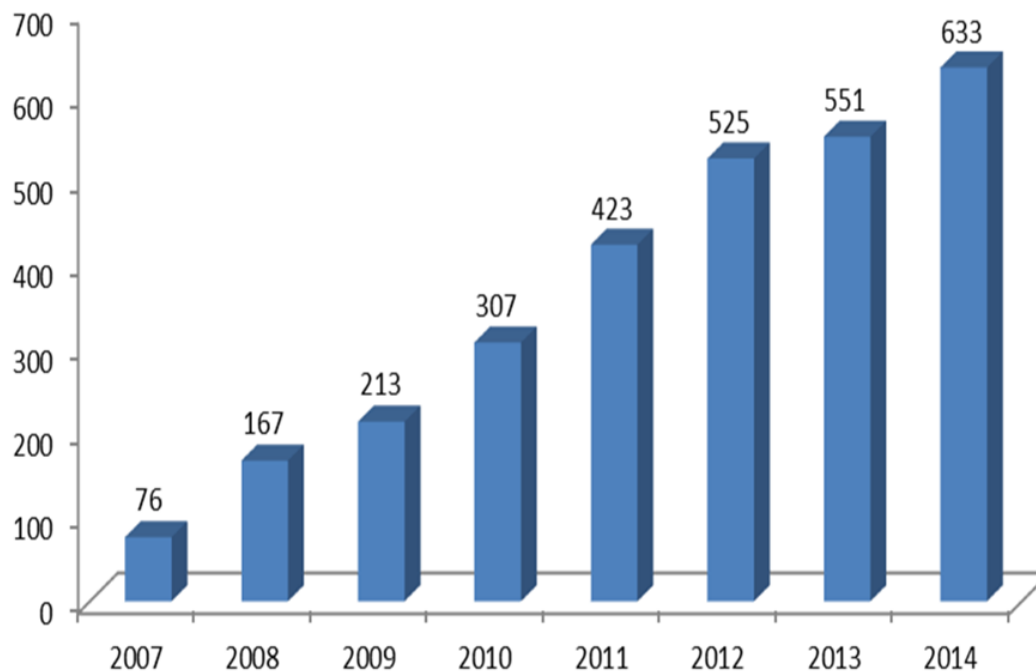
- Scientific Advisory Groups
- Scientific Advice Working Party

## Involvement in benefit-risk assessment (pilot):

- 1-2 experts input sought during CHMP meeting

## How representative?

Overall number of patient & consumer involvement in EMA activities 2007–2014





## More formal methods: feasibility study

- Multi-criteria decision analysis (MCDA) was used to elicit patient preferences for different outcomes in the treatment of an advanced cancer
- Individual preferences were collected through an online questionnaire
  - Melanoma patients & advocates (Melanoma Patient Network Europe)
  - Myeloma patients & advocates (Myeloma Patients Europe)
  - Regulators (colleagues @ EMA)
- Group preferences were obtained through a decision conference



# Attributes considered in the exercise

Criterion	Description	Worst	Best
Overall survival	Probability of surviving 12 months	45%	65%
Moderate symptoms	Probability of experiencing long-lasting symptoms (> 10 weeks) of moderate severity. Medical intervention is not immediately required, but the symptoms are likely to interfere somewhat with the patient's usual or daily activities	20%	5%
Severe symptoms	Probability of experiencing severe symptoms or events requiring medical intervention. The symptoms strongly interfere with the patient's usual or daily activities, but they are generally resolved within 3 weeks after onset. Hospitalization may be required	35%	15%



# Preference elicitation instrument – part 1

## Part 1: question 1/2

Imagine that you are currently on a treatment that has all of the following effects:

- Probability of surviving 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 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 %

Previous

Next





# Preference elicitation instrument – part 1

## Part 1: question 2/2

After your previous selection, the treatment now has the following effects:

- Probability of surviving 12 months = 65 %
- 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 yet another outcome.

Which of the following options would you prefer:

- 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 %



## Preference elicitation instrument – part 2

### Part 2: question 1/4

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 surviving 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

Previous

Next

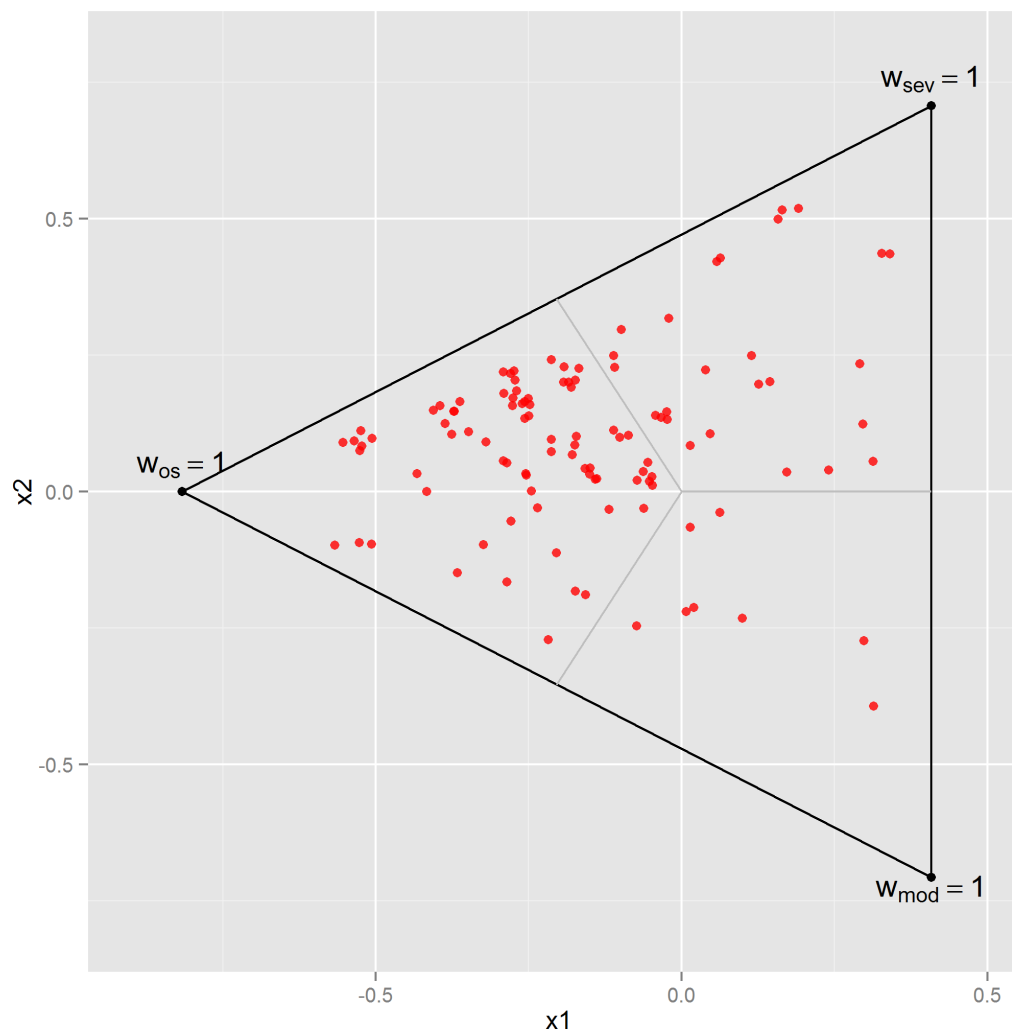


## Data analysis

- The results of the bisection swing weighting were translated into ratio constraints on the values of the weights
  - E.g., the weight for overall survival is between 1.33 and 2 times as large as the weight for severe symptoms and at least 4 times as large as the weight for moderate symptoms
- A slack-maximizing linear program formulation was used to generate a single representative weight vector
  - Weight vector that lies in the middle of the feasible weight space induced by the previously established ratio constraints as well as the requirement that the weights are non-negative and sum to one

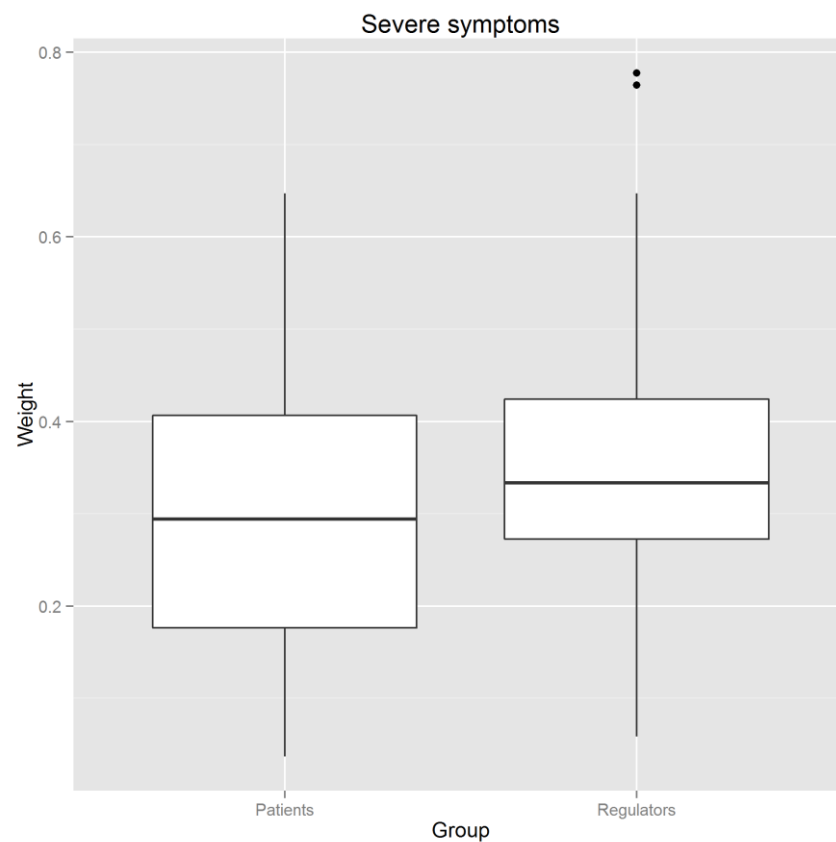
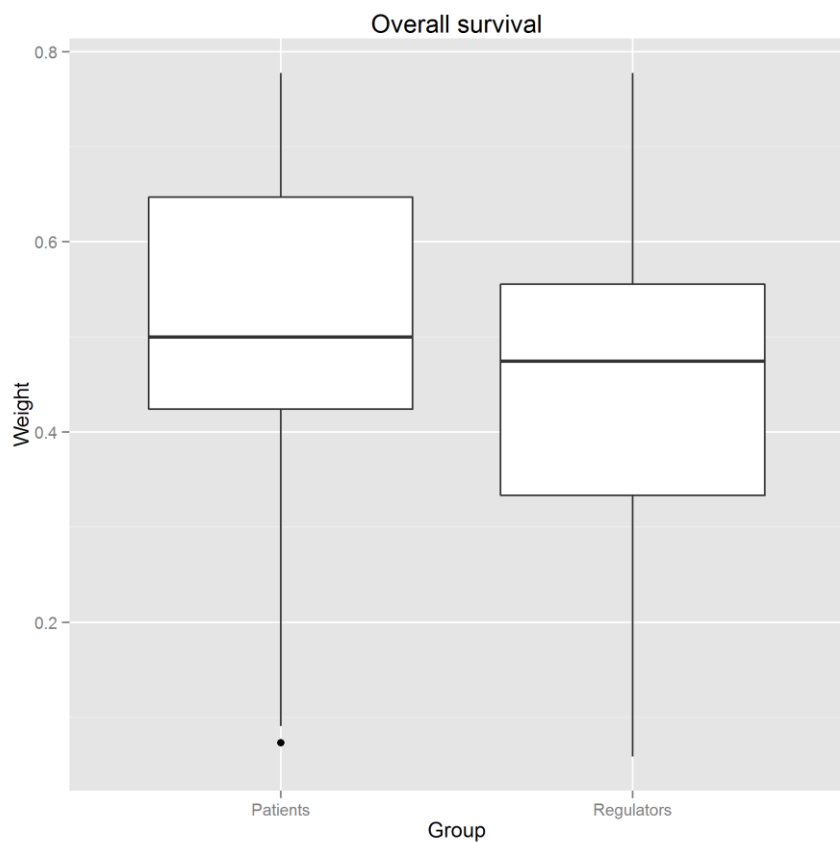


# Distribution of individual preferences



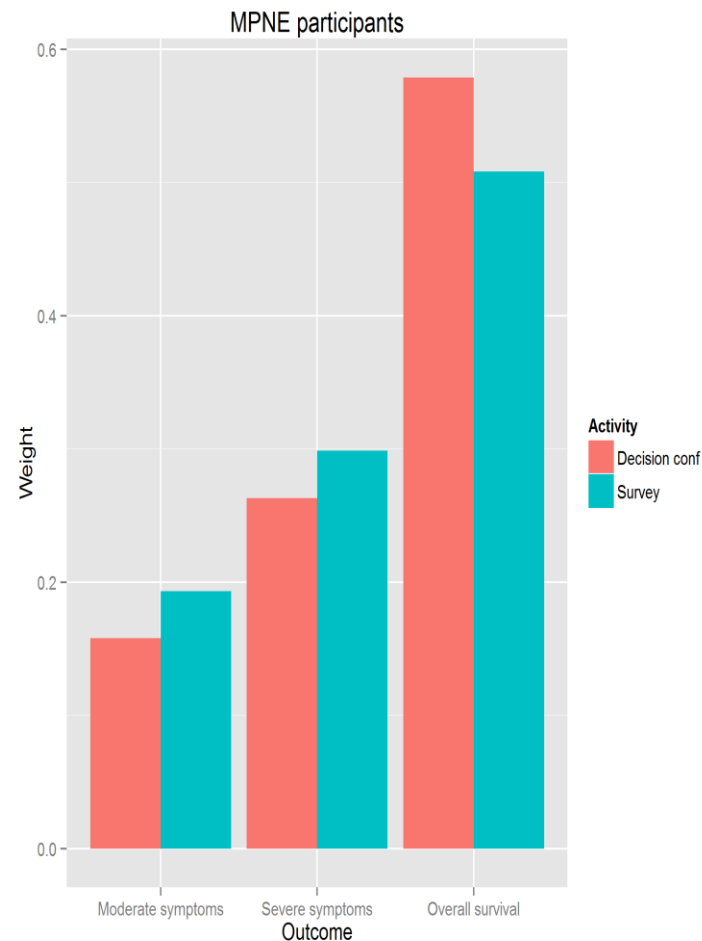
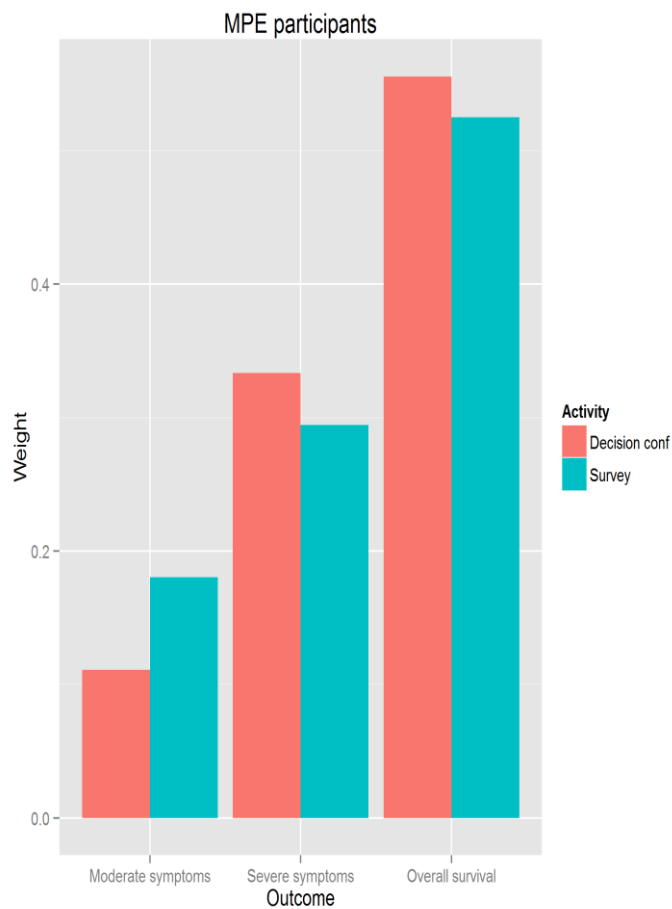


# Comparison across subgroups





# Survey versus decision conference





# Conclusions

- Ongoing pilot to explore feasibility and usefulness of eliciting patient values to inform the benefit-risk decision
- Preliminary learnings
  - It is possible to use “rapid methods” to elicit patient values
  - Need to “validate” the approach on larger samples
  - Need continue improving methodology/software
- Next steps:
  - Conduct additional studies
  - Explore synergies with other programmes and stakeholders



## Acknowledgments

EMA: F. Pignatti, N. Bere, M. Mavris, I. Moulon

UMCG: H. Hillege, J. Kuiper

MPNE: B. Ryll

MPE: A. Plate

## Further information

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