

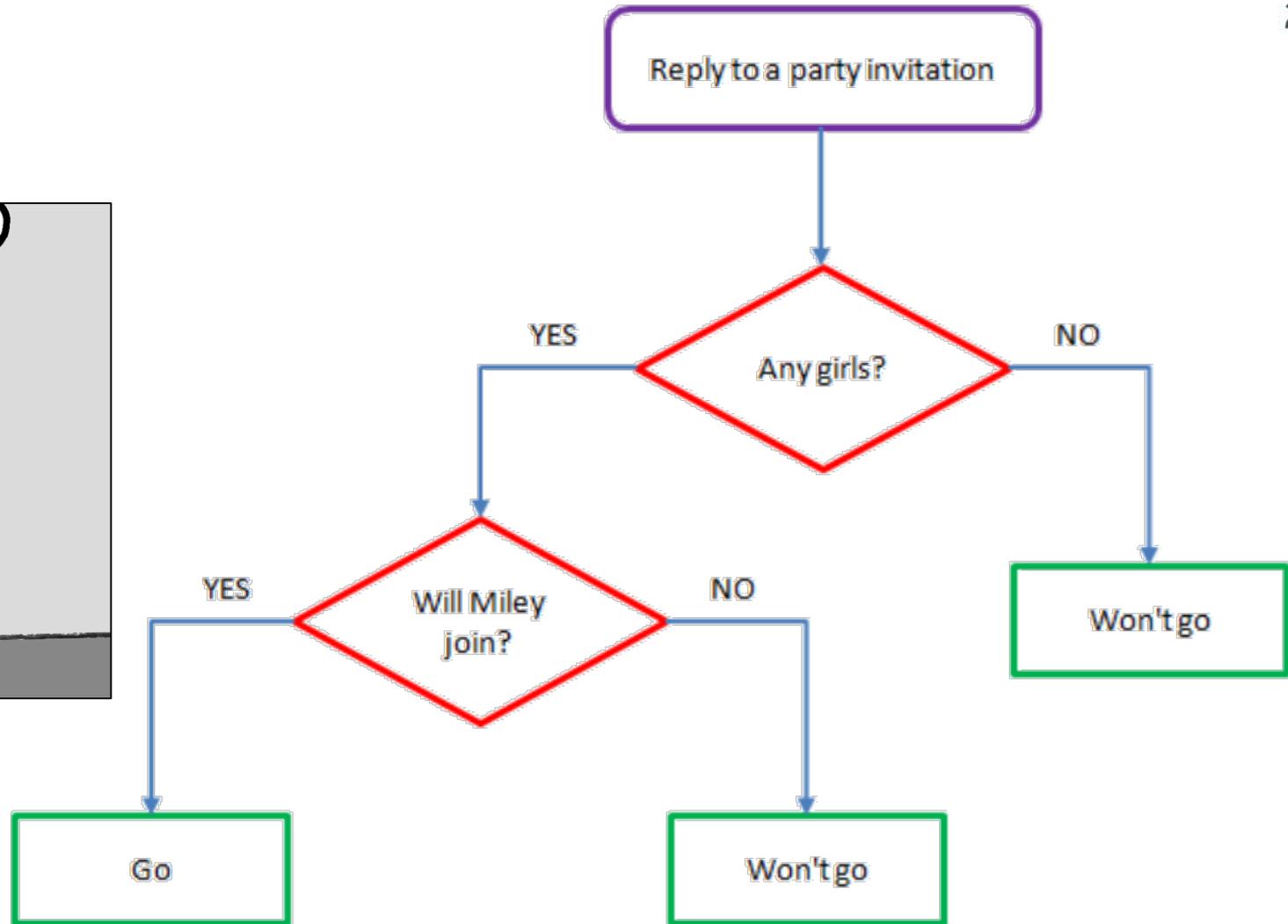
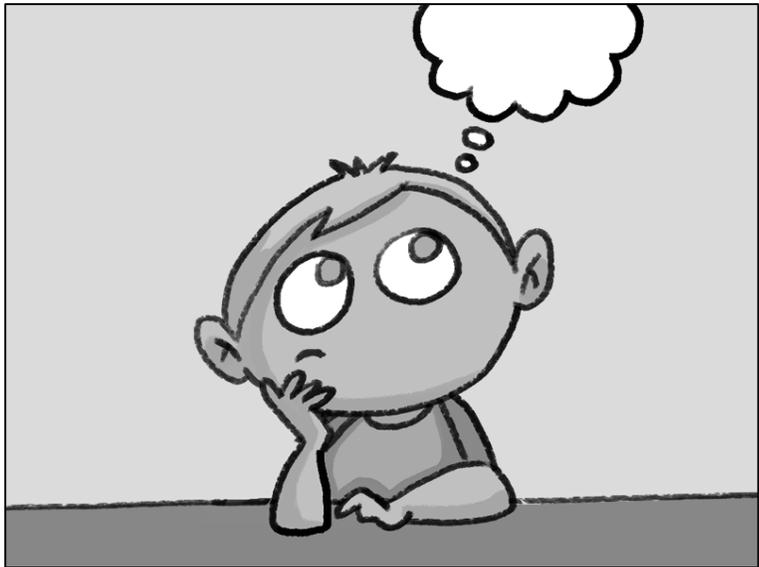
# Patient preferences

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# Some choices are tougher than others...



# Unattractive choices/trade-offs are sometimes the only chance for a patient to survive, live longer or die a less awful death



The unknown

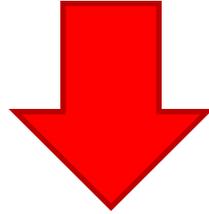


The bad



The ugly

# Patient preference studies are important for us as advocates because they enable us to influence key health system decisions in the interest of patients



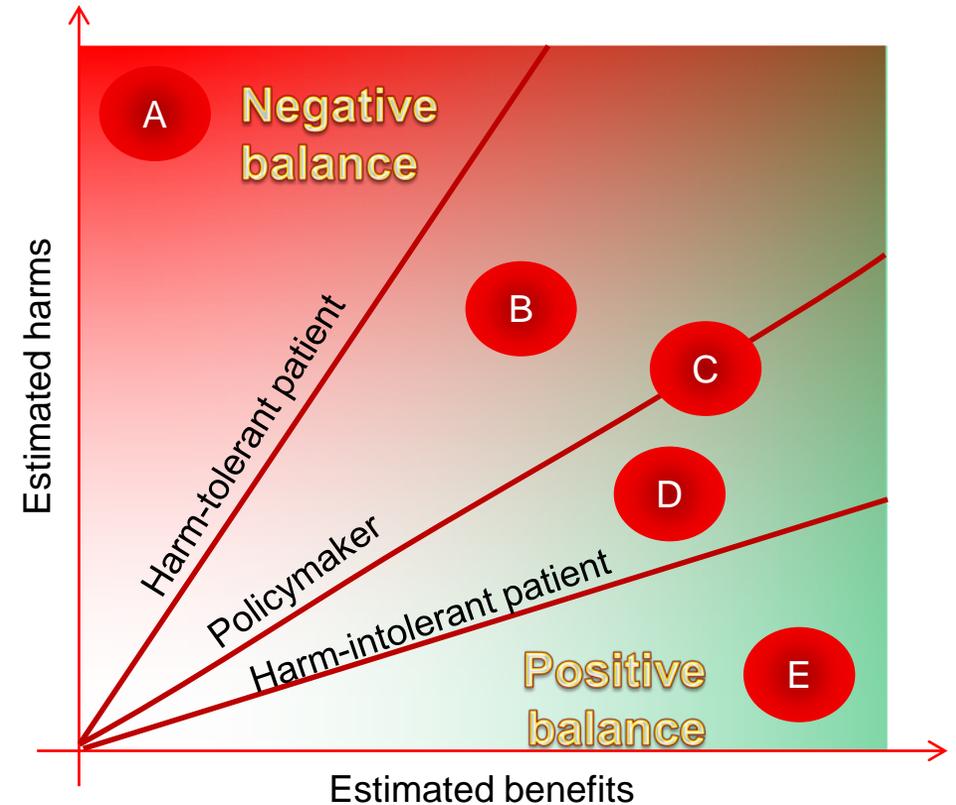
Although it is still very limited and doesn't happen in a systematic way, there is an increasing interest in incorporating patient preferences into decision making processes.

Therefore, we as advocates need to understand:

- What patient preference studies are
- What we can achieve for our patients through them
- How we can develop them
- How and when in the process we can have the highest impact, where we need it the most

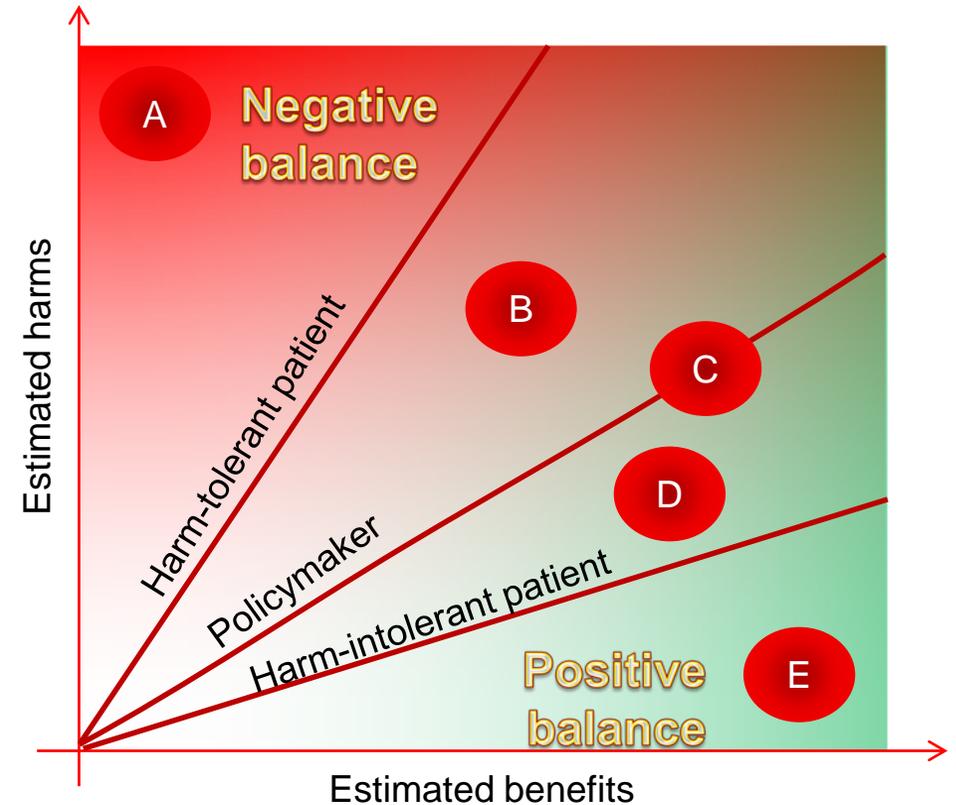
# Each of the players has own objectives, an own agenda and very different risk attitudes

- Regulators → safety, efficacy, market authorisation
- Payers → societal goals: cost-effectiveness, health care sustainability
- Industry and academic researchers → research questions, study design, high-tier publications
- Physicians → best clinical outcomes, keeping a customer
- Patients → personal goals: living a good life as long as possible



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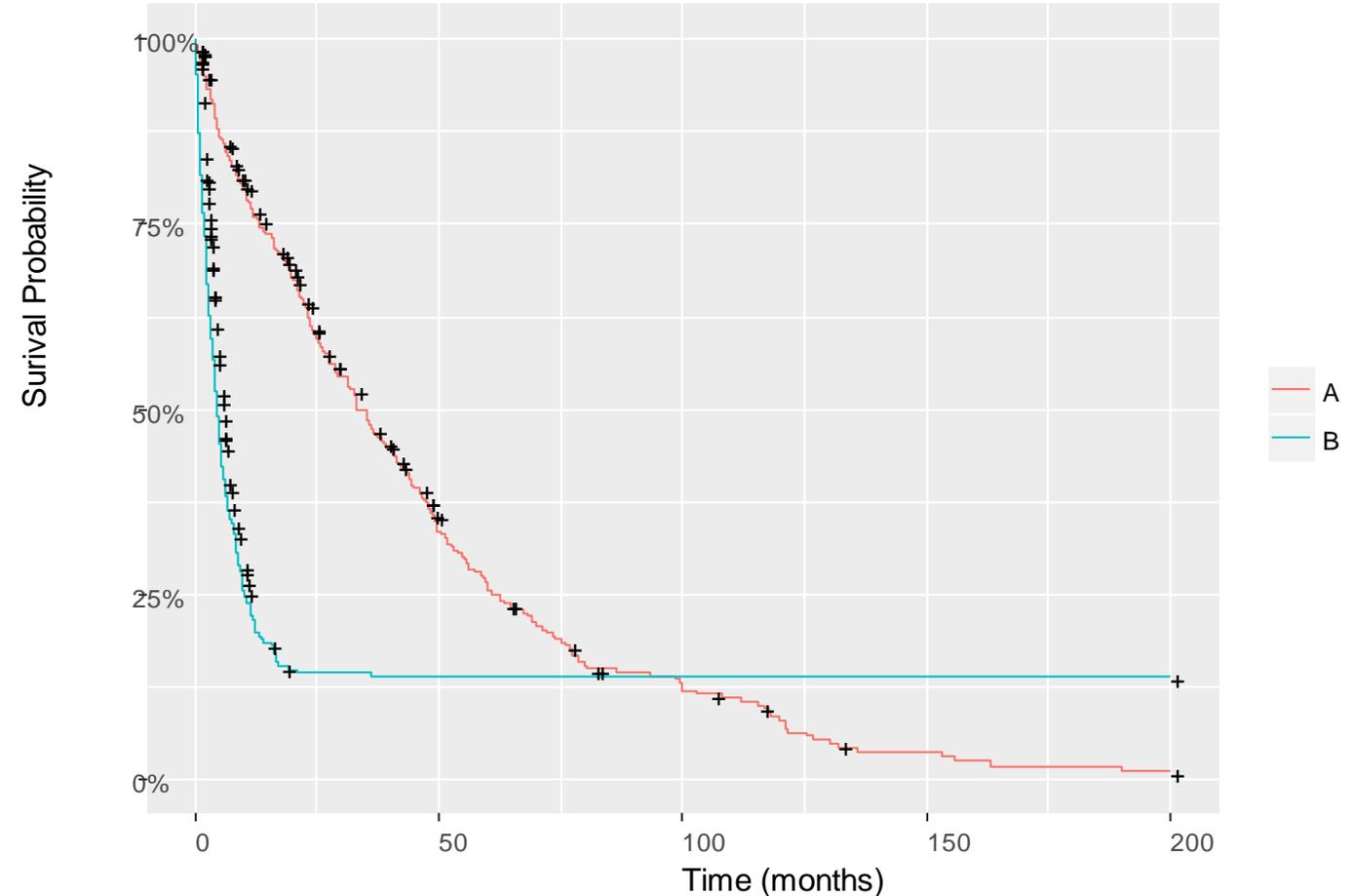


**So whose preferences should decision-makers take into account?**

# Preferences vary a lot depending on who expresses them

In the following example:

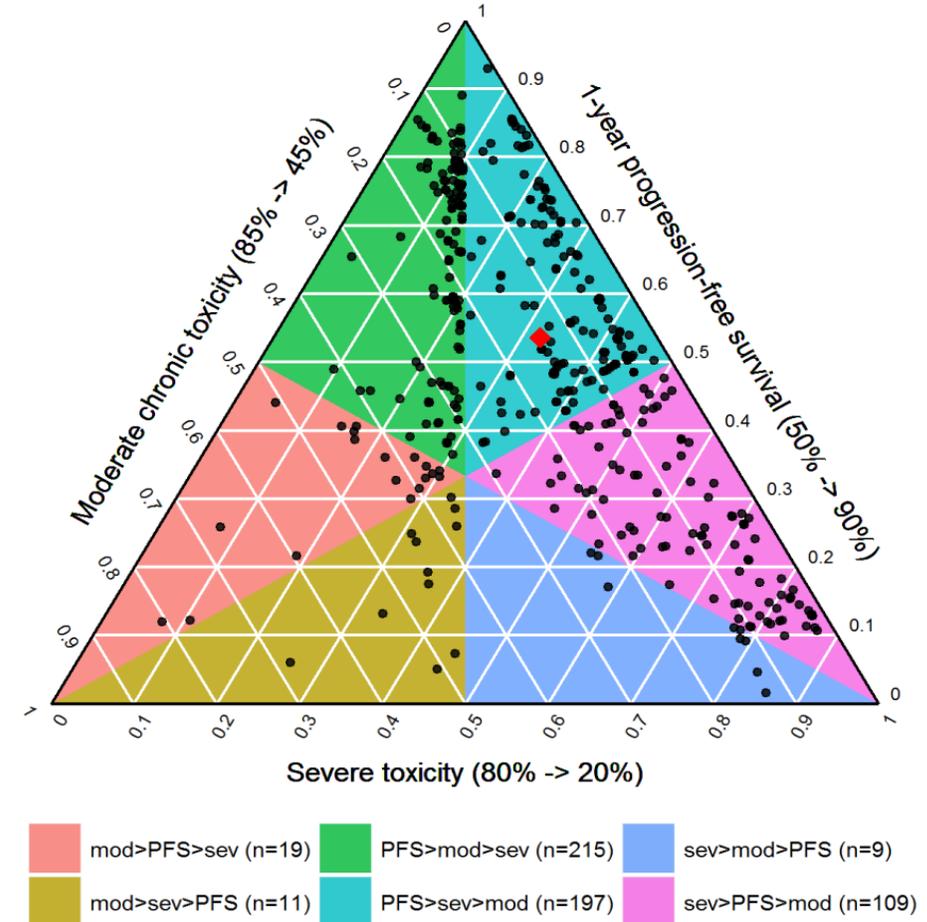
- Drug A:
  - 50% of patients will be alive in 3 years
  - all patients will be dead in 8 years
- Drug B:
  - 85% of patients will be dead in 2 years
  - 15% patients with long-term survival
- From a regulatory perspective, drug A might be better because more patients respond longer
- However, some patients may prefer treatment B because of the rare chance of surviving



Slide adapted from Francesco Pignatti

# They vary from one stakeholder to another, from one disease to another, and even within one single disease

- Needs and preferences have historically been captured in general as “the patient voice”
- However, there are clear subgroups within single diseases with very different preferences and risk attitudes
- In this example:
  - Considerable heterogeneity
  - Severe toxicity ranked higher among younger, working, and looking after dependent family members and who had more frequently experienced severe toxicity



Survey with 560 myeloma patients from the Myeloma UK, replicating the pilot of MPE, MPNE and EMA  
D. Postmus *et al.* (2017) *The Oncologist*

Slide adapted from Francesco Pignatti

# Unattractive choices are sometimes the only chance for a patient to survive, live longer or die a less awful death



The unknown



The bad



The ugly

**No-one could or should decide on behalf of individual or subgroups of patients, which of these nasty options they prefer!**

# Patient preferences tell us what certain subgroups of patients value the most when it comes to their treatment

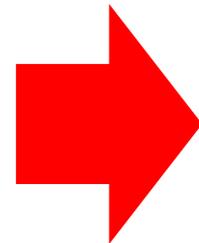
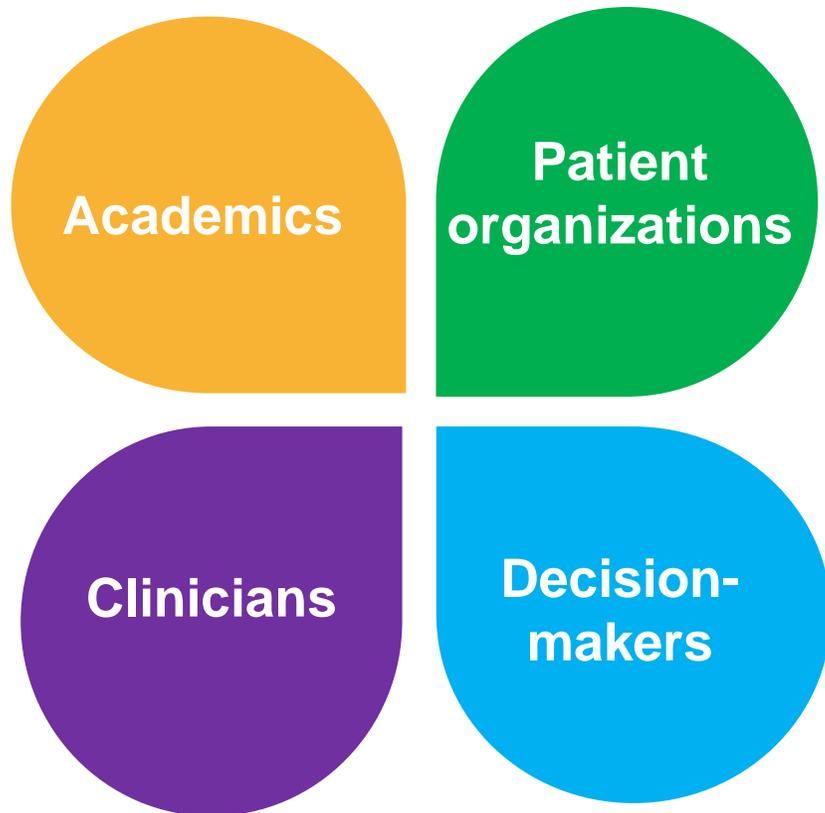
- **WHAT** outcomes of a medical product patients value the most
- **HOW MUCH** these outcomes are valued by patients
- **DIFFERENCES** in patient preferences according to patient characteristics (heterogeneity)

Pair 3	Characteristics treatment A	Characteristics treatment B
1 Life expectancy	Average	Much increased
2 Adverse events	Long-term, persistent	Middle-term
3 Therapy-free intervals	None	Short
4 Physical quality of life	Reduced mobility	Reduced mobility
5 Emotional quality of life	Always think of the disease	Always think of the disease
6 Social quality of life	Long term planning possible	No long term planning possible
7 Therapy application	Self-application	Self-application
8 Further therapies	Possible	Probably excluded
Please mark:	<input type="checkbox"/> I would choose treatment A	<input type="checkbox"/> I would choose treatment B

Attributes

Levels

**We need to develop studies with other experts to gather valid, reliable, reproduceable and generalizable data on patient preferences**



***Can decision-makers  
in regulators, clinical care,  
industry and academic research  
“trust” the results?***

# Areas of external expertise when developing patient preference studies

- Selecting the best methodology
  - Discrete choice vs. multi-criteria decision-making analysis (MCDA)
  - Quantitative vs. qualitative analysis vs. both?
- Avoiding bias in preference studies
- Selecting attributes to measure
- Measuring and dealing with preference heterogeneity
- Asking questions in the right way
- Reducing the cognitive burden of preference surveys
- Eligibility criteria
- Ensuring a “robust” sample or results
- Ethics approval (why, where and how?)
- Data analysis, storage and ownership
- Peer review and publication

Patient organisations increasingly take the lead in developing these type of studies. However, collaborations with academia are very important to ensure scientific rigour and utility

# Our role as advocates...

- Provide knowledge of the disease pathway and direct interaction with patients
- Work on protocol to make sure it reflects the core questions and what is acceptable to patients
  - Survey acceptability (length, complexity)
  - Sensitivity (e.g. survival questions)
- Recruit patients from within our membership
- Utilise findings for external engagement of patient organisations with industry, regulators, payers and policy-makers
- Help involved stakeholders understand how to determine what an acceptable risk is and whether an unknown risk is acceptable

Patient advocacy has a role in helping establish a non-paternalistic framework for patients to express their preferences, ensure that the content is accurate/appropriate and that the data generated is useful

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**Stakeholders are  
obsessed in being  
able to measure risk  
but there is little  
discussion about  
whether this is really  
in the interest of  
patients**

*“For a chance to survive, patients [...] prefer an unknown risk to a known risk with insufficient benefit [...]”*

B. Ryll (2019) Nature

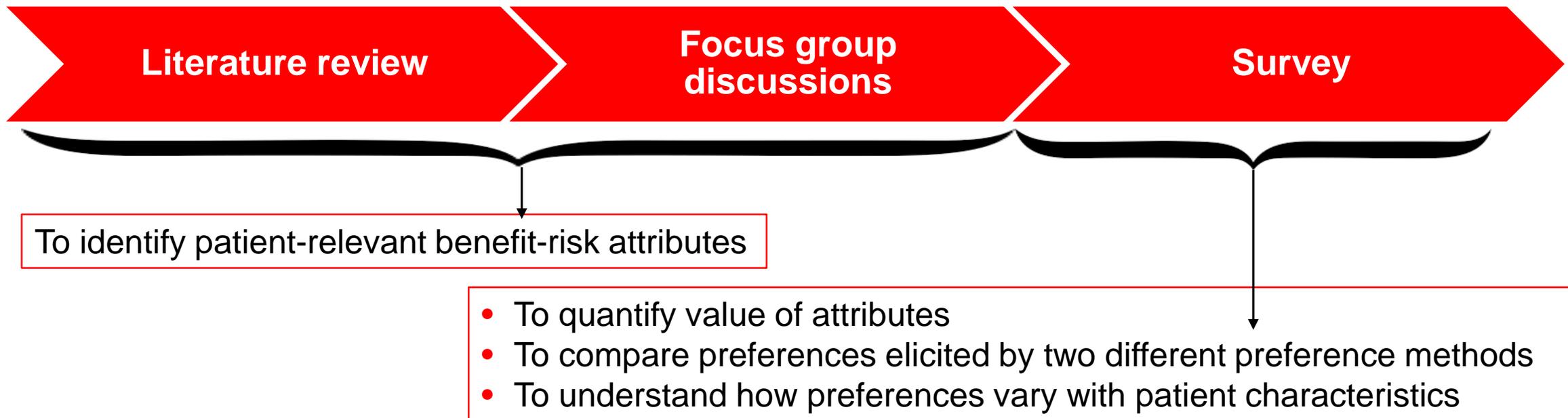
## Example: IMI PREFER

### Methodological objectives

- To compare preferences elicited by two different preference methods (DCE vs SW)
- To understand how preferences vary with patient characteristics

### Clinical objectives

- To identify and quantify patient-relevant benefit-risk attributes of MM treatments
- To quantify the value of benefit-risk attributes of MM treatment



# Conclusions

- **Patient Preferences vary** from one stakeholder to another, from one disease to another, but also within one single disease, forming sub-groups with preferences that are clearly different
- **Patients know best what to trade between the bad and the ugly, and whether they prefer an unknown risk to a known unsatisfying outcome**
  - No stakeholder should express patient preferences on their behalf
  - Data not directly gathered from patients should not have the same weight
- **Patient preferences** are becoming a key element of the decision-making process
- **Patient advocates need to understand patient preference studies** well and have a clear strategy on how to become involved in generating these data

# Acknowledgements



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