

Structural Implementation of Patient Preferences in Health Technology Assessment: *Will we ever get there?*

A question raised by IMI **PREFER**

ISPOR Europe 2018

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Panelists:

- HTA: Marco Petschulies, Federal Joint Committee (G-BA), Germany
- Patient advocate: Ken Mastris, European Cancer Patient Coalition (ECPC), Netherlands
- Academia: David Mott, Evidera, United Kingdom

About the PREFER project

Disclaimer: This presentation and its contents reflects the view of the presenter and not the view of PREFER, IMI, the European Union or EFPIA.



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Patient preferences

*“Qualitative or quantitative assessments of the relative **desirability or acceptability** to **patients** of specified **alternatives or choices** among **outcomes or other attributes** that differ among alternative **health interventions**”¹*

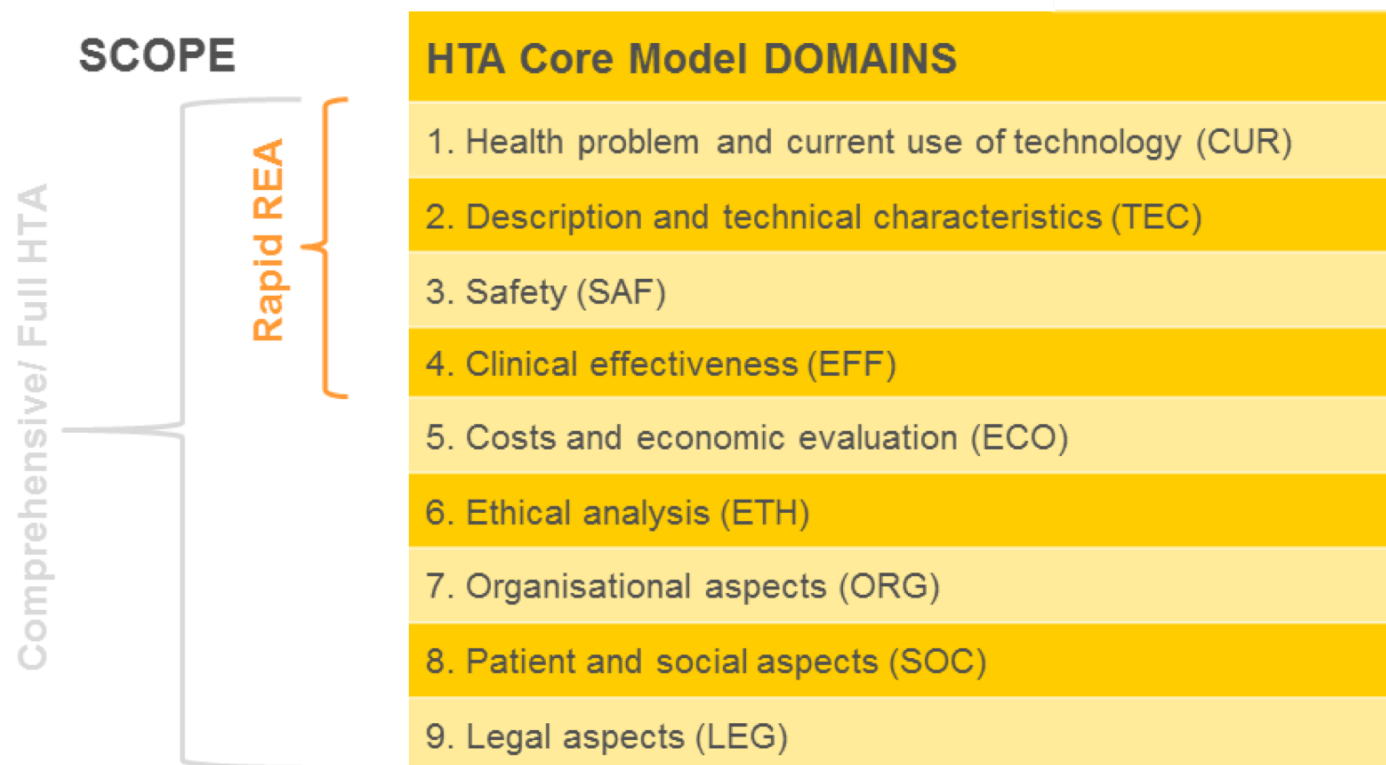


Patient preferences in HTA

- Concerns exist regarding:
 - **patient vs. public preferences**
 - **methodological issues**
 - when and how to **use them in HTA**

Patient preferences in HTA

EUnetHTA HTA Core Model[®]



Objectives of the issue panel

1 IF

Should we consider patient preferences in HTA?

2 WHEN

In what cases should we consider them?

3 HOW

Can they be included in the HTA process?

A

In what part of HTA?

B

Barriers: methodological or operational?

Panelists

HTA

Marco Petschulies
Federal Joint Committee (G-BA)
Germany



Patient

Ken Mastris
European Cancer Patient Coalition (ECPC)
Netherlands



Academic

David Mott
Evidera
United Kingdom



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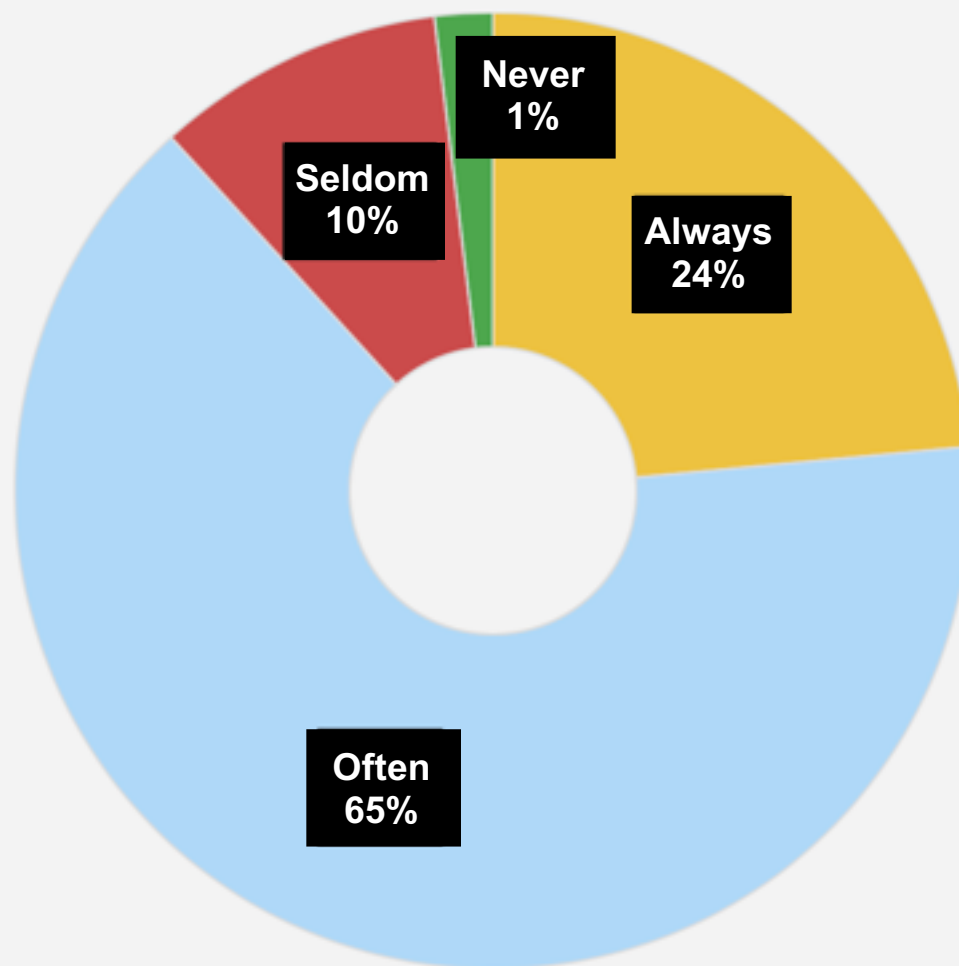
Poll with audience

- Should patient preferences be taken into account in HTA?
(choose one option only)
 - Always
 - Often
 - Seldom
 - Never

Poll with audience

Should patient preferences be taken into account in HTA?

(n=51)



HTA perspective

Marco Petschulies

Federal Joint Committee (G-BA)

Germany



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1

IF

Should we consider patient preferences in HTA?

We would like to...

- Potentially helpful in any assessment
- Patients' perspective key component of the benefit assessment in Germany

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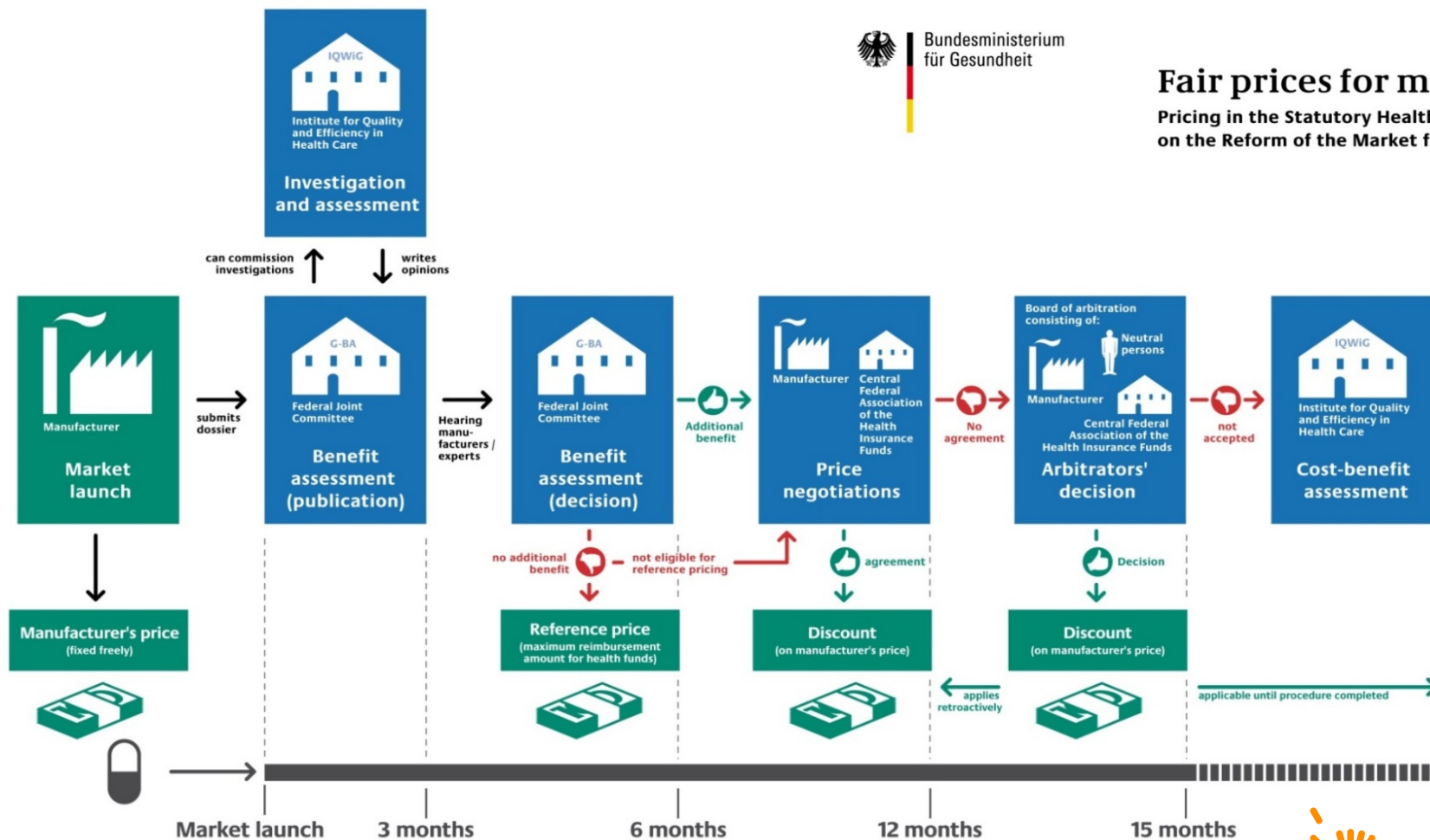


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2 WHEN

In what cases should we consider patient preferences?



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In what cases should we consider patient preferences?

- Determining patient-relevant endpoints
 - G-BA definition: directly and perceptibly effect health status
 - usually excludes laboratory values or radiographic assessments if not validated surrogates
- Weighing advantages and disadvantages
 - e.g. less relapses versus more gastrointestinal side effects
 - What's more important? Is there an additional benefit? To what extent?

Can patient preferences be included in the HTA process?

Status quo of integrating the patients' perspective

- Patient representatives
 - Present at all meetings
 - Nominated and organized in major national patient organizations
 - Participation facilitated by a special department in the G-BA
 - Vetting (Col) and educating of candidate representatives
- Patient-reported outcomes
 - Evaluated in majority of assessments
 - Methods to assess validity of questionnaires, determining MIDs better established



3

HOW

Can patient preferences be included in the HTA process?

A

In what part of HTA can patient preferences be included?

➤ Company dossier

- Submitted at the day of market entry in Germany
- 5 modules: section for additional evidence in M4

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3

HOW

Can patient preferences be included in the HTA process?

B

What barriers block the integration of patient preferences in HTA: methodological or operational?

- Operational/procedural barriers can be overcome
 - Code of procedures/dossier templates are regularly updated
- Methodological issues more significant
 - Potentially influenced/biased by interviewee selection, disease/treatment stage, phrasing of questions, elicitation method
 - Study objective has to match HTA questions

Thank you!



Patient perspective

Ken Mastris

European Cancer Patient Coalition (ECPC)

Netherlands



1

IF

Should we consider patient preferences in HTA?

- A large body of research on patient preferences acknowledges the importance of the patient's perspective: patients provide valuable experiential knowledge about living with a condition.
- Patient-related factors such as patients' adherence to treatment, patients' satisfaction with treatment, and experienced health outcomes, all determined by patient preferences, are important determinants for the uptake of healthcare interventions.

1

IF

Should we consider patient preferences in HTA?

- Consideration of patient preferences in healthcare policy decisions may improve the uptake and real-world efficiency of healthcare technologies. This may lead to a higher public acceptance of market authorization decisions where patients are empowered to take control over their own health.
- Outcome measures used in healthcare evaluations do not necessarily consider patients' perspectives. Although, the consideration of patient views is ethically the right thing to do: as it promotes transparency and legitimacy because the ones affected by the decisions are involved in the decision-making process.

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In what cases should we consider patient preferences?

Patients can be involved by different means:

1. direct representation through participation in a committee or advisory group
2. indirect approach using methods that allow the study of a patient's preferences regarding treatment characteristics (e.g., benefits and risk)
3. These are not mutually exclusive.

In what cases should we consider patient preferences?

- Preference elicitation methods can be considered a form of consultation for benefit-risk assessments. Furthermore, benefit-risk assessments, as a process, can determine "whether a treatment's benefits outweigh its harms when compared to standard-of-care or other alternative treatment."
- Decisions by HTA bodies have a large impact in determining which treatments patients and their clinicians have access to. However, there is no agreement about how best to use patient's perspectives with other types of evidence calculated in the cost of new treatments.

3

HOW

Can patient preferences be included in the HTA process?

A

In what part of HTA can patient preferences be included?

- There are many different aspects of HTA where patients can contribute: research, context, scientific assessment, value, appraisal, recommendation, decision, and governance.
- Patient views may be important in all stages from early drug development, market authorization, and can continue within HTA and after HTA recommendations are made.

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3

HOW

Can patient preferences be included in the HTA process?

A

In what part of HTA can patient preferences be included?

- A starting point for any patient or patient group is to examine the inputs to their local HTA process, and assess whether it is fair and accountable.
- HTA bodies need to understand how all patients with a condition will be affected by a new technology. (e.g, rare diseases).

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3

HOW

Can patient preferences be included in the HTA process?

B

What barriers block the integration of patient preferences in HTA: methodological or operational?

1. Variation: HTA is not used by all EU Member States and, where it is used, divergences in processes and methods result in delays and variations in approval decisions and inequities in access.
2. Limitations in data used for decision-making: HTA should involve a comprehensive evidence-based evaluation encompassing patient-reported outcomes (including QoL) and the wider economic implications of new treatments, in addition to survival outcomes.

3

HOW

Can patient preferences be included in the HTA process?

B

What barriers block the integration of patient preferences in HTA: methodological or operational?

3. Lack of patient involvement: Very few HTA agencies involve patients in their assessments and, where public engagement is sought, the approaches vary. Patient involvement in HTA is often at public consultations, in providing evidence and in appeals against decisions.
4. Duplication: Parallel assessments by individual HTA bodies based on a common evidence base represents a wasteful duplication of efforts and resources.

Thank you!



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Academic perspective

David Mott

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Structural Implementation of Patient Preferences in Health Technology Assessment: Will We Ever Get There? An Academic Perspective

David J. Mott, PhD

1

IF

Should we consider patient preferences in HTA?

- Economic theory **does not** provide an answer to this question.
- In many countries, **cost-utility analysis (CUA)** is a key component of HTA, which **does not require** patient preference information.
- However, **patients are often involved** in the HTA process in some capacity.

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1

IF

Should we consider patient preferences in HTA?

- Patient preferences are useful when trying to determine **the true value** of a health technology.
- However, **it is up to decision-makers** to determine whether patient preferences should be considered in the HTA process, and in what manner.

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2 WHEN In what cases should we consider patient preferences?

- The consideration of patient preferences **could be limited to special cases**, but is this really appropriate?
- If patient preferences are to be considered at all, arguably there should be scope for them to be considered **in any appraisal**.

A In what part of HTA can patient preferences be included?

Type	Application in the HTA Process	Methodology
Direct Incorporation	Health state utilities (for QALYs)	• Cost-Utility Analysis
	Willingness to pay estimates	• Cost-Benefit Analysis
	Estimates of uptake	• Model-Based Evaluations
	Weighting of criteria/endpoints	• Multi-Criteria Decision Analysis
Supporting Evidence	Considered by decision-makers alongside other evidence (e.g. clinical, cost-effectiveness)	• Discrete Choice Experiments
		• Best-Worst Scaling
		• Semi-Structured Interviews
		• Focus Groups

3

HOW

Can patient preferences be included in the HTA process?

B

What barriers block the integration of patient preferences in HTA: methodological or operational?

- There are a **range of methodologies** that can be used, many of which have a long history and have established best practice guidelines.
- The challenge is that the **extent to which decision-makers value patient preferences** is currently unclear in many countries.

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3

HOW

Can patient preferences be included in the HTA process?

B

What barriers block the integration of patient preferences in HTA: methodological or operational?

- Guidance should be provided by decision-makers on **how and when** patient preference information should be provided (if at all).
- With **greater commitment** from decision-makers, more resources can be invested to overcome any methodological concerns.

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Thank you for listening

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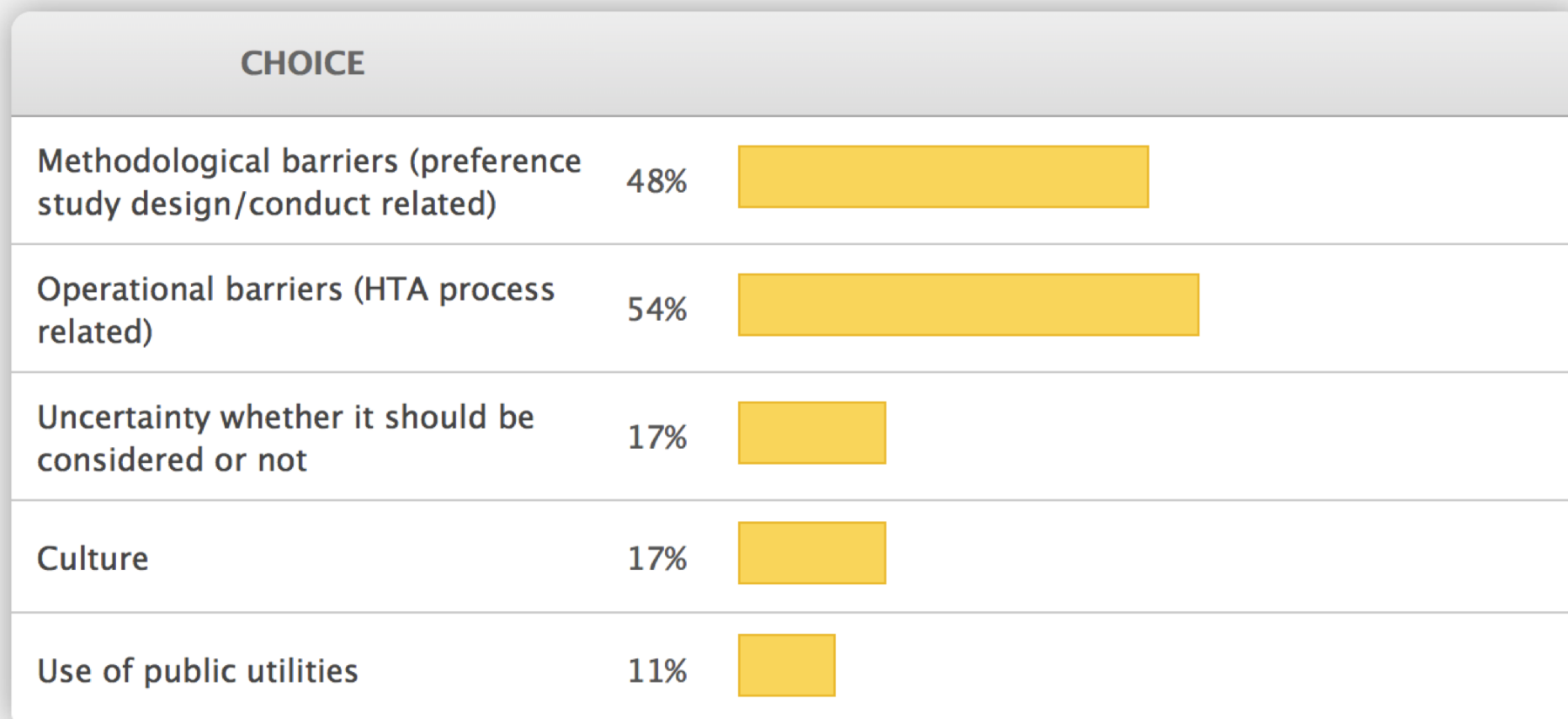
Poll with audience

- What is blocking the integration of patient preferences in HTA? (choose one or multiple options)
 - Methodological barriers (preference study design/conduct related)
 - Operational barriers (HTA process related)
 - Uncertainty whether it should be considered or not
 - Culture
 - Use of public utilities

Poll with audience

What is blocking the integration of patient preferences in HTA?

(n=46)



Q&A

Thank you!

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Publications:

- van Overbeeke E, Whichello C, Janssens R, Veldwijk J, Cleemput I, Simoens S, Juhaeri J, Levitan B, Kübler J, de Bekker-Grob E, Huys I. **Factors and situations influencing the value of patient preference studies** along the medical product lifecycle: a literature review. *Drug Discovery Today*. 2018
- ResearchGate: Patient Preferences in benefit risk assessments during the drug life cycle (PREFER)

Posters at ISPOR Europe 2018:

- PRM199: **Stakeholder perspectives** on conduct and use of patient preference studies along the medical product lifecycle: results from focus groups
- PMU100: Mapping benefit-risk **decision-making processes** and identifying decision points with the potential to include patient preference information throughout the medical product lifecycle
- PRM218: Characterising and appraising patient preference exploration and elicitation **methods** in the medical product lifecycle
- PMU103: Incorporating **psychological constructs** into patient preference studies: which you should consider including in your next study?