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Improving healthcare decisions

W7: Preference Research in HTA Agencies Europe – A Review of Its Use in Approval, Reimbursement and Pricing

Presented by the:

ISPOR Stated Preference Special Interest Group

ISPOR Europe 2018 in Barcelona, Spain

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Introduction

Background:

- Healthcare decisions include multiple tradeoffs. Therefore regulatory decisions often involve value judgments.
- Increased discussion about how to incorporate stakeholder preferences into health care decisions, to inform value judgements.
- However, no single method is appropriate for all health care decisions.
- The varied and evolving requirements of decision makers make it difficult for evidence generation to support market access in Europe.
- In 2017-2018, ISPOR engaged a team of ISPOR members in a review of the use of preference data and health preference research in approval, reimbursement, pricing and guideline decisions.

Objective:

- To improve evidence gathering and potentially develop standards in the future, the ISPOR Stated Preference Special Interest Group (SIG) mapped European decision makers usage of quantitative preference data generated **using any preference method**, **for any stakeholder group**, to inform approval, reimbursement or pricing decisions.

  - *Which decisions* are informed by preference data
  - *Whose preferences* are elicited using *which methods*
  - *Which EU member states* already incorporate stakeholder preferences in their healthcare decisions
  - *Which EU member states* do not use stakeholder preferences to inform value judgments

Scope:

- Decisions: “regulatory” – approval, reimbursement, pricing and guidelines
- EU: EU member states (+ Norway, the Russian Federation and Switzerland)
- Preferences elicitation: quantitative
- Technology: pharmaceuticals, devices and diagnostics
- Stakeholders: patients, decision makers, providers, citizens, clinicians, insurees, other
- Language: English (+ website review, if team member was familiar with the local language)

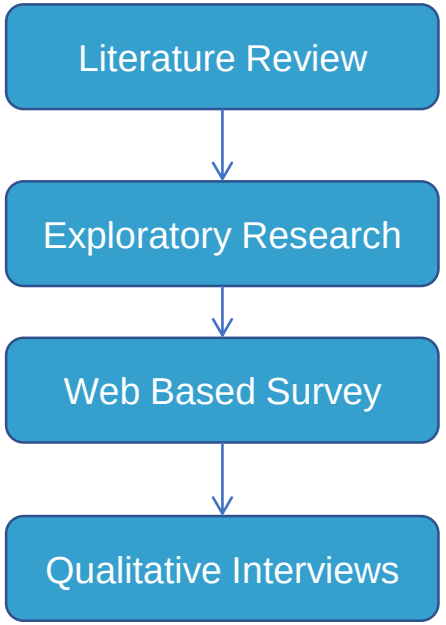
2

Methods

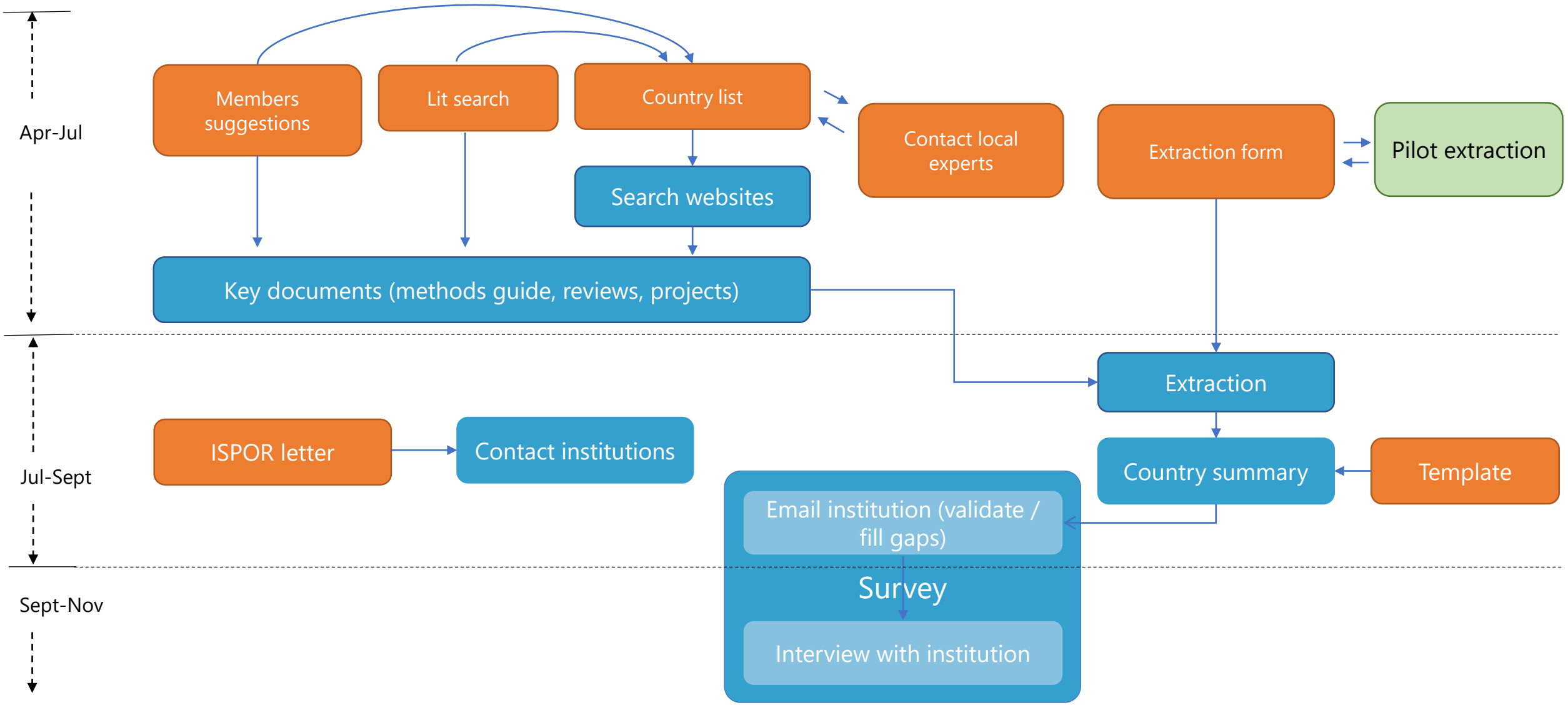
Study Design: Mixed methods and triangulation

- A mixed method study was performed which collected, analyzed and integrated **quantitative and qualitative research**.

- **Triangulation**, i.e., the use of several methods, data sources and researchers to examine the same phenomenon:



Overview of the Review



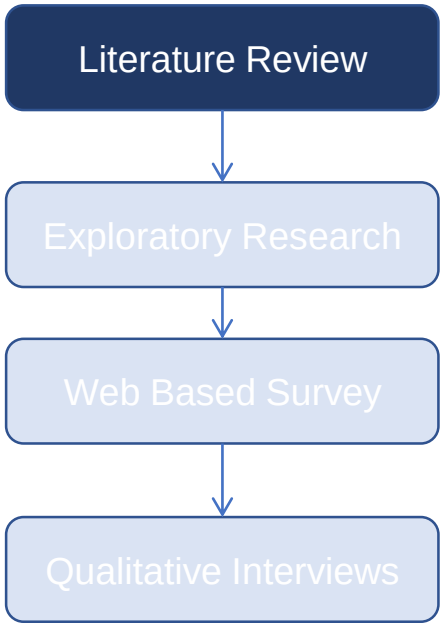
Inclusion/ exclusion criteria: literature review

	Inclusion	Exclusion
Study/ document type	Research to elicit preferences Research on preference methods Guidance on preference methods	
Methods	Quantitative preferences (for either criteria / attributes or for a technology)	Qualitative methods e.g. stakeholders involved in committees
Decision makers	Used or commissioned by a decision maker, or an assessment agency that is associated with a decision maker	Independent academic or industry research not used in decision making*
	Approval, reimbursement, pricing	All other decisions
	All levels (national, regional, local)	None
Stakeholders	All	None
Technology	All	None
Location	EU28, Norway, Switzerland and Russia	Other countries
Language	English	Non-English

*The definition of independent research that does not influence decision will be refined during the project. Please share examples of research that test this boundary, so that we can discuss as a team

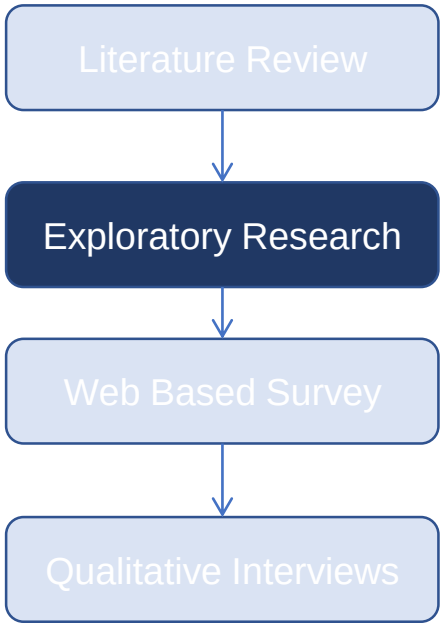
Literature Review

- The aim was to identify the use of preference data collected using any quantitative method – one that placed a quantitative estimate on preferences.
- Conducted in Medline, EMBASE and EconLit on the 26th June 2017
- Following a review of titles and abstracts by two members, 140 publications were identified that might include reference to the use of preference data for relevant decisions.



Exploratory Research

- The aim was to identify and report on activities in the use of preference data for each EU member state.
- All EU member states were included in the country review, institutions were selected if they were members of EUnetHTA .
- Members of research group drafted a country report on the use of preference data (the reports were reviewed by a co-investigator).



Web Based Survey

- Quantitative dimensions of ho
  members use preference data
  - Ranking
  - Rating
  - Pairwise comparison
  - Choice-based
  - Matching methods
- Descriptive analysis (frequenc
  to analyse the response .
- Qualitative answers were anal
  separately and are addressed
  text were relevant.

Discrete choice experiments (DCEs)

Description

Stakeholders are asked a number of choice tasks, in each indicating their preference between 2 or more treatments. The treatments are described by levels of performance on a set of attributes.

Analysis of stakeholder choices can determine how variation in the levels of performance on different attributes influences their choices.

Illustration

Treatments are distinguished by three attributes, each defined by 2 levels of performance, X or Y.

Which treatment do you prefer?

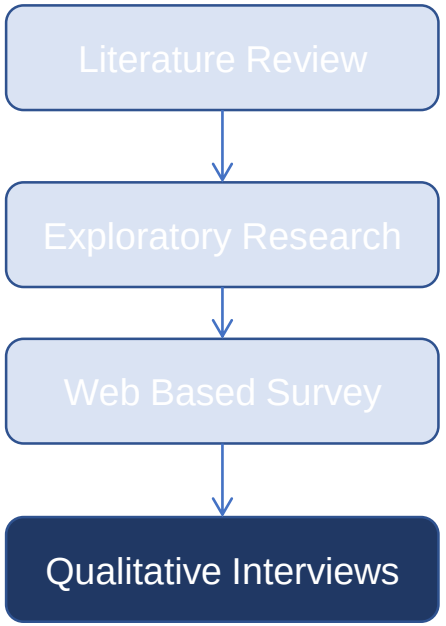
	Treatment 1	Treatment 2
Attribute 1	X	X
Attribute 2	X	Y
Attribute 3	Y	X
	☐	☐

Examples of these methods are:

- US Food and Drug Administration, evaluation of the maximum acceptable mortality risk associated with weight loss devices. CLICK FOR PAPER
- IQWiG pilot of DCE to prioritize patient-relevant outcomes from an antiviral therapy for chronic hepatitis. CLICK FOR PAPER

Qualitative Interviews

- To fill in any gaps in the survey and confirm the study's findings in-country experts and ISPOR members.
- Performed to document what meanings decision makers give to their actions, and what issues concern them while implementing guidelines and methods
- Experts were identified by reviewing the authorship of relevant studies identified in the literature and the website review .
- In-depth interviews were performed to refine country reports.



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Results

Live Content Slide

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Poll: Whose preferences are being quantified as part of approval decisions?

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Poll: Whose preferences are being quantified as part of pricing and reimbursement decisions?

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Poll: Which methods are being used for eliciting general population preferences?

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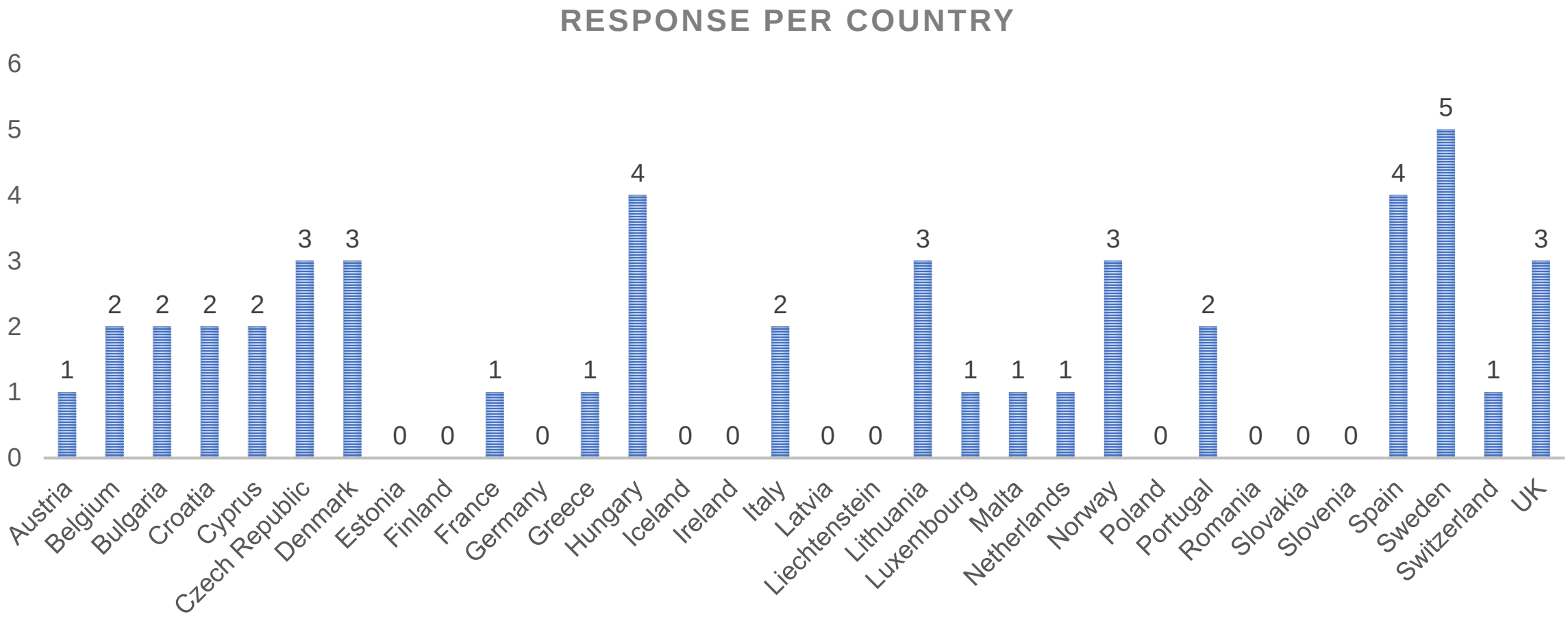
Poll: Which methods are being used for eliciting patient preferences?

3.1

Survey Results

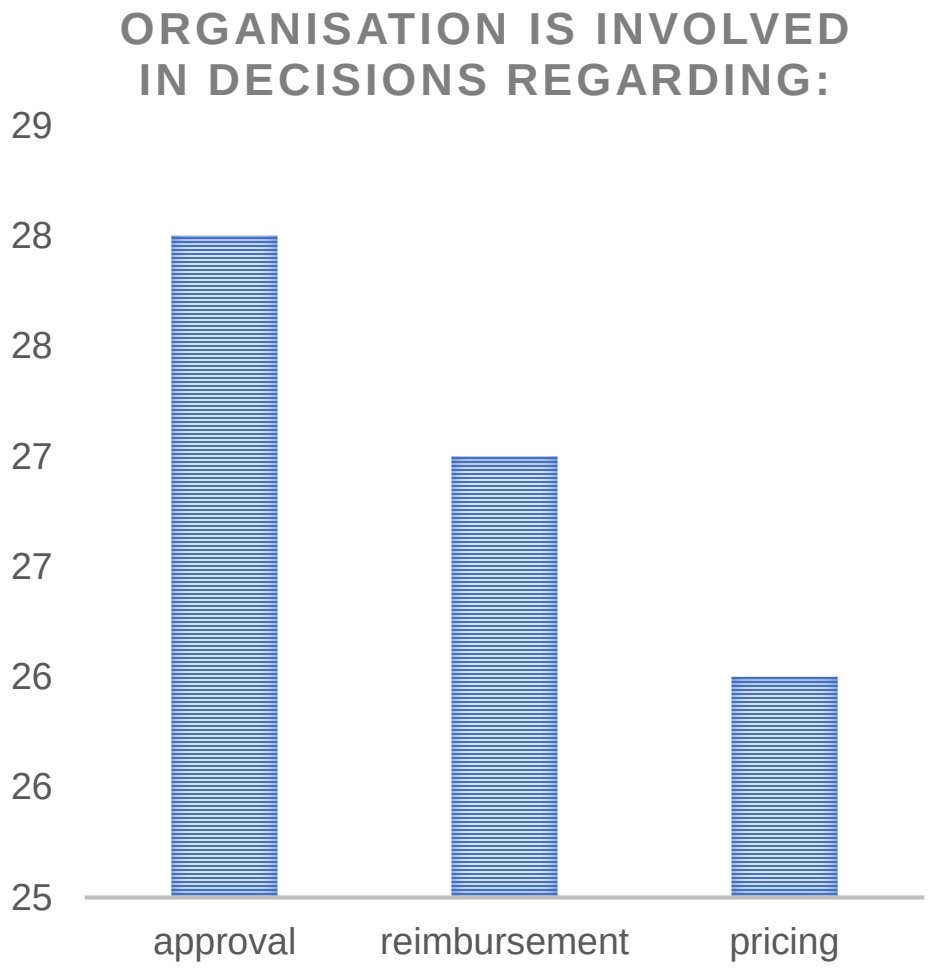
Survey responses - countries

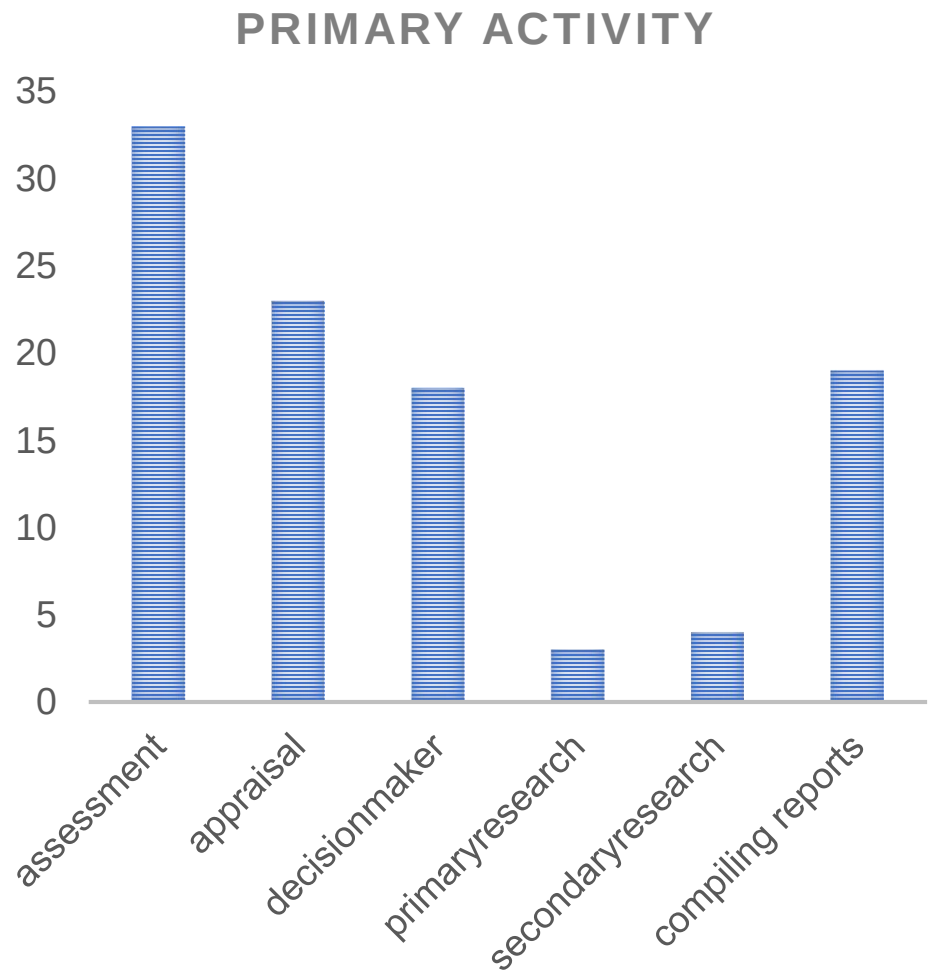
- 47 valid responses from 21 countries, and 1 for response for Europe



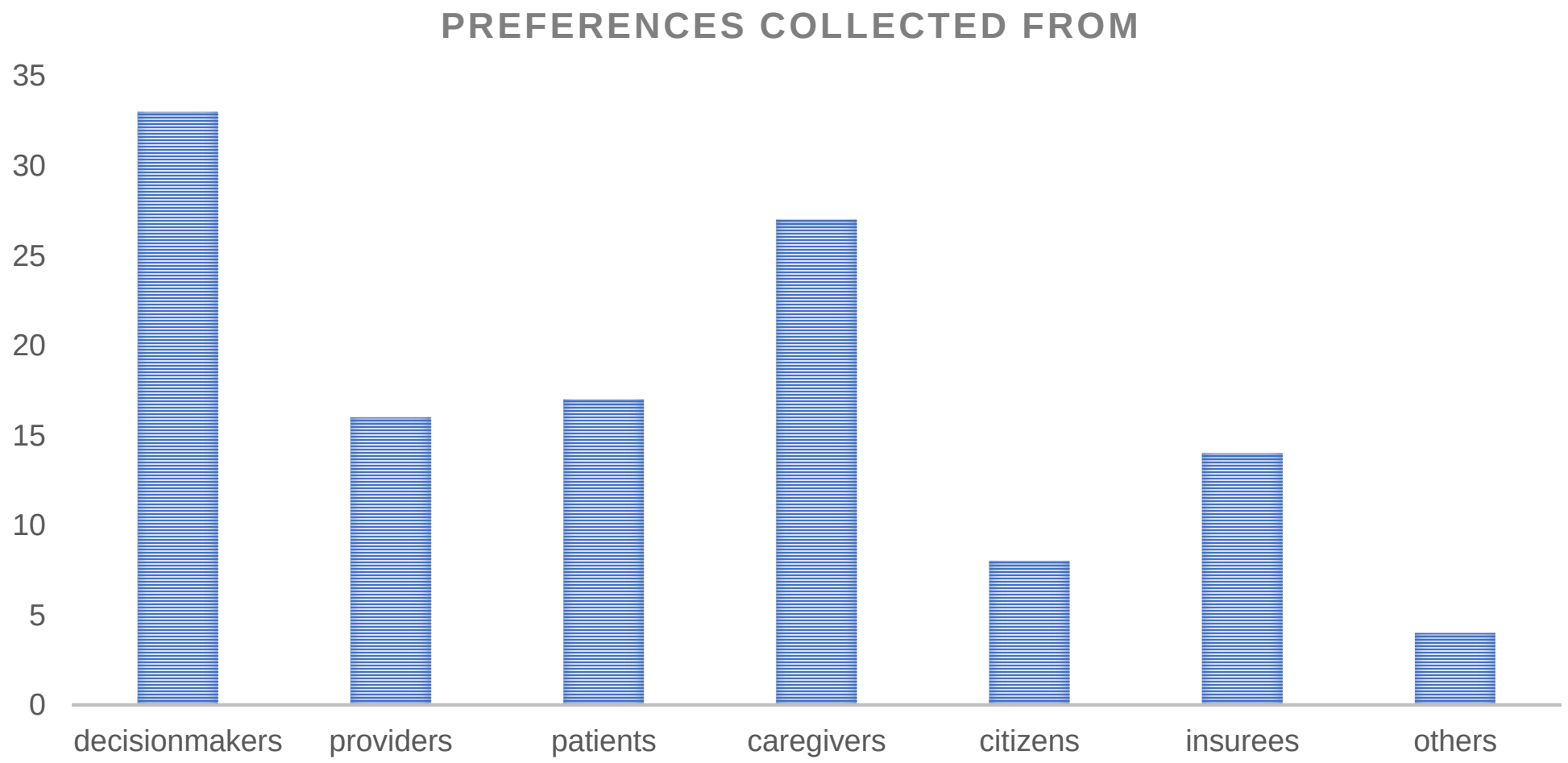
Results until 1 October 2018

Survey responses - organisations

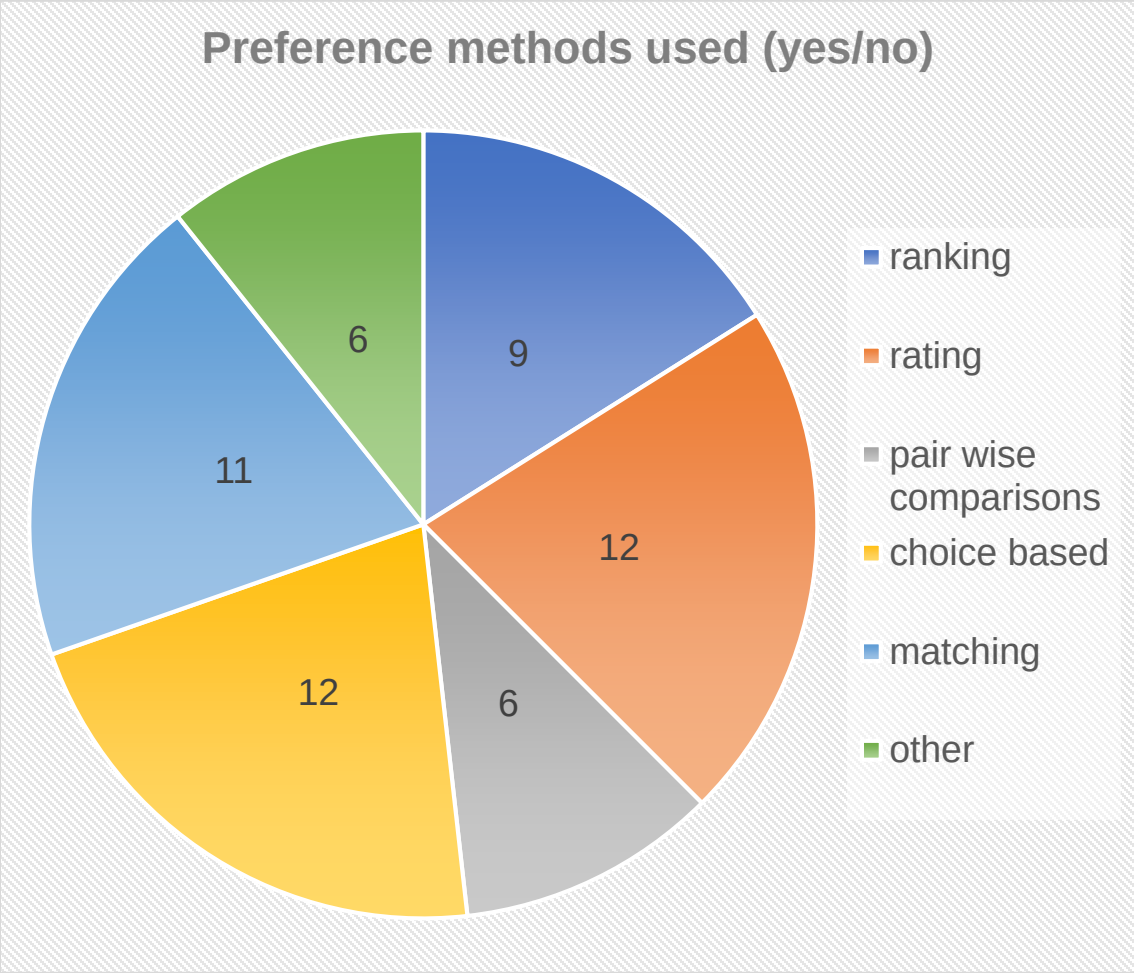




Whose preferences are being quantified as part of healthcare decisions?



Which methods are being used for eliciting stakeholder preferences?



	decision-makers	providers	patients	caregivers	citizens	insurees	others
ranking	9	3	4	5	0	1	1
rating	8	3	6	4	0	2	1
pair wise comparisons	5	2	1	3	0	1	0
choice based	5	3	3	8	3	2	0
matching	6	1	1	6	2	8	1

Use of preference data in Reimbursement & Pricing

Reimbursement decisions

*Overview (Source: all)**

Rk = ranking method
Rt = rating method
P = pairwise comparison
C = choice-based method
M = matching method

Actual Guidance on the Use of Preference Data
Examples of the use of Preference Data
Expert testimony only on the use of Preference Data

	Patients	Citizens	Decision makers	Experts	Providers	Caregivers
Austria			P	P		
Belgium	Rk	C, M		Rk		
Bulgaria		R, M				
Czech Republic	M	M		Rk, Rt	Rk, Rt	
Denmark	TBD~					
Estonia		?^				
Finland		M				
France	C	M	C			
Germany	P, C					
Hungary	C	Rt, M	Rt			C
Ireland		M				
Italy		M	Rt			
Latvia		?~				
Lithuania		?~				
Netherlands	Rt, P, C, M	C. M	Rt,	P, M	M	M
Norway		M				
Poland		M				
Portugal		M		M		
Slovenia			P			
Spain		M				
Sweden	Rk, Rt, C, M	Rt, m		Rk, Rt, M		
United Kingdom	C	C. M				

* No use of preference data was identified in: Croatia, Cyprus, Greece, Luxembourg, Malta. Romania, Russian Federation, Slovak Republic, Switzerland

~ Piloting the use of preference data. All methods being considered.

^ Guidance is not clear on which methods should be employed

Reimbursement decisions

1. Cost-utility analysis (Source: all)

- The most prevalent use of preference data to support reimbursement and/or pricing decisions
- General population (or ‘citizen’) preferences, TTO or SG methods, estimation of health state utilities.
- 19 countries: Belgium, Bulgaria, Czech Republic, Estonia, Finland, France, Hungary, Ireland, Italy, Latvia, Lithuania, the Netherlands, Norway, Poland, Portugal, Spain, Sweden, and the UK (England and Wales, and Scotland).

Reimbursement decisions

2. Other uses of preference data (Source: Document review)

	Extending the definition of health impact		**Weighting health impact of other factors**
	General population preferences	Patient preferences	
Austria			✓
Belgium			✓
England and Wales	✓		
France			✓
Germany		✓	
Hungary			✓
Italy			✓
The Netherlands	✓		✓
Scotland	✓		
Sweden		✓	

Reimbursement decisions

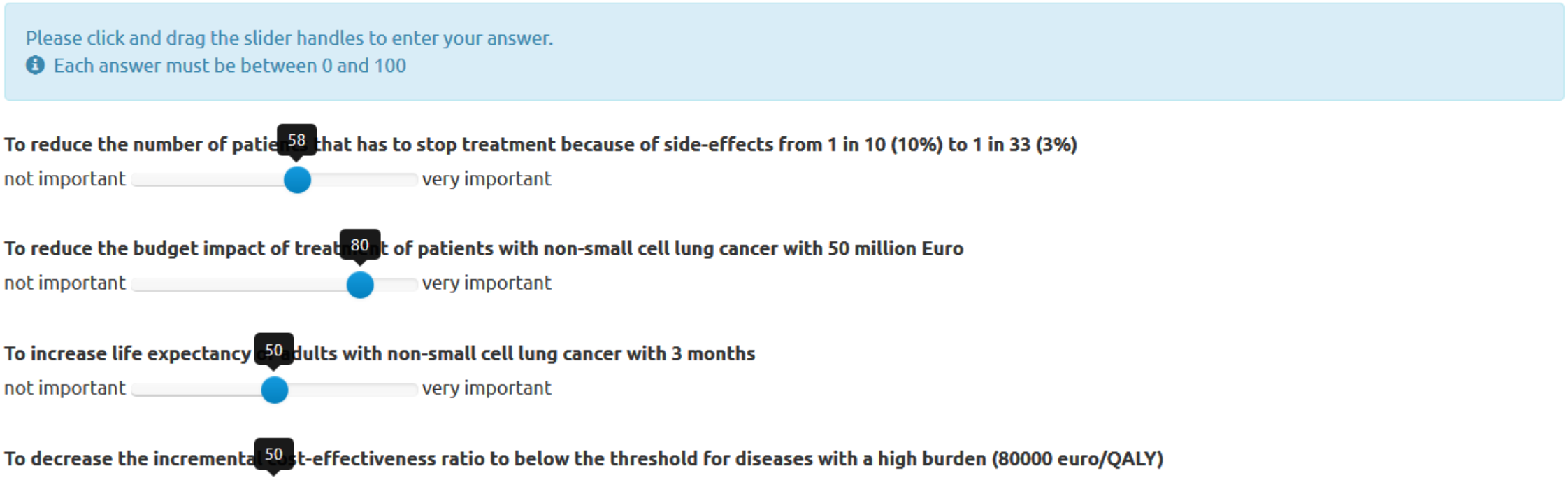
3. Other uses (Source: Survey)

Stakeholder		patients			citizens			decision makers			other		
Country	Method	required	recommended	submitted	required	recommended	submitted	required	recommended	submitted	required	recommended	submitted
Austria	Pairwise								1	1			2
Belgium	Ranking			1									1
	Choice Based						1						1
Czech Republic	Ranking												4
	Rating												4
	Matching		1			1							
Hungary	Choice Based		1									1	
Netherlands	Pairwise			1									1
	Choice Based									1			
	Matching			1									3
	MCDA									1			
Norway	Matching				3		1						
Portugal	Matching					1							1
Sweden	Ranking			2								2	
	Rating			2								2	
	Choice Based			4									
	Matching	2	4				4						4
	Questionaires like Eq-5D.	2			2						2		

Reimbursement decisions

Case study 1: The Netherlands

- Aim: to test the feasibility of MCDA to support decision maker committee in decisions regarding reimbursement
- Method: rating scales; swing weighting
- Preferences elicited from: decision makers
- Status: pilot test, preferences were submitted
- Identified through: survey; expert input; website search



Reimbursement decisions

Case study 2: Belgium

- Aim: to measure the relative importance of health care coverage criteria according to the Belgian general public and policy makers
- Method: discrete choice experiments
- Preferences elicited from: citizens and decision makers
- Status: pilot test, preferences were submitted
- Identified through: literature search; survey

Two patient groups are described below. Both patient groups currently receive treatment. The discomfort associated with the treatment, the quality of life and life expectancy of patients getting this treatment and the typical age of patients with this condition are as follows:

Patients of group 1	Patients of group 2
• have a quality of life of **8 on 10**	• have a quality of life of **5 on 10**
• experience **much** discomfort from treatment	• experience **little** discomfort from treatment
• are between **18 and 64 years of age**	• are **older than 80** years of age
• **no longer** die from the disease	• **no longer** die from the disease

For which patients do you consider it most important to develop a new and better treatment? You may define yourself what you consider to be "better".

Choose one group.

□ Patients of group 1	□ Patients of group 2

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DOI: 10.1177/2381468318799628
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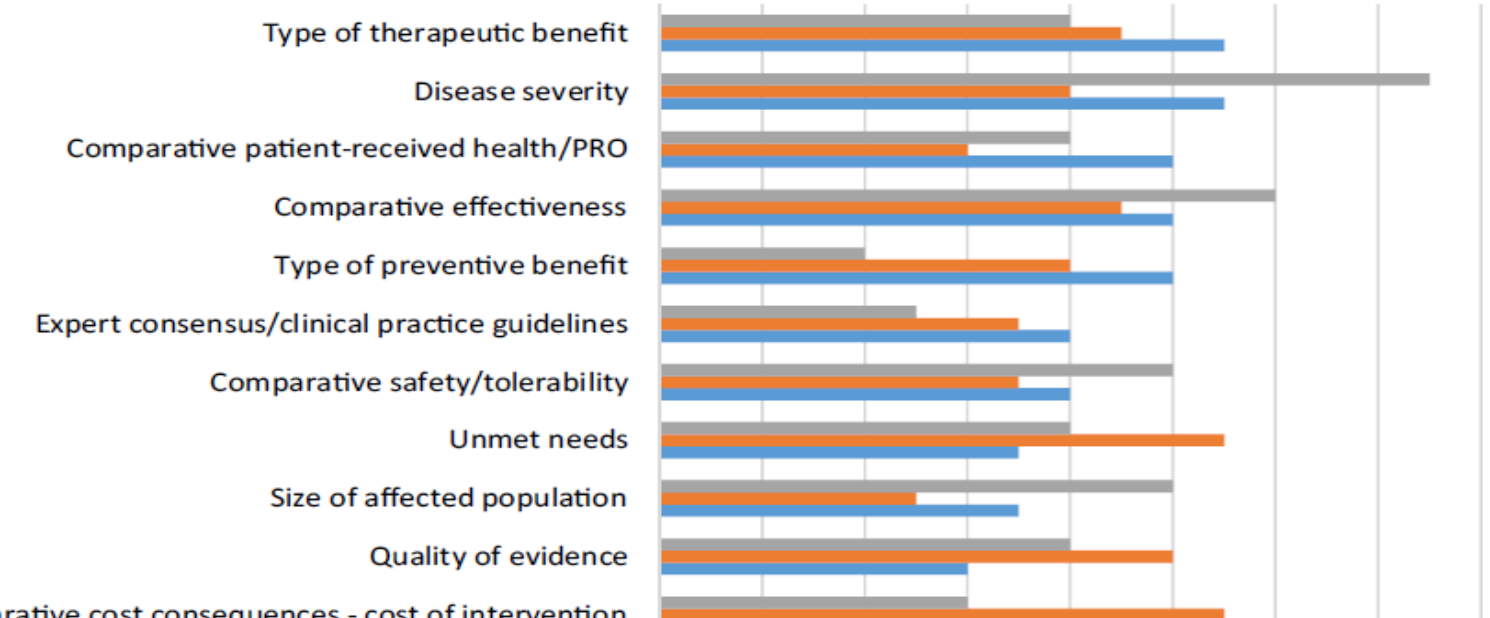
Reimbursement decisions

Case study 3: Italy

- Aim: to obtain preferences and views on decision criteria across three stakeholder groups (patients, clinicians and payers) and to use these to assess the performance of obinutuzumab for rituximab-refractory indolent non-Hodgkin lymphoma (iNHL).
- Method: EVIDEM 3.0 (point allocation)
- Preferences elicited from: patients, clinicians and payers
- Status: pilot test
- Identified through: literature search

Fig. 2 Relative weights of individual criteria by stakeholder group



Reimbursement decisions

Case study 4 and 5: UK and Sweden

- Guidance, SMC and TLV

	Example	Proposal
SMC	Delivery system	DCE or WTP, GP
TLV	Severe, acute pain	TTO or WTP, PP

- Example, NICE (Fabry)
  - Non-health endpoint – the disutility associated with mode of administration
  - DCE, UK general population
  - Utility decrements associated with infusion was estimated at 0.05

- Example, NICE (RA)
  - Unmet need – burden of mode of administration
  - Literature review: DCE, patient
  - Innovation section of submission:

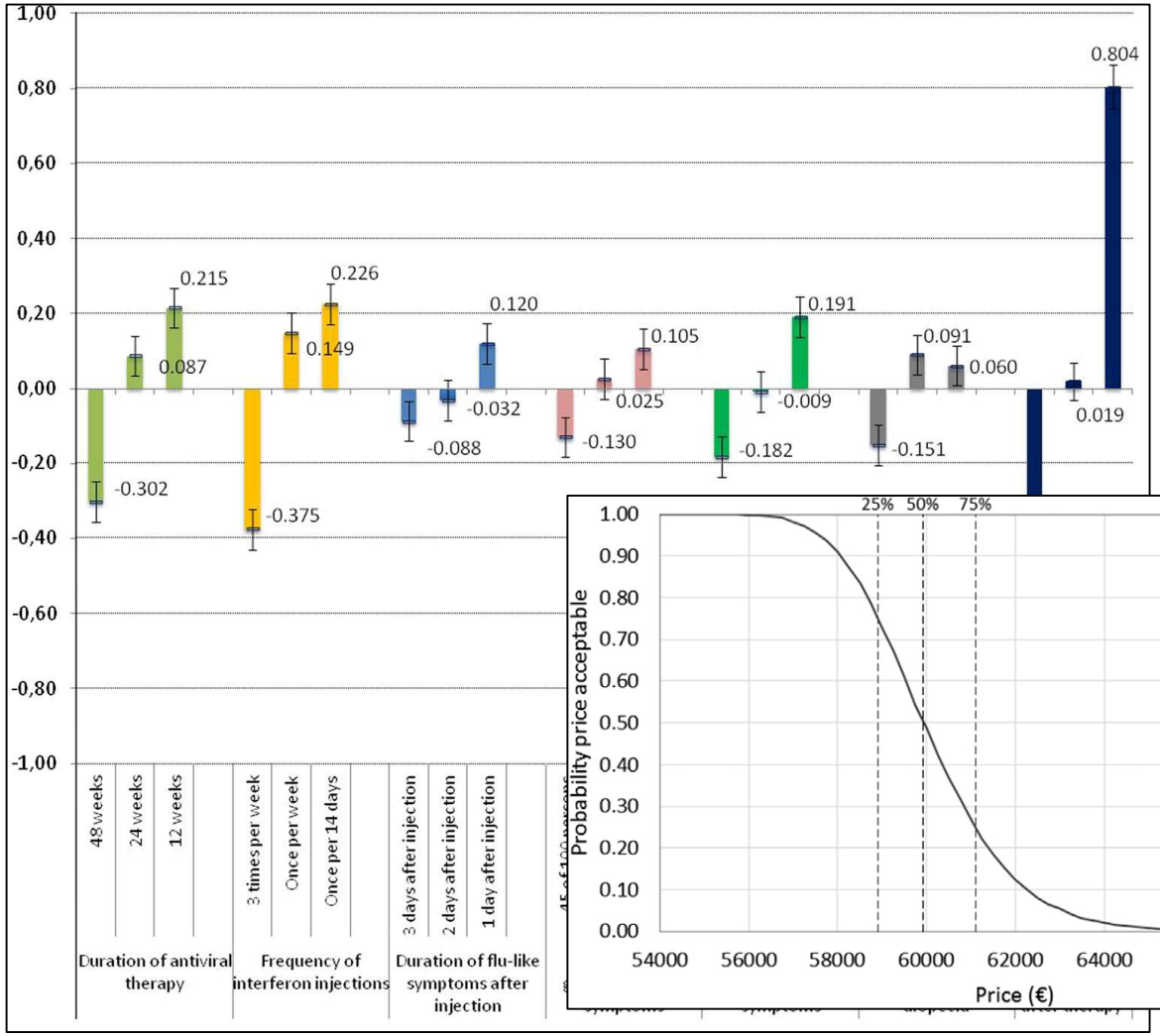
Reimbursement decisions

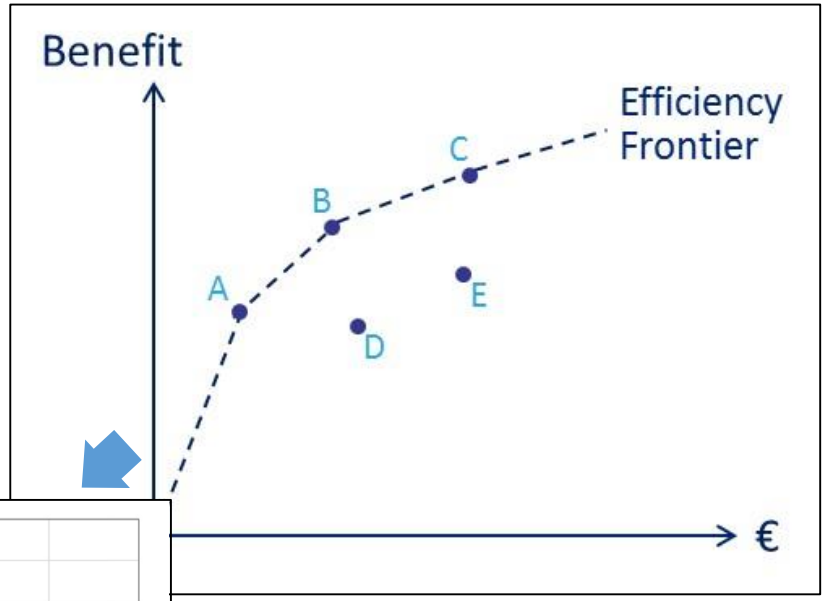
Case study 6: Germany

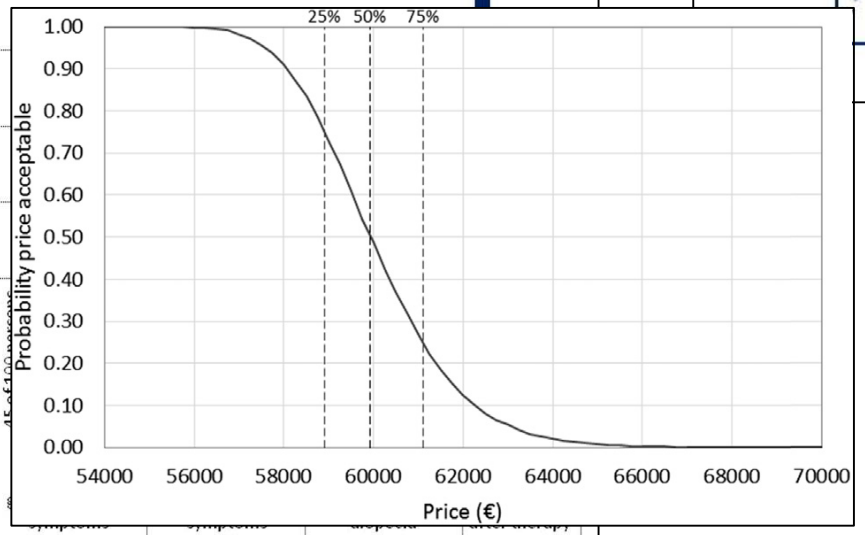
If a measure of overall benefit for the comparison of interventions is to be determined […] *procedures for multi-criteria decision-making or determining preferences can be applied*…the analytic hierarchy process (AHP) and the conjoint analysis (CA)

Reimbursement decisions

Case study 6: Germany







Reimbursement decisions

Future potential: Pilots

	Ranking	Rating	Pairwise	Choice	Matching
Belgium				GP (in MCDA)	
Czech Republic	DM				
Denmark	P				
England and Wales				P	
France				P	
Ireland				P	P
The Netherlands		DM			
Sweden	P	P		P	P

Source: Survey

Source: Document review

P Patients

DM Decision maker

GP General population

3.3

Use of preference data in approval decisions

Approval decisions

EMA (Source: all)

Method	Stakeholder: Patient	Stakeholder: General population	Stakeholder: Decision maker	Stakeholder: Other
Ranking				
Rating				
Pairwise				
Choice-based				
Matching				
Other				

- Guidance on the use of preference data identified
- No guidance identified, but examples of the use of preference data identified
- Neither guidance or examples identified, experts identified use of preference data.
- No use of preference data identified

Decision Context		approval											
Stakeholder		patients			citizens			decision makers			other		
Country	Method	required	recommended	submitted	required	recommended	submitted	required	recommended	submitted	required	recommended	submitted
Belgium	Rating								1				
Bulgaria	ranking							1				2	1
	rating							1				2	1
	Pairwise									1		1	
	Choice Based								1			1	1
	Matching								1			1	
	Multictiteria Decision Analyses								1		1		
Czech Republic	ranking												2
	rating												2
Hungary	Choice Based			1									1
	Matching			1			1						2
Italy	ranking			1			1						2
	rating												
Norway	Matching				1		1						
Portugal	rating												
	Matching					1							
Sweden	Method according to James			1									2
UK	Choice Based			1									1
	Matching			1			1						1

3.4

Country Summaries

Country summaries

Illustration: The Netherlands (reimbursement decisions) (Source: all)

Method	Stakeholder: Patient	Stakeholder: General population	Stakeholder: Decision maker	Stakeholder: Other
Ranking				
Rating	*			
Pairwise				
Choice-based				
Matching				
Other				

- Guidance on the use of preference data identified
- No guidance identified, but examples of the use of preference data identified
- Neither guidance or examples identified, experts identified use of preference data.
- No use of preference data identified

*ZINL guidance recommends DCE or MCDA methods are used to elicit patient preferences for technologies whose value is not well captured by the QALY.

SECTION

4

Conclusion

Conclusion

- In this study several sources have been used since no exhaustive list of activities was available in the published literature and no single source of experts could be used to identify all activities.
- In all stages the applied methods aim to analyze the same phenomena working towards the mapping of the different usage and elicitation of preference data.
- By mixing quantitative and qualitative methods and data, this study gains in breadth and depth of understanding the phenomena of patient and public participation in regulatory decision making, while offsetting the weaknesses inherent to using each approach by itself.

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Poll: Do you know of any other uses of preference data in Europe?

SECTION

5

Acknowledgements

Individual Contributors by Country - 1

- **Austria** - Gottfried Endel
- **Belgium** - Francis Arickx
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- **Spain** – AETS-ISCIII, Health Technology Assessment Agency, and IENS IMIENS-UNED ISCIII, National School of Health, Instituto de Salud Carlos III
- **Sweden** – Dental and Pharmaceutical Benefits Agency (TLV)
- **Switzerland** - Federal Office of Public Health
- **UK** – Healthcare Improvement Scotland and NICE

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