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THE ADDED VALUE OF THE USE OF PATIENT PREFERENCE STUDIES IN POLICY DECISION-MAKING: A LITERATURE REVIEW

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Background

- Health policy decision makers largely draw their conclusions on the benefits and harms of medical interventions based on the different outcomes investigated in clinical trials; however, patients are claiming a greater role in such traditional assessments.
- In the context of regulatory authorities' and payers' increasing interest in patient-focused decisions, eliciting patient preferences (PPs) to inform healthcare decisions is a growing field of research.

OBJECTIVES

The aim of this study was to identify PP studies using qualitative and/or quantitative methods and to investigate to what extent these studies can inform and support healthcare decision-making.

METHODS

- A systematic search was conducted in the PubMed database to identify PP studies conducted in order to inform healthcare decision-making.
- Studies conducted in Europe and the US published from 2014 to 2019 were included.
- PP studies were classified based on their objective:
  - Informing benefit-risk assessment (BRA) decision-making,
  - Informing health technology assessment (HTA) decision-making.
- Key information related to the year, the type of study (quantitative or qualitative), and the disease area were extracted.

Table 1: Methodology of the literature review

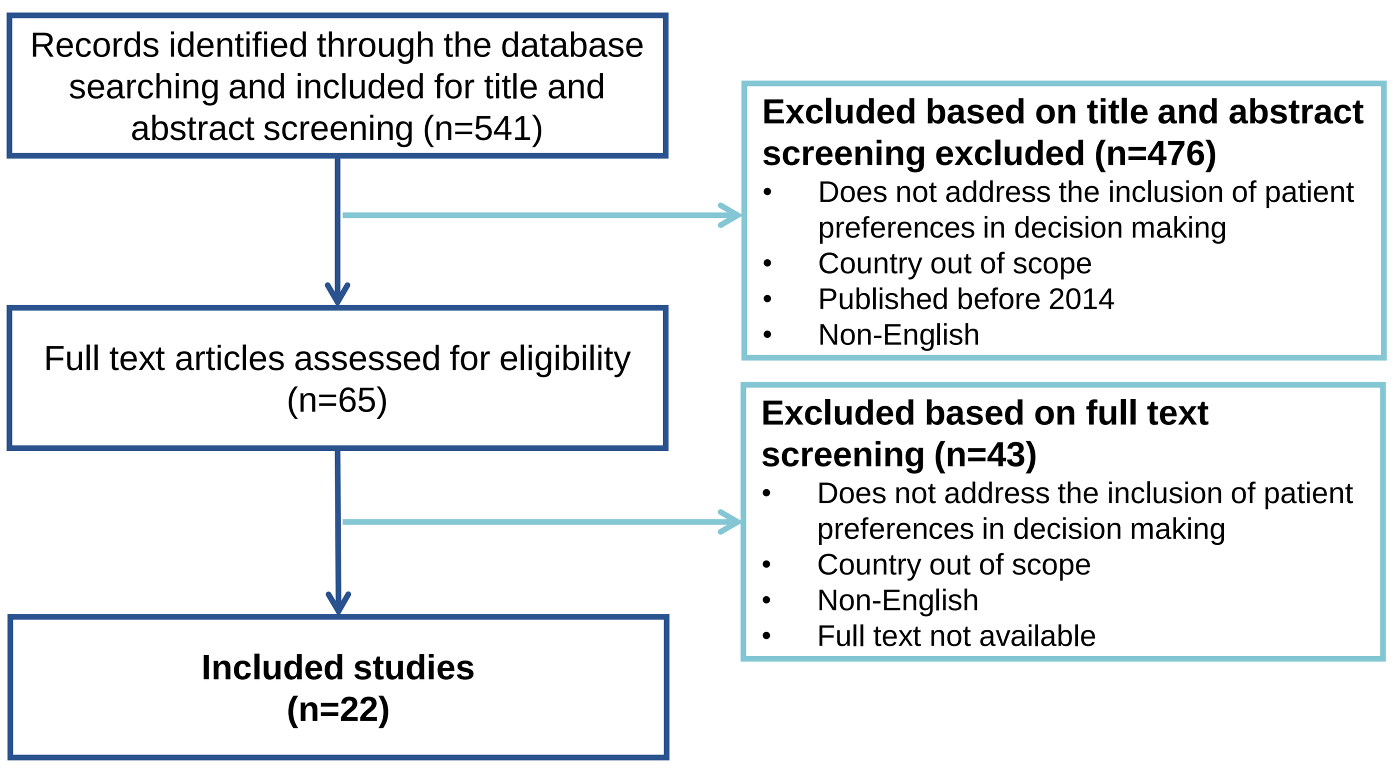
	Inclusion criteria	Exclusion criteria
Database	PubMed	--
Population / Stakeholder	Patient	Policy decision maker, Healthcare professional, Society/General population, Independent academic
Type of studies	Qualitative and Quantitative	None
Technology	Drugs and Devices	Other technologies
Disease area	All disease areas	None
Type of decision	Approval, reimbursement and pricing	All other decisions
Publication date	January 2014 to April 2019	
Location / Country	Europe, US	Other countries
Language	English	

RESULTS

Search Results and Overview of Included Literature

- Out of 541 articles retrieved, 65 were selected for full text screening. Among these, 22 articles met all the inclusion criteria and were included in the final review.

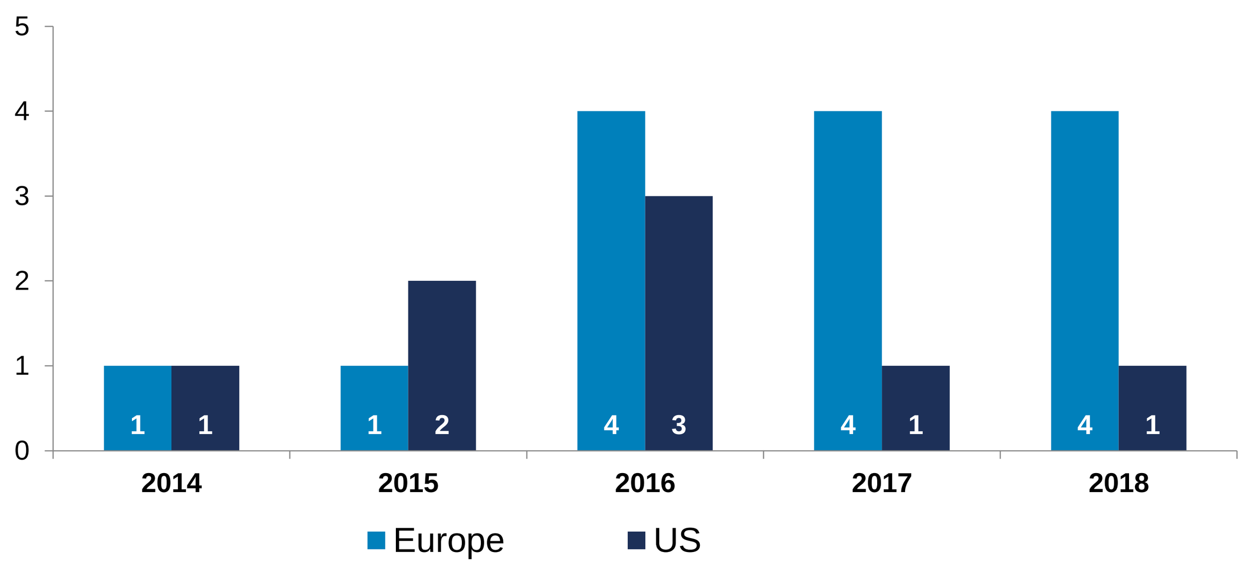
Figure 1. PRISMA flow diagram for study selection



Geographic Scope of PP Studies

- Of the 22 PP studies conducted between 2014 and 2019 identified through the literature review:
  - 14 studies were conducted across Europe (EU level[6], Germany[1,8,13,19], Sweden[17], UK[3,4,7,10], Italy[18], Denmark[5], Netherlands[16]),
  - 8 studies were conducted in the US [2,9,11,12,14,15,20-22].

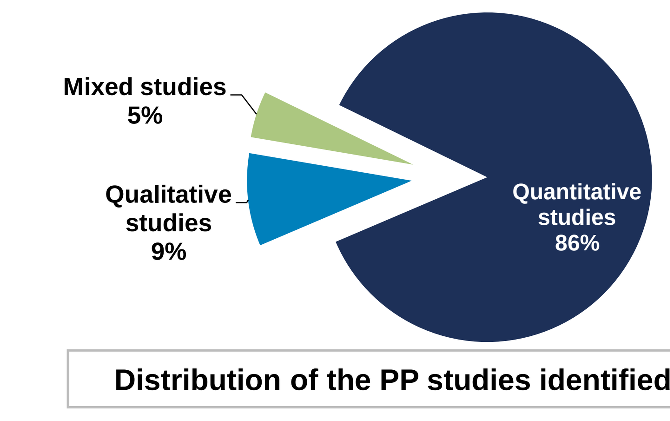
Figure 2. Number of studies identified by region

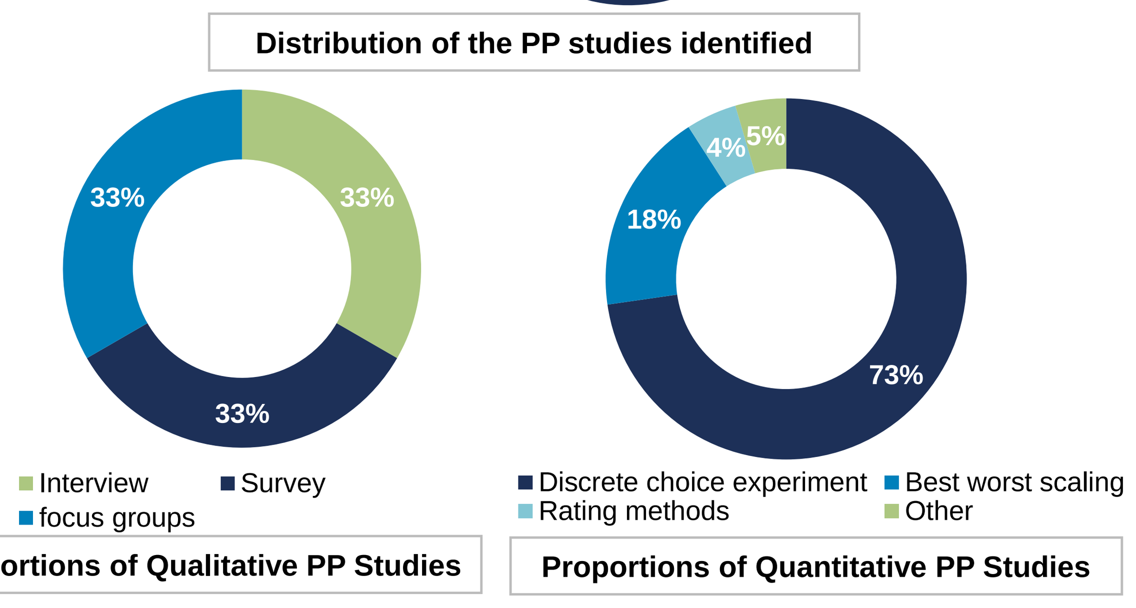


Methods Used for Eliciting PPs

- A high trend toward using quantitative tools to elicit PPs was identified:
  - 86% of the studies used quantitative methods, mainly discrete choice experiments[1-16] (n=16) and at a lower rate best-worst scaling[9,12,22] (n=4) or rating methods[19] (n=1);
  - Qualitative methods were less frequently used (9%), such as surveys[18], interviews[17], and focus groups[17] ;
  - One study (5%) used both qualitative and quantitative tools[21].

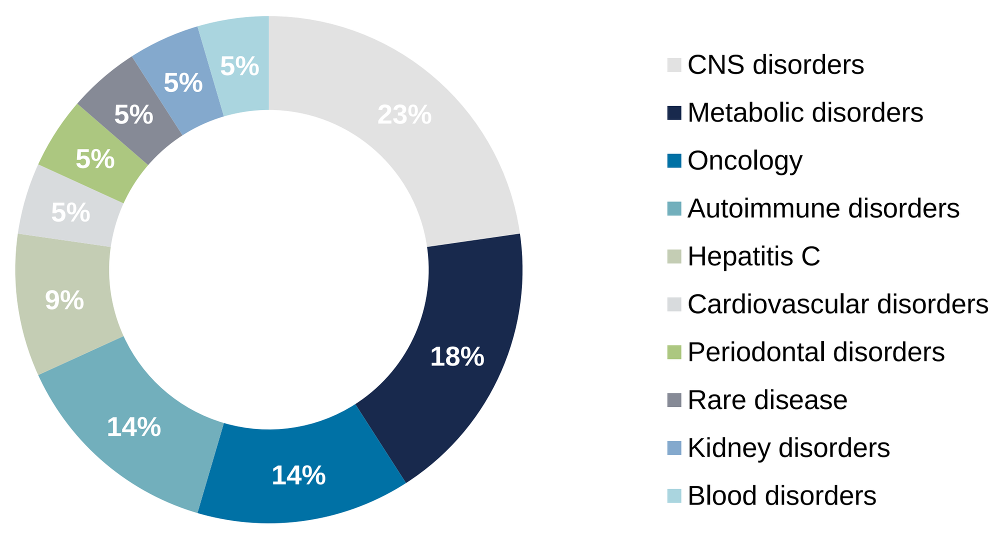
Figure 2: Type of PP studies identified in the literature review





- PP information has been elicited across various disease areas:
  - Central nervous system disorders, 23%
  - Metabolic disorders such as diabetes and obesity, 18%
  - Oncology and autoimmune disorders 14% each
  - Hepatitis C, 9%
  - Rare diseases, cardiovascular diseases, periodontal disorders, kidney and blood disorders, 5% each

Figure 3: Disease areas identified in the literature review



Purpose of Collecting PPs

- PP studies identified in the literature review had 2 main objectives:
  - 15 studies (68%) aimed at informing BRA,
  - 6 studies (27%) aimed at informing HTA,
  - 1 study (5%) aimed at informing both decisions[7].

Table 2: Objectives of PP studies identified in the literature review

Type of decision	Benefit-Risk Assessment	Health Technology Assessment
Number of studies (%)	16 (73%)	7 (32%)
Purpose of the study	• Patients' benefit-risk tradeoffs • Outcome prioritization • Value appreciation of treatment characteristics	• Patients' benefit-risk tradeoffs • Valuing patient willingness-to-pay
References	2-6,9-12,15-17,20-21	1,8,13,14,18,19

CONCLUSIONS

Currently, PP studies are not required to be included in BRAs and HTAs. There is no consensus on the role and methodology for the inclusion of PP studies for either types of assessments. Nevertheless, PP studies have the potential to become an important tool and play an important role in systematically collecting patients' priorities to better inform healthcare decision-making.

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