Use of Patient Preference Studies in NICE Submissions in the Last 5 years

HTA128

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Introduction and objective

- There is growing interest in patient preference (PP) data from payers and Health Technology Assessment (HTA) bodies.
- PP studies can be a robust way of getting patient inputs as compared to patient's direct involvement in HTA discussions, which is seen as subjective[1].
- HTA bodies want PP studies to investigate attributes related to benefit-risk, as well as aspects of treatment process, such as dose frequency and route of administration[2].
- National Institute for Health and Care Excellence (NICE) in the UK considers PP studies complementary to the clinical evidence package, considering that it brings value to the assessment where treatment options are being compared[3].
- This analysis aimed to identify use of PP studies submitted to NICE in the last 5 years and understand the methodologies (qualitative vs quantitative) used to elicit patient preferences.

Methodology

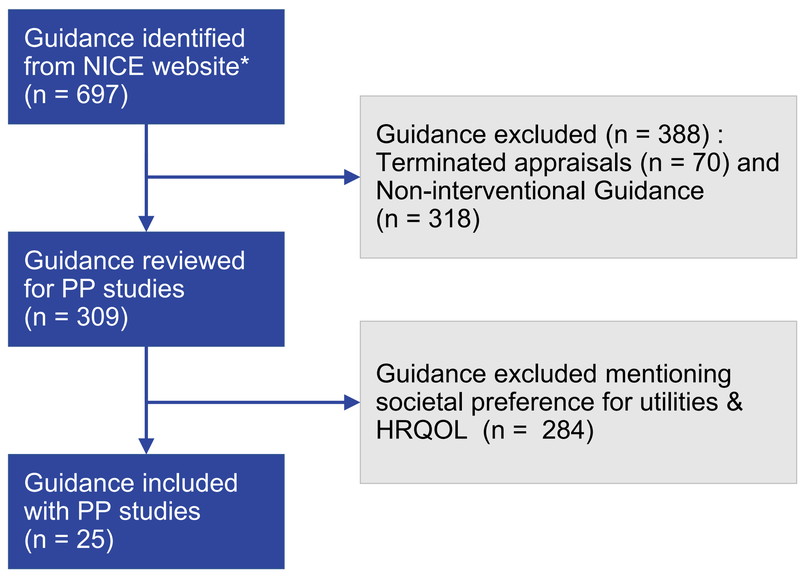
- Guidance published on the NICE website for the period between May 2018 to May 2023 were screened to determine the inclusion of PP studies.

Further analysis was conducted to determine how PP was included in the submission to gather PP insights.

Results

- The search performed on NICE website identified a total of 697 appraisals published from May 2018 till May 2023.
- A total of 388 guidance were eliminated from the analysis because 70 were terminated and others did not meet the inclusion criteria of interventional assessments.
- Subsequently, 309 guidance were screened for PP studies. After removing those mentioning the societal preference for utilities and Health Related Quality of Life (HRQoL), 25 were finally identified (see **Figure 1**).

Figure 1: Flow diagram depicting the identification, selection, and exclusion of records for subsequent analysis



*Published from May 2018 till May 2023

- Among the 25 guidance identified, the majority were focused on non-oncology indications (see **Figure 2**) and mostly on autoimmune conditions such as ulcerative colitis and various types of arthritis. In oncology, the most frequent therapy area was multiple myeloma (see **Figure 3**).

Figure 2: Identified Guidance with PP included

- Most of the submissions (n=16) used qualitative method i.e. literature review and qualitative survey to report PP. Discrete Choice Experiment (DCE) was the most common quantitative methodology used in six guidance including indications like migraine, ulcerative colitis, growth disturbance, prostate cancer, and multiple sclerosis.
- Submitted DCE studies identified importance of treatment attributes and trade-offs between attributes (in most of the studies <6 attributes were tested).

Conclusions

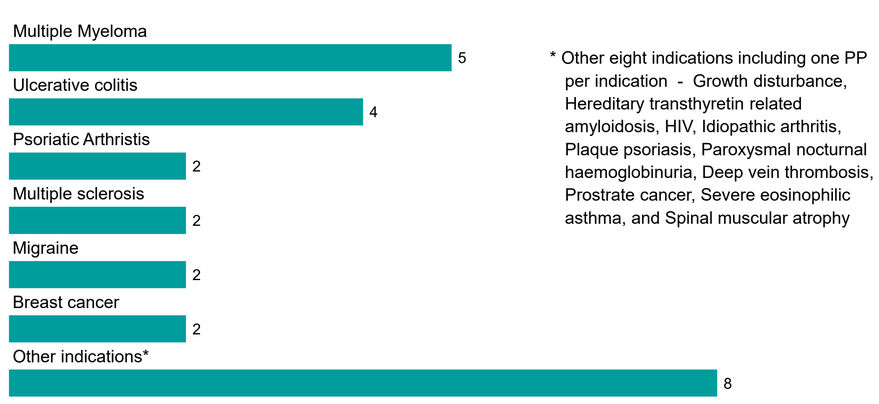
- The insights derived from PP studies captures a wider and more diverse patient population's preferences making them more reliable and robust, and often having a greater impact compared to the more limited scope of direct patient involvement in HTAs.
- DCE is the most commonly used quantitative methodology submitted to NICE, eliciting PP for non-clinical outcome such as dose frequency and route of administration. Whereas qualitative methodologies often utilize literature reviews as a valuable option, as demonstrated in the guidance for Multiple Myeloma which typically use a well-designed and widely recognized UK PP study in this indication.
- A well-designed PP study can provide valuable information to inform HTA reviews of new therapeutic options and support decision making. However, there is a potential for further standardization and integration of PP data in HTAs.
- Further research can enhance our understanding of the practical implementation and impact of patient preferences in HTA evaluations by exploring the actual influence of patient preference studies on HTA assessments and decision-making processes.

Results continued…

- PP in prostate cancer studied patients' trade-off between different end-points and adverse events related with treatment. However, PP submitted in autoimmune diseases (Idiopathic arthritis, Ulcerative colitis, Plaque psoriasis) evaluated the treatments preference with convenient methods of administration (Oral vs SC vs IV).
  In these chronic indications, treatment is highly individualised, dependent on both patient preference and clinician judgement.

Figure 3: PP therapy area wise

Guidance with PP per therapy area



* Other eight indications including one PP per indication - Growth disturbance, Hereditary transthyretin related amyloidosis, HIV, Idiopathic arthritis, Plaque psoriasis, Paroxysmal nocturnal haemoglobinuria, Deep vein thrombosis, Prostrate cancer, Severe eosinophilic asthma, and Spinal muscular atrophy

- Mode and frequency of administration were important attributes preferred by patients in therapies like Migraine and Growth disturbance.
- All guidance identified with PP data were single technology assessments (STA) and only three were Fast technology assessment (FTA).
- Our research findings are in line with previously published poster[4] where no evidence of the use of quantitative methodology submitted to value dossiers until 2020 in oncology was reported. In addition, we found that DCE was the most commonly used quantitative methodology in non-oncology to elicit PP.

Limitations

- The analysis conducted over a 5-year period, along with the influence of the COVID-19 pandemic, has the potential to introduce bias by affecting the types and number of HTA dossiers submitted, leading to limited diversity and potentially influencing representation in certain therapeutic areas.
- Considering that guidelines from regulatory authorities and ISPOR have been published relatively recently, there is still ongoing development and growth in the field of patient preference studies. This may influence the frequency of these studies being used in HTA guidance and thus the number of NICE guidance identified in the present analysis

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