Reporting Patient Preference Studies in Health Technology Assessment Submissions: Does it Make a Difference?

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HTA132

Objective

To investigate the reporting of patient preference studies (PPS) in non-oncology NICE technology appraisals and the extent to which the inclusion of PPS followed through into decision-making by the Committee.

Background

- Interest in patient preferences (PP) is growing as stakeholders, such as payers and patient organisations, increasingly seek to integrate the patient voice in reimbursement processes.
- PPS evaluate the relative desirability or acceptability to patients of specific outcomes or attributes of health interventions which differ between alternative treatment options.
- In 2020, the National Institute for Health and Care Excellence (NICE) published their perspective on the use of PPS in Health Technology Assessment (HTA) decision-making, highlighting the potential value of considering PPS alongside cost-per-quality-adjusted life year health economic frameworks.[1]

Methods

- Non-oncology NICE technology appraisals (TAs) with guidance published between January 2020 and May 2023 were identified from the NICE website on 11th May 2023 (n=88), with the post-2020 timeframe corresponding with the publishing of the NICE perspective on PPS.[1] Submissions citing PPS to support intervention attribute(s) (n=19) were included for subsequent further review via targeted searches of the available documentation published by NICE.
- During targeted review, relevant information from each included appraisal was extracted into a pre-formatted extraction grid. Details extracted included:
  - intervention and indication
  - reported PPS type
  - intervention attribute(s) supported by PPS
  - presence of a standalone PP data section
  - acknowledgement of PP-evidenced attribute(s) in final appraisal documents (FADs).

Results

- Of the 88 non-oncology TAs identified, 19 (21.6%) cited PPS to support intervention attribute(s) (**Figure 1**).
- From 2021 to 2022, the proportion of submissions citing PPS nearly doubled (15.4% versus 27.8%, respectively), and the proportion including a standalone PP section more than tripled (2.5% versus 8.3%, respectively).
- Notably, while the External Assessment Group (EAG) positively acknowledged the presented PP-evidenced attribute(s) in only 26.3% (5/19) of these TAs, a substantially larger proportion of PP-evidenced attribute(s) in the TAs were subsequently acknowledged by the Committee in FADs (15/19, 78.9%).
- Of the five submissions that presented dedicated PP-related sections, the Committee acknowledged the PP-evidenced attribute(s) in standalone PP sections in two FADs (TA807 and TA757).
- The 19 TAs collectively cited a total of 39 PPS, although the study type was not reported for a substantial proportion (35.9%) of these. Of the cited PPS where the study type was reported, discrete choice experiment was most common (25.6% ◖) (**Table 1**).
- Key intervention attributes supported by PPS were administration route (53.8% ◆) and dosing frequency (33.3% ●), as well as treatment setting and onset speed.
- Only two TAs (TA807 and TA757) incorporated into their cost-utility analysis (CUA) a utility decrement or advantage associated with an intervention attribute, informed by the results of the PPS;
  - TA757 evaluated long-acting cabotegravir with long-acting rilpivirine (CAB LA + RPV LA) versus daily oral antiretroviral therapies (ARTs) for treating human immunodeficiency virus (HIV)-1. PPS describing preference for CAB LA + RPV LA over daily oral ARTs were incorporated in the Company submission. These PP findings were accounted for in the CUA, and their acknowledgement by the EAG in their report and by the Committee in the FAD suggests a contribution to the positive recommendation made by the Committee. Key components of the evaluation process and outcomes for TA757 are presented in **Figure 2**.
- TA807 evaluated oral roxadustat for the treatment of symptomatic anaemia in chronic kidney disease versus injectable erythropoietin stimulating agents and captured PPS findings in the model by including a utility gain associated with oral pill administration versus subcutaneous injection. The benefits of an oral alternative were subsequently acknowledged by the Committee in the FAD, indicating that the presented PPS findings were considered in the Committee decision-making.

Conclusions

Whilst PPS were infrequently and often poorly reported in TAs, their inclusion in the company submission led to Committee consideration of the intervention attributes supported by the PPS in the majority of FADs.

However, clearer guidance on the integration and reporting requirements for PPS data in TAs may be valuable in order to further establish the role of PPS in informing Committee decision-making.

FIGURE 1

Summary of post-2020 NICE non-oncology TAs

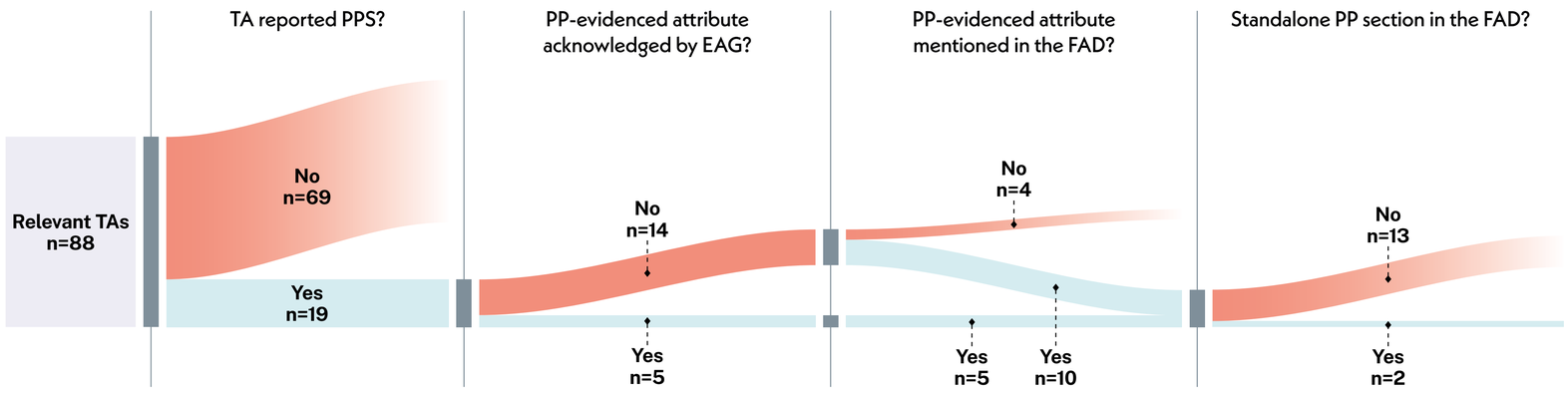


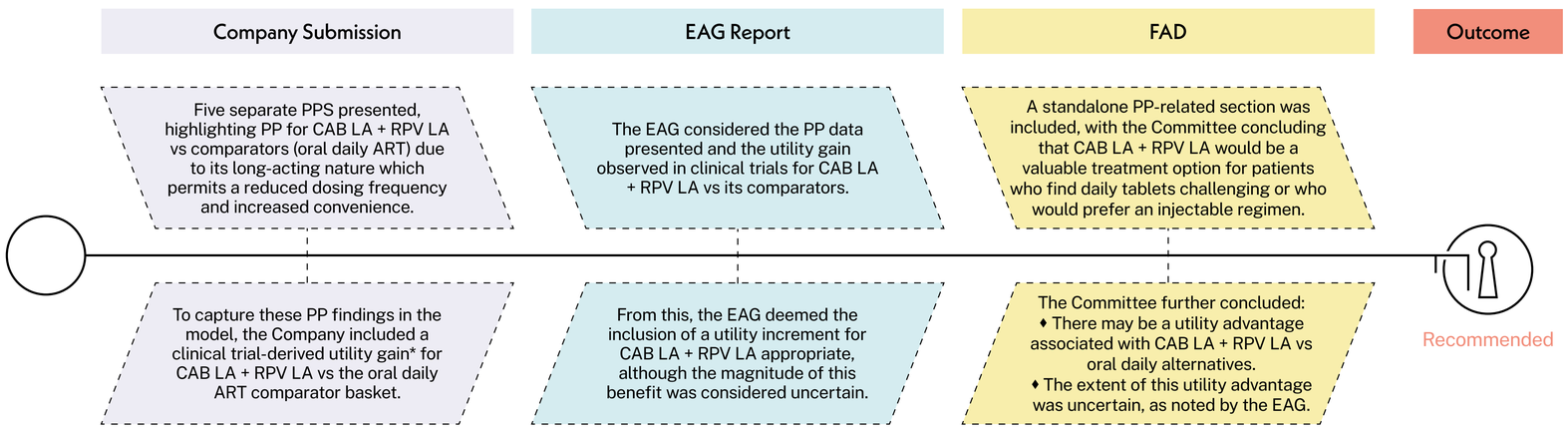
TABLE 1

Summary of extracted TAs reporting PPS

TA	Indication	Intervention	Intervention attribute(s) supported by cited PPS	Type of PPS
TA871	Migraine	Eptinezumab	● Adminstration frequency	NR
			Treatment setting	◖ Discrete choice experiment
			Onset of action	◖ Discrete choice experiment
TA861	Active non-radiographic axial spondyloarthritis	Upadacitinib	◆ Administration route	Survey
TA863	Growth disturbance	Somatrogon	● Adminstration frequency	◖ Discrete choice experiment
			● Adminstration frequency	◖ Discrete choice experiment
TA853	Primary chronic immune thrombocytopenia	Avatrombopag	◆ Administration route and dietary restrictions	NR
TA828	Moderately to severely active ulcerative colitis	Ozanimod	Mechanism of action	Survey
TA829	Active ankylosing spondylitis	Upadacitinib	◆ Administration route	NR
			◆ Administration route and setting	NR
TA820	Diabetic macular oedema	Brolucizumab	● Adminstration frequency	Survey
TA807	Symptomatic anaemia in chronic kidney disease	Roxadustat	◆ Administration route	◖ Discrete choice experiment
TA799	Diabetic macular oedema	Faricimab	● Adminstration frequency	Survey
TA792	Moderately to severely active ulcerative colitis	Filgotinib	◆ Administration route	◖ Discrete choice experiment
TA767	Relapsing–remitting multiple sclerosis	Ponesimod	Symptom improvement	◖ Discrete choice experiment
			◆ Administration route	NR
			◆ Administration route	Conjoint analysis
			◆ Administration route	Conjoint analysis
TA768	Active psoriatic arthritis after inadequate response to DMARDs	Upadacitinib	◆ Administration route	Conjoint analysis
			◆ Administration route	NR
TA757	HIV-1	Cabotegravir with rilpivirine	◆ ● Adminstration route and frequency	NR
			● Administration frequency	NR
			● Administration frequency and convenience	NR
			● Administration frequency and convenience	Questionnaire
			● Administration frequency	Survey
TA755	Spinal muscular atrophy	Risdiplam	Avoidance of disease progression	◖ Discrete choice experiment
			◆ Administration route	NR
			◆ Administration route	◖ Discrete choice experiment
			◆ Administration route	Survey
TA744	Moderate rheumatoid arthritis	Upadacitinib	◆ Administration route	NR
			◆ Administration route	◖ Discrete choice experiment
			◆ Administration route	Conjoint analysis
TA738	Hereditary angioedema	Berotralstat	◆ Administration route	NR
			◆ Administration route	NR
TA708	Eosinophilic oesophagitis	Budesonide	Tolerability of formulation	NR
TA698	Paroxysmal nocturnal haemoglobinuria	Ravulizumab	● Administration frequency	Survey
			● Administration frequency	Interviews
			● Administration frequency	Questionnaire
TA676	Moderate to severe rheumatoid arthritis	Filgotinib	◆ Administration route	Survey

FIGURE 2

Case study: TA757



*The utility gain was derived from a *post hoc* analysis of SF-6D utility data from pooled clinical trial data (ATLAS; FLAIR).

Abbreviations: ART: antiretroviral therapy; **CAB LA + RPV LA:** long-acting cabotegravir with long-acting rilpivirine; **DMARDs:** disease-modifying antirheumatic drugs; **EAG:** External Assessment Group; **FAD:** final appraisal document; **HIV:** human immunodeficiency virus; **NICE:** National Institute for Health and Care Excellence; **NR:** not reported; **PP:** patient preference; **PPS:** patient preference study; **QALY:** quality-adjusted life year; **SF-6D:** Short Form 6 Dimension; **TA:** technology appraisal.

References: [1]Bouvy JC *et al.* Patient. 2020;13(2):145–149. **Acknowledgements:** The authors thank Amie Ennew and Brylle Vistal, Costello Medical, for graphic design assistance. We also thank Alex Porteous, Costello Medical, for their review and editorial assistance in the preparation of this poster.

Presented at ISPOR Europe 2023 | Copenhagen, Denmark | 12–15 November 2023

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