No Strong Preference? Acceptance of the Quantitative Incorporation of Patient Preference in Economic Modelling in Past NICE Technology Appraisals

Prockter A,[1] Morgan J,[2] Sowman A[3]

[1]Costello Medical, Cambridge, UK; [2]Costello Medical, London, UK; [3]Costello Medical, Edinburgh, UK

12th November 2025 | ISPOR Podium Presentation | P63

PRESENTED BY
ANEKA SOWMAN

costello medical

Background and Objectives

Background (1/2)

- Capturing patient preferences in reimbursement decisions provides insights into what matters to patients, and helps to ensure that healthcare decisions are aligned with patient values and needs[1,2]
- Stakeholders such as payers and patient organisations are increasingly seeking to integrate the patient voice into reimbursement decision making
- Previous research investigated the incorporation of patient preference evidence in NICE technology appraisals (TAs)[3]

Parke *et al.* 2023

Aimed to investigate:

- Reporting of patient preference evidence in NICE TAs
- **Extent to which** this impacted **decision-making** by the Committee

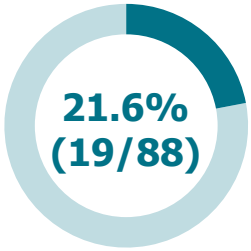
Abbreviations: NICE: National Institute for Health and Care Excellence; **TA:** technology appraisal.

References: [1]Cachoua et al. Front. Med. 2020 Oct 26;7:543046; [2]Bouvy et al. Patient. 2020 Apr;13(2):145–149; [3]Parke E. et al. Presented at ISPOR Europe, 12–15 November 2023. Copenhagen, Denmark. HTA132.

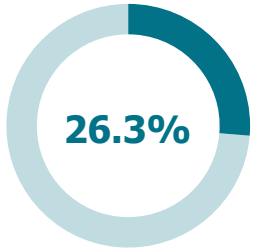
Background (2/2)

Parke *et al.* 2023[1]

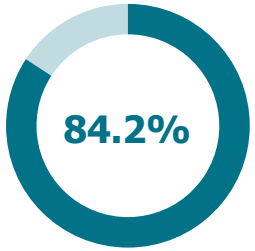
Results



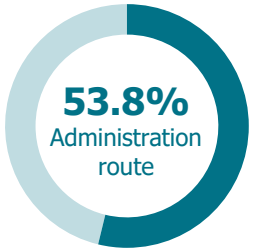
Patient preference evidence was reported in only **21.6% (19/88)** of NICE non-oncology TAs published between 2020–2023

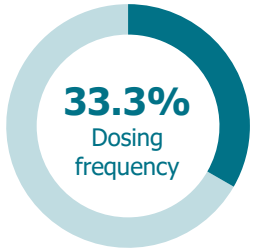


EAGs positively acknowledged patient preference in **26.3%** of these TAs (5/19)



Committees acknowledged patient-preferred intervention attributes in most final appraisal documents (FADs) (16/19, **84.2%**)





The most commonly mentioned patient preference-evidenced attributes in the 19 TAs were administration route (**53.8%**) and dosing frequency (**33.3%**)

Conclusion

- Patient preference evidence was **infrequently** and **poorly reported in TAs**
- In TAs that did **include intervention attributes** supported by **patient preference studies**, these considerations were factored into **the majority of FADs**

Abbreviations: EAG: External Assessment Group; **FAD:** final appraisal document; **NICE:** National Institute for Health and Care Excellence; **TA:** technology appraisal.

References: [1]Parke E. et al. Presented at ISPOR Europe, 12–15 November 2023. Copenhagen, Denmark. HTA132.

Objectives

- Previous research by Parke *et al.* found that the inclusion of patient preference evidence led to **consideration** by Committees in FADs, with Committees broadly **acknowledging** the inclusion of patient preference studies in Company submissions
- However, the **extent** to which this **qualitative** perspective was taken into account in the decision making remained unclear, with few TAs incorporating patient preference **quantitively** in their modelling[1]
- Interest in **quantitative methods** to incorporate patient preference into the HTA process is growing, including the level of acceptance of such approaches by EAGs and Committees

Therefore, this research investigated:

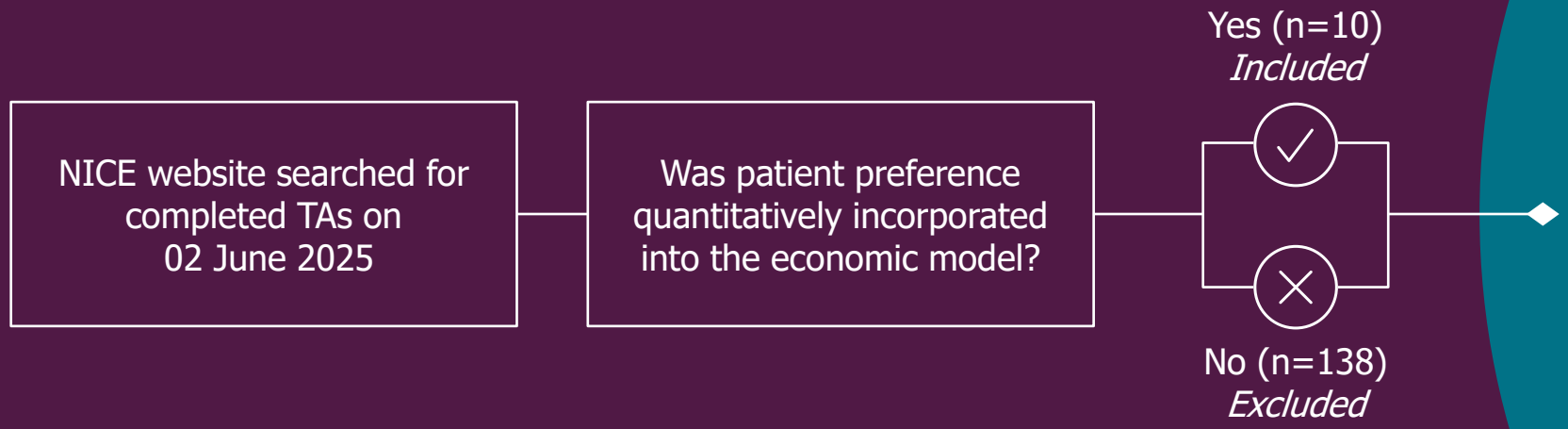
- The methods of quantitative incorporation of patient preference in economic modelling in prior NICE appraisals
- The extent to which these were accepted by the Committee/assessors

Abbreviations: EAG: External Assessment Group; **FAD:** final appraisal document; **HTA:** Health Technology Assessment; **NICE:** National Institute for Health and Care Excellence; **TA:** technology appraisal.

References: [1]Parke E. et al. Presented at ISPOR Europe, 12–15 November 2023. Copenhagen, Denmark. HTA132.

Methods

Methodology



TAs were search until 10 relevant TAs were identified, resulting in a total of N=148 TAs being reviewed, spanning October 2023 to June 2025

Relevant information from each appraisal was extracted. Details included:

- Treatment and disease area
- Resulting decision from NICE
- Type of patient preference included in the economic model
- Source of patient preference
- Justification for inclusion of patient preference in the economic model
- EAG/Committee comments, critiques and conclusions on the approach, including overall acceptance

Abbreviations: EAG: external assessment group; **NICE:** National Institute for Health and Care Excellence; **TA:** technology appraisal.

Results

Summary of Extracted TAs

- All 10 TAs incorporated a utility increment or decrement based on administration route, all favouring oral administration

TA	Specific disease area	Treatment	Source of patient preference	Method of preference elicitation	Base case or scenario analysis?	Was utility adjustment a key model driver?	Acceptance of utility adjustment
TA1055	Epithelial ovarian, fallopian tube, or primary peritoneal cancer	Rucaparib	Vignette study	TTO	Base case	N	Accepted
TA1035	Anaemia associated with chronic kidney disease	Vadadustat	Published cost-effectiveness study identified in economic SLR	DCE	Base case	N	Accepted
TA1012	Systemic mastocytosis with or without associated hematologic neoplasm, mast cell leukaemia	Avapritinib	Vignette study	TTO	Base case	N	Accepted
TA1010	Paroxysmal nocturnal hemoglobinuria	Danicopan	UK general population and Global population preferences surveys	DCE	Base case	N	Accepted
TA1003	Thymic dysplasia	Exagamglogene	Vignette study	TTO	Base case	Y	Rejected
TA1000	Paroxysmal nocturnal hemoglobinuria	Iptacopan	Previous appraisals (TA698 and TA778)	DCE	Scenario	N	Accepted with critique
TA977	Glioma	Dabrafenib and trametinib	Interviews from the UK population	TTO	Base case	N[a]	Rejected
TA973	Migraine	Atogepant	Vignette study	TTO	Base case	N	Rejected
TA949	Graft-versus-host disease	Belumosudil	Vignette study	TTO	Base case	N	Accepted with critique
TA924	Type 2 diabetes	Tirzepatide	Economic report for NG28 and published literature	TTO	Base case	N	Accepted with critique

Footnotes: [a] In TA977, the impact on cost-effectiveness results of an alternative scenario was noted to be modest.
Abbreviations: DCE: discrete choice experiment; **NICE:** National Institute for Health and Care Excellence; **SLR:** systematic literature review; **TA:** technology appraisal; **TTO:** time-trade-off; **UK:** United Kingdom.

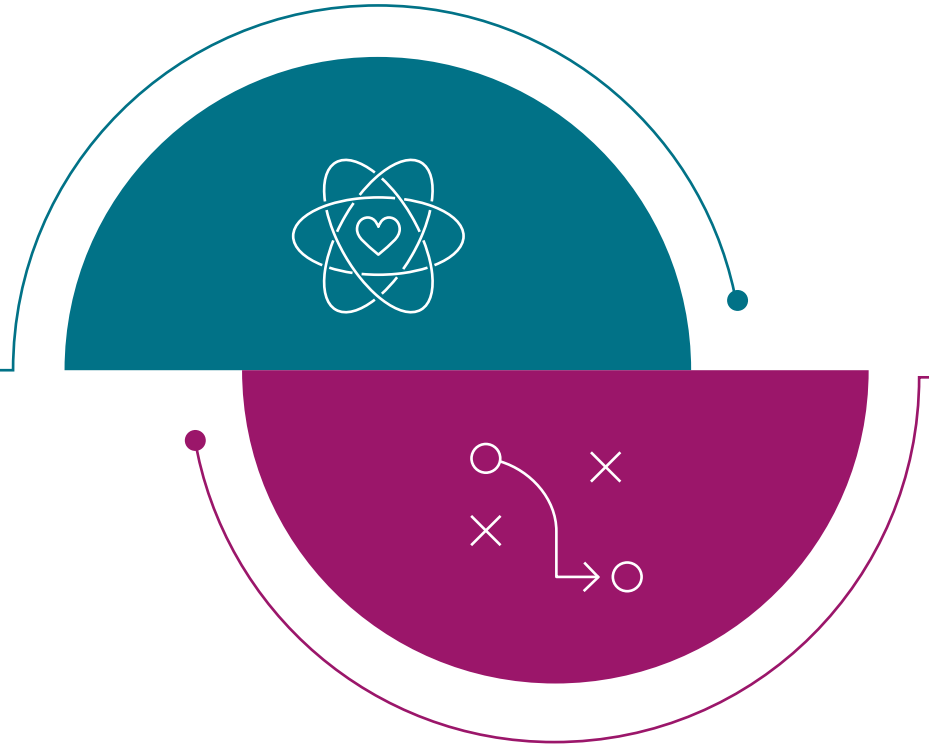
Sources of Patient Preference

Study type and elicitation method

Patient preference for mode of administration was sourced from **vignette studies** in **five** TAs and **other published sources** in **five** TAs

Seven of the appraisals cited studies that utilised a **time-trade-off (TTO)** approach to elicit preferences[a]

In all instances, utility was calculated based on the point of indifference between x years in full health and y years in the health state being evaluated, calculated as utility = x/y



Three of the appraisals cited studies that utilised **discrete choice experiments (DCE)**

The methods used to derive utility adjustments were unclear in all studies utilising DCE

Footnotes: [a] One appraisal (TA924) utilised two patient preference studies, both utilising TTO.
Abbreviations: DCE: discrete choice experiment; **TA:** technology appraisal; **TTO:** time trade-off.

Sources of Patient Preference

Population details

5/10

TAs utilised studies eliciting patient preference from only **general population** participants

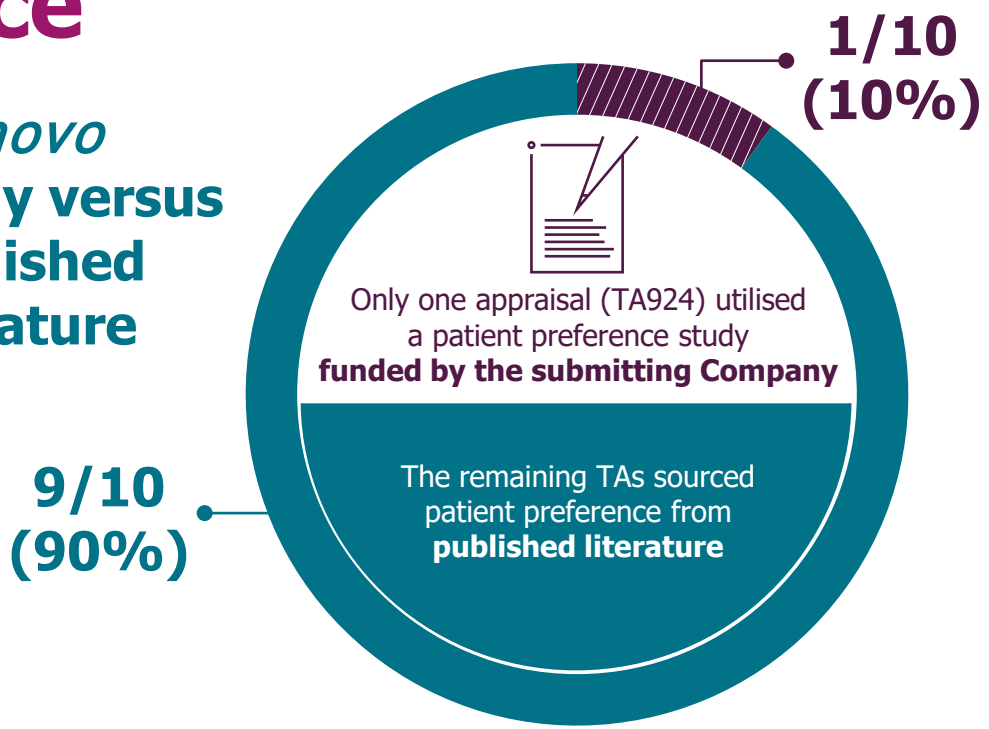
3/10

TAs utilised studies eliciting patient preference from **patients with the disease**[a]

2/10

TAs utilised studies with **mixed populations** (including some patients with the disease and general population participants)[a]

De novo study versus published literature



Key takeaways:

- Both **disease-specific** and **general population** sources have been accepted by the Committee/assessors
- In the most recent TAs incorporating patient preference into economic modelling, Companies most commonly opted to utilise **available published studies** as opposed to carrying out *de novo* studies

Footnotes: [a] One appraisal (TA924) utilised two patient preference studies, one included patients with the disease and one including a mixed population of patients (including some patients with the disease and some general population participants)
Abbreviations: TA: technology appraisal.

Methods of Application Across Economic Models

6/10 TAs incorporated a **utility decrement** for injectable treatments

2/10 TAs incorporated a **utility increment** for oral administration

2/10 TAs incorporated a one-off **QALY loss** associated with intravenous (IV) treatment

3/10 TAs incorporated patient preference as an adjustment to the **health state utility value**

3/10 TAs incorporated a **per-cycle** utility increment or decrement for patients on treatment

2/10 TAs incorporated an **annual disutility adjustment** for patients on treatment

TA	Description of adjustment	Method of adjustment	Utility adjustment value
TA1055	**Utility decrement** applied to IV administration for PF health state	Applied to PF **health-state utility value**	−0.02 (IV)
TA1003	**Utility decrement** applied for SC injection	Applied to **health-state utility value**	−0.1 (SC)
TA924	• **Utility increment** applied for administration using pen device (applied only in the first year) • **Utility increment** applied for oral treatment	Annual adjustment to the **health-state utility value**	• 0.007 (pen device) • 0.004 (oral treatment)
TA1035	**Utility increment** applied to vadastutat (oral) and erythropoietin stimulating agents (IV), respectively	**Per-cycle** adjustment for patients on treatment	• 0.0103 (oral) • 0.0015 (IV)
TA973	**Utility decrement** applied for IV treatment	**Per-cycle** decrement applied to IV treatment	−0.01 (IV)
TA949	**Utility decrement** applied for IV infusion		−0.037 (IV)
TA1000	**Utility decrement** applied for IV infusion	**Annual** disutility adjustment for patients on treatment	−0.025 (IV)
TA1010	**Utility decrement** for IV administration		−0.025 (IV)
TA1012	**QALY loss** associated with injection infusion	One-off QALY loss applied to IV treatments upon model entry	−0.074 QALY loss (IV)
TA977	**QALY loss** applied associated with IV treatment		−0.187 QALY loss (IV)

Abbreviations: IV: intravenous; **PF:** progression-free; **SC:** subcutaneous; **TA:** technology appraisal; **QALY:** quality-adjusted life year.

Summary of the Observed Trends

- Where patient preference was incorporated into economic modelling, this was commonly modelled as a utility increment or decrement for the mode of treatment administration
- When applied, oral treatments were favoured over other modes of administration

The modelling of patient preference as a utility increment or decrement was largely accepted, with little/no comment from the EAG or Committee in many cases. This may be due to a number of factors:

- The impact of the utility adjustments on the model being generally minimal, meaning this was rarely a driver of cost-effectiveness
- Precedence from NICE in accepting a quality of life benefit with oral administration
- Support from clinical and patient experts

Key critiques highlighted by the EAG/Committees around the incorporation of patient preference in economic modelling included:

1. A lack of supporting evidence or justification for the specific method of incorporation
2. Limitations around the sources informing the utility adjustments
3. The potential overestimation of the impact of patient preference on HRQoL through the use of treatment-dependent utility values

Abbreviations: EAG: External Assessment Group; **HRQoL:** health-related quality of life; **NICE:** National Institute for Health and Care Excellence.

Key Critiques (1/3): Methods

EAGs/Committees highlighted a lack of justification and/or clinical validity for the methods used to incorporate patient preference in utility calculations

Case study: TA1010

- This was an appraisal of an orally administered drug delivered as an **add-on** to an IV treatment, compared with the IV treatment only
- The Company applied a **disutility** to the mode of administration for the **comparator IV treatment** only and not the novel intervention, even though this was given in combination with the IV treatment
- The EAG preferred to **remove the disutility** for the comparator IV treatment due to a lack of supporting evidence
- This disutility was also highlighted as a **key driver** of cost-effectiveness

Recommendations

- A **clear justification** should be provided for the method of utility adjustment
- Conducting **sensitivity/scenario analyses** may help to demonstrate transparently the impact of including/excluding utility adjustments for patient preference

Abbreviations: EAG: External Assessment Group; **IV:** intravenous.

Key Critiques (2/3): Sources

Limitations of sources informing the utility adjustments were highlighted

Case study: TA1003

The Matza *et al.* 2020 vignette study utilised in TA1003 was critiqued for **not following the Decision Support Unit (DSU) best practices**, such as avoiding use of value laden statements, and a lack of justification for rejecting the use of EQ-5D

Case study: TA973

- The Matza *et al.* 2019 vignette study utilised in TA973 was also critiqued given that the utility difference between 1 injection per month and oral medication was **not statistically significant**
- Utility decrement was calculated as follows:

$$utility = -\frac{Time\ in\ full\ health}{Total\ lifespan}$$

- The utility values were also **not based on EQ-5D** and therefore the EAG believed this **disqualified** it from **being used in the model**

Takeaways

- **Vignette studies** have been **accepted** as sources of patient preference. However, these were **often criticised** by the EAG/Committees
- Committees/EAGs have expressed a **preference** for basing utility values on **EQ-5D**

Recommendations

- If **vignette studies** are to be utilised, adhering to **DSU best practices** as far as possible, such as avoiding the use of **value laden statements**, is likely to increase chance of acceptance
- If **mapping or anchoring utility values to EQ-5D** is not possible, **clear justification** for such an approach may alleviate EAG and committee concern with deviating from NICE preference for EQ-5D

Abbreviations: DSU: Decision Support Unit; **EAG:** External Assessment Group; **EQ-5D:** EuroQol five-dimension questionnaire; **NICE:** National Institute for Health and Care Excellence.

Key Critiques (3/3): Overestimation

The potential overestimation of the impact of patient preference on HRQoL through the use of treatment-dependent utility values

Case study: TA1000

- Whilst the EAG **agreed** that administration-based utility **may be appropriate**, they considered this to be **overestimated** through the use of treatment-dependent health state utility values
- The Committee ultimately concluded that an **administration-based utility decrement** should be applied to **treatment independent utility values**

Recommendations

- Ensure utilities are **applied transiently** and **linked to administration**, rather than applied continuously

Abbreviations: EAG: External Assessment Group; **HRQoL:** health-related quality of life.

Summary and Future Considerations

Administration route

To date, only patient preferences related to **administration route** have been incorporated into economic modelling as utility adjustments. The potential acceptance of utility adjustment based on any **other patient-preferred characteristics**, such as frequency of administration[1], remains unclear.

Variation in acceptance

Variation in acceptance has largely been driven by EAG and committee critique of **methodological implementation**, rather than committees disagreeing with the concept of utility adjustment based on patient preference

Lack of specific guidance

Despite patient preferences contributing to more holistic decision making, at present, there exists **no specific guidance** from NICE on this topic

Low impact?

In most prior appraisals that included a utility adjustment to reflect patient preference, this was found **not to be a key model driver**. The extent to which this reflects **true patient preferences** is unclear.

Abbreviations: EAG: External Assessment Group;.

References: [1]Parke E. et al. Presented at ISPOR Europe, 12–15 November 2023. Copenhagen, Denmark. HTA132.

Summary and Future Considerations

- Incorporating patient preference **quantitatively** into economic models:
  - Would ensure that impact is directly and transparently accounted for in HTA decision-making
  - However, there do not appear to be established or consistent methods, and it is unclear whether such methods fully capture what patients value
- Patient preference could be integrated into economic analyses **qualitatively**
- Either way, incorporation needs to be accompanied by increased transparency regarding its impact on decision-making
- Prior to the development of specific guidance by HTA bodies and/or the standardisation of approach across appraisals, **research** is warranted to establish more clearly:
  - What do patients value?
  - At this time, do economic models typically reflect this appropriately?
- Current **recommendations**:
  - Submitting companies should seek early advice and align with decision-maker expectations on patient preference elicitation methods, EQ-5D anchoring, and modelling choices
  - Patient groups should be invited to comment on the validity of quantifying patient preference for different characteristics, such as treatment administrations and dosing frequencies, throughout the HTA appraisal process

Abbreviations: EQ-5D: EuroQol five-dimension questionnaire; **HTA:** health technology assessment.

Thank you for listening.

Any questions?

The authors thank Niki Lim, Costello Medical for the graphic design assistance in the preparation of this presentation.

For more information about our services, please don't hesitate to get in touch

Aneka Sowman
Consultant in HTA and Health Economics
aneka.sowman@costellomedical.com