PSI Session: Patient Preference Studies: Challenges and Opportunities

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Exec Dir Patient Experience Data Science

NOVARTIS | Reimagining Medicine

What are Patient Preferences?

Patient Experience Data (FDA)

- Data that are collected by any persons and are intended to provide information about patients' experiences with a disease or condition
- Includes the experiences, perspectives, needs and priorities of patients related to (but not limited to):
  - Symptoms of their condition and its natural history
  - Impact of the conditions on their functioning and quality of life
  - Experience with treatments
  - Input on which outcomes are important to them
  - Patient preferences for outcomes and treatments
  - Relative importance of any issues as defined by patients

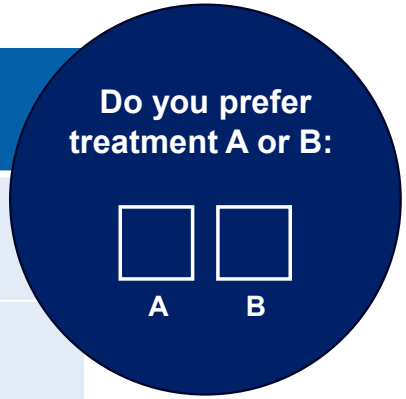
Patient Preference Studies (FDA, Novartis)

collect and **measure the value or importance** that **patients** place on various **characteristics** of a **medical product** or **disease**, ensuring a **patient-centric approach**.

One typical way to collect patient preferences

- More than 30 different methodologies exist which can answer different types of questions
- Discrete Choice Experiments (DCE) are one of the more common preference methods

DCE Attributes	Treatment A	Treatment B
Average Survival	3 years	2 years
Severe Pain	10% chance	20% chance
Severe Fatigue	40%	30%



Patient Preference Studies – External Expectations

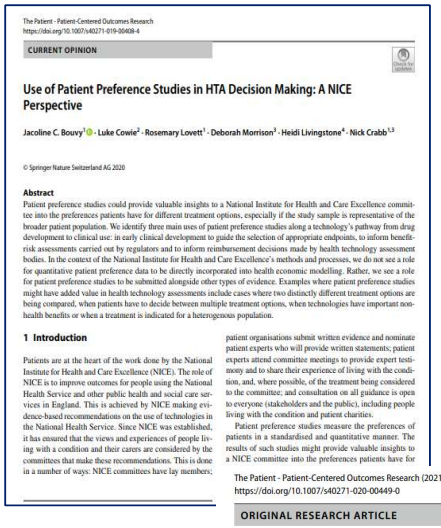
Regulators across the globe are requiring evidence of patient involvement and are expecting robust patient experience data to inform decisions. One form of generating this evidence is the conduct of Patient Preference Studies

- **FDA:** e.g. Patient-Focused Drug Development guidances, Benefit-Risk guidance, CDRH PPS guidance
- **EMA:** e.g. Qualification Opinion on IMI PREFER in 2022, Patient Experience Data reflection paper expected in Q3 2025 with guidance on PPS methods
- **ICH E22** «General considerations for Patient Preference Studies»; Planned finalization in 2026

Patient Preference Studies – External Expectations

HTA bodies / payers are also increasingly demanding evidence of patient involvement, still with a wide variation in approaches.

They might consider patient preferences as supportive evidence as investigated in special HTA projects and communicated in publications and at conferences. However, there are less guidances from HTA than for regulators on when and how preference information might support HTA decisions.



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CURRENT OPINION

Use of Patient Preference Studies in HTA Decision Making: A NICE Perspective

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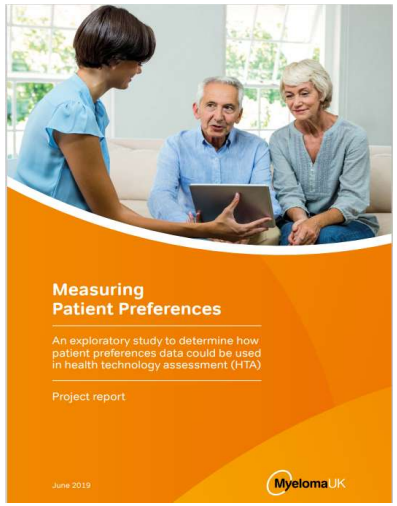
Abstract

Patient preference studies could provide valuable insights to a National Institute for Health and Care Excellence committee into the preferences patients have for different treatment options, especially if the study sample is representative of the broader patient population. We identify three main uses of patient preference studies along a technology's pathway from drug development to clinical use: in early clinical development to guide the selection of appropriate endpoints, to inform benefit-risk assessments carried out by regulators and to inform reimbursement decisions made by health technology assessment bodies. In the context of the National Institute for Health and Care Excellence's methods and processes, we do not see a role for quantitative patient preference data to be directly incorporated into health economic modelling. Rather, we see a role for patient preference studies to be submitted alongside other types of evidence. Examples where patient preference studies might have added value in health technology assessments include cases where two distinctly different treatment options are being compared, when patients have to decide between multiple treatment options, when technologies have important non-health benefits or when a treatment is indicated for a heterogenous population.

1 Introduction

Patients are at the heart of the work done by the National Institute for Health and Care Excellence (NICE). The role of NICE is to improve outcomes for people using the National Health Service and other public health and social care services in England. This is achieved by NICE making evidence-based recommendations on the use of technologies in the National Health Service. Since NICE was established, it has ensured that the views and experiences of people living with a condition and their carers are considered by the committees that make these recommendations. This is done in a number of ways: NICE committees have lay members; patient organisations submit written evidence and nominate patient experts who will provide written statements; patient experts attend committee meetings to provide expert testimony and to share their experience of living with the condition, and, where possible, of the treatment being considered to the committee; and consultation on all guidance is open to everyone (stakeholders and the public), including people living with the condition and patient charities.

Patient preference studies measure the preferences of patients in a standardised and quantitative manner. The results of such studies might provide valuable insights to a NICE committee into the preferences patients have for



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Commentary

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How to integrate evidence from patient preference studies into health technology assessment: a critical review and recommendations

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Abstract

Health technology assessment (HTA) agencies vary in their use of quantitative patient preference data (PP) and the extent to which they have formalized this use in their guidelines. Based on the authors' knowledge of the literature, we identified six different PP "use cases" that integrate PP into HTA in five different ways: through endpoint selection, clinical benefit rating, predicting uptake, input into economic evaluation, and a

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ORIGINAL RESEARCH ARTICLE

Use of Patient Preferences in Health Technology Assessment: Perspectives of Canadian, Belgian and German HTA Representatives

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prefer. PATIENT PREFERENCES

Abstract

Objective Patient preferences can be informative for health technology assessment (HTA) and payer decision making. However, applications may be different per country. The aim of this study therefore was to investigate HTA representatives' opinions on whether and how to incorporate patient preferences in HTA in their respective countries.

Methods Three country-specific focus groups were conducted with three to seven HTA representatives from Germany, Belgium, and Canada. A predefined focus group guide was used that covered topics relating to how patient preferences can be used in HTA, namely HTA stage, weight, impact, and quality, as well as a case example of gene therapy. Transcripts were analyzed using NVivo 12 following thematic analysis.

Results Across all HTA bodies, an interest in the use of patient preferences was observed for scientific advice and value assessments, but not through incorporation in quality-adjusted life-years and multi-criteria decision analysis. HTA representatives found it difficult to determine the weight patient preferences may receive in decision making, but thought it could have an impact on payer decision making if the study is of acceptable quality.

Conclusions In the near future it may be impossible to achieve structural integration of patient preferences with other evidence in HTA (e.g., in cost-effectiveness analysis), but HTA bodies are willing to incorporate patient preferences in other HTA sections as supportive evidence. To allow for that use, future work should focus on meeting HTA and payer needs when conducting patient preference studies and on education of HTA and payer representatives regarding these studies.

Patient Preference Studies – External Expectations

Regulators, HTA bodies & payers provided consistent messages during IMI PREFER about the use of patient preference information and the robustness of data:

- **Regulators & HTA:** in early clinical development Patient Preference Information is useful to guide the selection of appropriate endpoints
- **Regulators:** to inform benefit-risk assessments
- **HTA:** to inform reimbursement decisions as supportive evidence
- **Regulators & HTA** expect
  - scientifically robust patient preference studies
  - an early dialogue with the study sponsor on the study design and methodology (scientific advice)

These expectations require the right statistical skill set and experience of Patient Preference Studies.

Speaker intro

- Byron Jones (Novartis)
- Michael Bui (University Twente)
- Divya Mohan (Open Health)
- Cecilia Jimenez Moreno (Kielo Research)