FORUM

The Role of Patient Preferences in Healthcare Decision-Making

Dear Reader

The involvement of patients in decision-making processes is becoming increasingly important in healthcare. Patient preferences are not only an expression of individual needs but also an important guide for high-quality, patient-centred care. They help to align treatment decisions more closely with the realities of patients' lives while at the same time promoting the efficiency of the healthcare system.

This whitepaper explores the role patient preferences play today, how they can be captured methodologically, and the opportunities they offer for a modern, sustainable healthcare system.

We wish you an insightful read.

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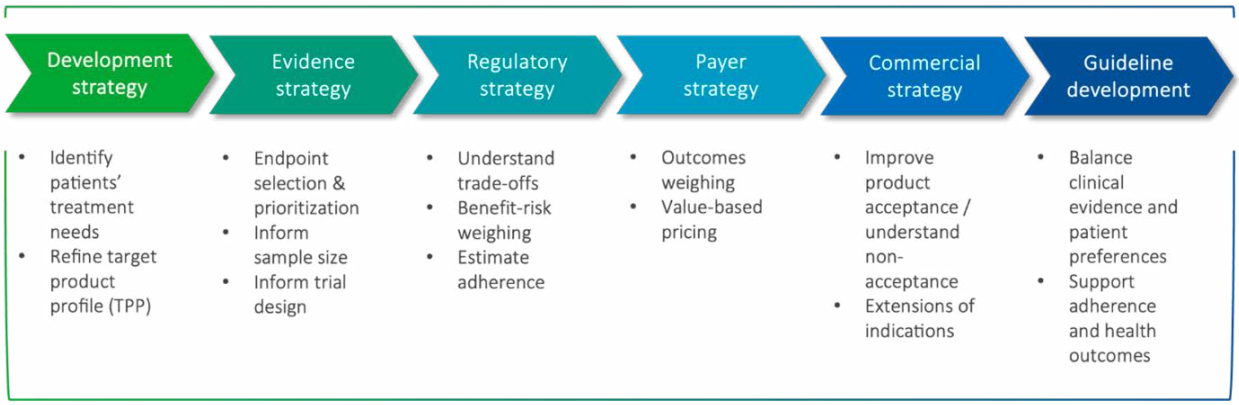
Patient preferences as a guide for decision-making in healthcare

Medical treatments, the application of medical technology, or pharmaceuticals require the consent and usually the active collaboration with patients. Therefore, it is logical to systematically consider what patients do or do not want. This is the core of patient preference research. Consequently, the consideration of patient preferences is gaining increasing importance in medical development and health policy decisions. Patient preferences are defined as the relative desirability or acceptability of attributed outcomes or other attributes of medical interventions.

Patient preferences are particularly important when decisions are „preference-sensitive“. This occurs, for example, when multiple treatment options exist, none of which is clearly superior, or when patients‘ views on the most important benefits and acceptable risks vary significantly among patients or differ from those of clinical experts. In clinical practice, patient preferences can aid in Shared Decision-Making to make the best possible choice for an individual. In regulatory approval or reimbursement decisions, information on patient preferences can help to systematically incorporate the perspective of affected individuals into the value judgments underlying these decisions. There is now broad consensus that patients‘ priorities, needs, and experiences should be considered during the development and throughout the life cycle of medical products and innovations to achieve the highest possible patient centricity.



PREFER Recommendations for Patient Preference Studies

Scientifically, Good Practice Task Forces of the „International Society for Pharmacoeconomics and Outcomes Research“ (ISPOR) have primarily focused on developing a methodological standard for preference elicitation. The U.S. Food and Drug Administration (FDA) was the first regulatory authority to consider preference studies in decisions in individual cases starting in 2015 and issued recommendations for conducting preference studies. A milestone for the consideration of patient preferences in European regulatory processes was the recognition of the recommendations of the IMI PREFER project by the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) in 2022.

PREFER Recommendations for Patient Preference Studies

The IMI PREFER project was initiated to clarify open methodological questions regarding the conduct, evaluation, and integration of results from patient preference studies. The goal was to systematically integrate findings from preference studies into decision-making processes throughout the entire life cycle of medical products, from drug development to commercialization.

Based on almost six years of research by a consortium of 33 public and private partners, the project identified over 100 different research questions and 32 qualitative and quantitative methods for eliciting patient preferences. In addition to literature-based studies, 13 methods were tested in 10 prospective application-oriented preference studies.

The PREFER recommendations cover three essential aspects:

- The **specific study objective and the decision-making situation** must be clearly defined.
- The **organization, methodological planning, and execution** of a preference study must be transparently presented in advance. A qualified, usually interdisciplinary, team should conduct the preference study based on methodological guidelines.
- The **planned application of preference data** to inform decision-making must be determined during study planning and execution. Preference data can inform various decisions throughout a product‘s life cycle.

Patient involvement as research partners is crucial at all steps of the framework to enhance the relevance, appropriateness, feasibility, and acceptability of the study design.

Methodological Approaches to Eliciting Patient Preferences

Patient preference information can be gathered through various qualitative or quantitative methods. The choice of the appropriate method depends, among other factors, on the study objective and feasibility:

- Qualitative methods provide initial insights into preference structures and primarily help to understand why certain treatment aspects are important to people. Examples include exploratory questionnaires, interviews, focus groups, or social media listening.
- Quantitative methods are suitable for quantifying the relative importance and trade-offs of treatment characteristics. Discrete Choice Experiment (DCE) is among the most frequently used methods.

Regulatory Consideration of Patient Preference Information

Among other authorities, the FDA encourages the submission of patient preference information to inform its benefit-risk assessments in approval decisions.

The FDA considers the quality of patient preference studies as „valid scientific evidence“ based on criteria such as:

- Good informing of study participants about the decision-making situation to be evaluated.
- Representativeness of the sample and generalizability of the results.

- Consideration of methodological guidelines.
- Effective and understandable communication of benefits, harms, risks, and uncertainties in the preference study.
- Inclusion of all relevant aspects of harm, risk, benefit, and uncertainty in preference elicitation, or justified omission thereof.
- Transparent assessment of the robustness of analysis results, for example, by using comprehension questions in the preference study, disclosing uncertainties, and reporting statistical measures of result certainty.

Early interactions with relevant regulatory and HTA authorities are recommended to ensure that patient preference studies provide added value and deliver results that can be integrated into the decision-making process.

Practical Application of Preference Studies

Several examples demonstrate how preference studies can significantly influence healthcare decisions and align them more closely with patient needs:

1. **In an FDA study, patients with obesity accepted a 0.01% mortality risk for a sustained 10% weight loss over five years.** These findings contributed to the benefit-risk assessment and approval of a brain stimulator.
2. **A preference study on the treatment of alopecia areata showed that patients were willing to accept a cancer risk as an adverse event of treatment to achieve hair regrowth.** This data significantly contributed to the dosing decision in the approval process.
3. **Another FDA study classified the factors that were most and least important to patients with symptomatic uterine fibroids when considering surgical treatment options.** These findings also informed the FDA‘s assessment.

Conclusion and Outlook

The integration of patient preferences into medical decision-making is a promising, but still relatively young, methodological field, even though comprehensive guidelines for conducting patient preference studies now exist. Regulatory authorities like the FDA recognize the value of patient preference information and have established criteria for its acceptance as valid scientific evidence.

Application studies demonstrate the potential that preference studies offer to better align individual therapy decisions, as well as processes and treatment methods, with the needs and preferences of patients.

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