prefer.

PATIENT PREFERENCES

PUBLICATIONS

2017-2019

PREFER looks at how and when it is best to perform and include patient preferences in decision making during the medical product life cycle. We include patient stakeholders at every level of the project.

The end-result will be recommendations to support development of guidelines for industry, Regulatory Authorities and HTA bodies. This booklet contains a list of, and links to, our publications and abstracts.

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Abstracts of publications from the PREFER project

In chronological order by date of publication.

Opportunities and challenges for the inclusion of patient preferences in the medical product life cycle: a systematic review

Janssens, Rosanne; Huys, Isabelle; van Overbeeke, Eline; Whichello, Chiara et al., BMC Medical Informatics and Decision Making, online 4 October 2019

ABSTRACT

Background: The inclusion of patient preferences (PP) in the medical product life cycle is a topic of growing interest to stakeholders such as academics, Health Technology Assessment (HTA) bodies, reimbursement agencies, industry, patients, physicians and regulators. This review aimed to understand the potential roles, reasons for using PP and the expectations, concerns and requirements associated with PP in industry processes, regulatory benefit-risk assessment (BRA) and marketing authorization (MA), and HTA and reimbursement decision-making.

Methods: A systematic review of peer-reviewed and grey literature published between January 2011 and March 2018 was performed. Consulted databases were EconLit, Embase, Guidelines International Network, PsycINFO and PubMed. A two-step strategy was used to select literature. Literature was analyzed using NVivo (QSR international).

Results: From 1015 initially identified documents, 72 were included. Most were written from an academic perspective (61%) and focused on PP in BRA/MA and/or HTA/reimbursement (73%). Using PP to improve understanding of patients' valuations of treatment outcomes, patients' benefit-risk trade-offs and preference heterogeneity were roles identified in all three decision-making contexts. Reasons for using PP relate to the unique insights and position of patients and the positive effect of including PP on the quality of the decision-making process. Concerns shared across decision-making contexts included methodological questions concerning the validity, reliability and cognitive burden of preference methods. In order to use PP, general, operational and quality requirements were identified, including recognition of the importance of PP and ensuring patient understanding in PP studies.

Conclusions: Despite the array of opportunities and added value of using PP throughout the different steps of the MPLC identified in this review, their inclusion in decision-making is hampered by methodological challenges and lack of specific guidance on how to tackle these challenges when undertaking PP studies. To support the development of such guidance, more best practice PP studies and PP studies investigating the methodological issues identified in this review are critically needed.

http://dx.doi.org/10.1186/s12911-019-0875-z

Patient Preferences in the Medical Product Life Cycle: What do Stakeholders Think? Semi-Structured Qualitative Interviews in Europe and the USA

Janssens, Rosanne; Russo, Selena; van Overbeeke, Eline; Whichello, Chiara et al., Patient, 2019 vol 12, issue 5, pp 513-526

ABSTRACT

Background: Patient preferences (PP), which are investigated in PP studies using qualitative or quantitative methods, are a growing area of interest to the following stakeholders involved in the medical product lifecycle: academics, health technology assessment bodies, payers, industry, patients, physicians, and regulators. However, the use of PP in decisions along the medical product lifecycle remains limited. As the adoption of PP heavily relies on these stakeholders, knowledge of their perceptions of PP is critical.

Objective: This study aimed to characterize stakeholders' attitudes, needs, and concerns with respect to PP in decision making along the medical product lifecycle.

Methods: Semi-structured interviews ($n = 143$) were conducted with academics ($n = 24$), health technology assessment/payer representatives ($n = 24$), industry representatives ($n = 24$), patients, caregivers and patient representatives ($n = 24$), physicians ($n = 24$), and regulators ($n = 23$) from seven European countries and the USA. Interviews were conducted between April and August 2017. The framework method was used to organize the data and identify themes and key findings in each interviewed stakeholder group.

Results: Interviewees reported being unfamiliar (43%), moderately familiar (42%), or very familiar (15%) with preference methods and studies. Interviewees across stakeholder groups generally supported the idea of using PP in the medical product lifecycle but expressed mixed opinions about the feasibility and impact of using PP in decision making. Interviewees from all stakeholder groups stressed the importance of increasing stakeholders' understanding of the concept of PP and preference methods and ensuring patients' understanding of the questions asked in PP studies. Key concerns and needs in each interviewed stakeholder group were as follows: (1) academics: investigating the validity, reliability, reproducibility, and generalizability of preference methods; (2) health technology assessment/payer representatives: developing quality criteria for evaluating PP studies and gaining insights into how to weigh them in reimbursement/payer decision making; (3) industry representatives: obtaining guidance on PP studies and recognition on the importance of PP from decision makers; (4) patients, caregivers, and patient representatives: providing an incentive and adequate information towards patients when participating in PP studies; (5) physicians: avoiding bias as a result of commercial agendas in PP studies and clarifying how to deal with subjective and emotional elements when measuring PP; and (6) regulators: avoiding the misuse of PP study results to overrule the traditional efficacy and safety criteria used for marketing authorization and obtaining robust PP study results.

Conclusions: Despite the interest all interviewed stakeholder groups reported in PP, the effective use of PP in decision making across the medical product lifecycle is currently hampered by a lack of standardization and consensus on how to both measure and use PP.

http://dx.doi.org/10.1007/s40271-019-00367-w

Factors and Situations Affecting the Value of Patient Preference Studies: Semi-Structured Interviews in Europe and the US

Whichello, Chiara; van Overbeeke, Eline; Janssens, Rosanne; Schölin Bywall, Karin et al., Frontiers in Pharmacology, online 18 September 2019

ABSTRACT

Objectives: Patient preference information (PPI) is gaining recognition among the pharmaceutical industry, regulatory authorities, and health technology assessment (HTA) bodies/payers for use in assessments and decision-making along the medical product lifecycle (MPLC). This study aimed to identify factors and situations that influence the value of patient preference studies (PPS) in decision-making along the MPLC according to different stakeholders.

Methods: Semi-structured interviews (n = 143) were conducted with six different stakeholder groups (physicians, academics, industry representatives, regulators, HTA/payer representatives, and a combined group of patients, caregivers, and patient representatives) from seven European countries (the United Kingdom, Sweden, Italy, Romania, Germany, France, and the Netherlands) and the United States. Framework analysis was performed using NVivo 11 software.

Results: Fifteen factors affecting the value of PPS in the MPLC were identified. These are related to: study organization (expertise, financial resources, study duration, ethics and good practices, patient centeredness), study design (examining patient and/or other preferences, ensuring representativeness, matching method to research question, matching method to MPLC stage, validity and reliability, cognitive burden, patient education, attribute development), and study conduct (patients' ability/willingness to participate and preference heterogeneity). Three types of situations affecting the use of PPS results were identified (stakeholder acceptance, market situations, and clinical situations).

Conclusion: The factors and situation types affecting the value of PPS, as identified in this study, need to be considered when designing and conducting PPS in order to promote the integration of PPI into decision-making along the MPLC.

https://doi.org/10.3389/fphar.2019.01009

Methods for exploring and eliciting patient preferences in the medical product lifecycle: a literature review

Soekhai, Vikas; Whichello, Chiara; Levitan, Bennet; Veldwijk, Jorien et al., Drug Discovery Today, 2019, Volume 24, Issue 7, pp 1324-1331

ABSTRACT

Preference studies are becoming increasingly important within the medical product decision-making context. Currently, there is limited understanding of the range of methods to gain insights into patient preferences. We developed a compendium and taxonomy of preference exploration (qualitative) and elicitation (quantitative) methods by conducting a systematic literature review to identify these methods. This review was followed by analyzing prior preference method reviews, to cross-validate our results, and consulting intercontinental experts, to confirm our outcomes. This resulted in the identification of 32 unique preference methods. The developed compendium and taxonomy can serve as an important resource for assessing these methods and helping to determine which are most appropriate for different research questions at varying points in the medical product lifecycle.

HIGHLIGHTS

- Preference studies are becoming increasingly important within the medical product decision-making context.
- Currently, there is limited understanding of the range of methods to gain insights into patient preferences.
- We developed a compendium and taxonomy of preference exploration (qualitative) and elicitation (quantitative) methods.
- We identified 32 unique preference methods.
- Our results can serve as an important resource for determining which preference methods are most promising in the medical product lifecycle.

https://doi.org/10.1016/j.drudis.2019.05.001

Understanding Patients' Preferences: A Systematic Review of Psychological Instruments Used in Patients' Preference and Decision Studies

Russo, Selena; Jongerius, Chiara; Faccio, Flavia; Pizzoli, Silvia F.M. et al., Value in Health, 2019, Volume 22, Issue 4, pp 491-501, 2019.

ABSTRACT

Background: Research has been mainly focused on how to elicit patient preferences, with less attention on why patients form certain preferences.

Objectives: To assess which psychological instruments are currently used and which psychological constructs are known to have an impact on patients' preferences and health-related decisions including the formation of preferences and preference heterogeneity.

Methods: A systematic database search was undertaken to identify relevant studies. From the selected studies, the following information was extracted: study objectives, study population, design, psychological dimensions investigated, and instruments used to measure psychological variables.

Results: Thirty-three studies were identified that described the association between a psychological construct, measured using a validated instrument, and patients' preferences or health-related decisions. We identified 33 psychological instruments and 18 constructs, and categorized the instruments into 5 groups, namely, motivational factors, cognitive factors, individual differences, emotion and mood, and health beliefs.

Conclusions: This review provides an overview of the psychological factors and related instruments in the context of patients' preferences and decisions in healthcare settings. Our results indicate that measures of health literacy, numeracy, and locus of control have an impact on health-related preferences and decisions. Within the category of constructs that could explain preference and decision heterogeneity, health locus of control is a strong predictor of decisions in several healthcare contexts and is useful to consider when designing a patient preference study. Future research should continue to explore the association of psychological constructs with preference formation and heterogeneity to build on these initial recommendations.

HIGHLIGHTS

- Patients' preferences are a growing topic of interest. There has been a call by stakeholders (eg, regulators, payers, industry, and patient organizations) for greater involvement of patients in the healthcare decision-making process. To date, most of the attention has been focused on how to elicit preferences, with less attention focused on why patients form certain preferences and why they make certain decisions. An overview of psychological dimensions and instruments used in patients' preferences and health-related decision studies is lacking.
- To our knowledge, our article is the first to review psychological constructs and instruments in the context of patients' preferences and health-related decision studies. This review identifies constructs and instruments able to evaluate the psychological profile of patients that may reveal crucial determinants of the patients' preferences and decisions and their heterogeneity in the healthcare setting(s).
- Our article provides a starting point to further develop a theoretical framework for inclusion of psychological dimensions and related instruments in preference elicitation studies of medicinal products and medical devices.

https://doi.org/10.1016/j.jval.2018.12.007

Giving patients' preferences a voice in the medical product lifecycle: why, when and how?: The public-private PREFER project: Work package 2

de Bekker-Grob, Esther; Juhaeri, Juhaeri; Kihlbom, Ulrik; Levitan, Bennett, ISPOR Value & Outcomes Spotlight, 2018, Vol 4, No 3, pp 19-21

ABSTRACT

PREFER is a five-year project funded equally by the Innovative Medicines Initiative (IMI; Europe's largest public-private initiative aiming to speed the development of better and safer medicines for patients) and by industry as in-kind contribution. IMI is a partnership between the European Union's Horizon 2020 program and the European pharmaceutical industry represented by EFPIA (the European Federation of Pharmaceutical Industries and Associations). This paper describes the structure of the project, the work package devoted to answering when and how patient preferences should be considered in the medical product life-cycle.

http://urn.kb.se/resolve?urn=urn:nbn:se:uu:diva-350905

Factors and situations influencing the value of patient preference studies along the medical product lifecycle: a literature review

van Overbeeke, Eline; Whichello, Chiara; Janssens, Rosanne; Veldwijk, Jorien et al., Drug Discovery Today, 2018. Volume 24, Issue 1, January 2019, pp 57-6

ABSTRACT

Industry, regulators, health technology assessment (HTA) bodies, and payers are exploring the use of patient preferences in their decision-making processes. In general, experience in conducting and assessing patient preference studies is limited. Here, we performed a systematic literature search and review to identify factors and situations influencing the value of patient preference studies, as well as applications throughout the medical product lifecyle. Factors and situations identified in 113 publications related to the organization, design, and conduct of studies, and to communication and use of results. Although current use of patient preferences is limited, we identified possible applications in discovery, clinical development, marketing authorization, HTA, and postmarketing phases.

HIGHLIGHTS

- Use of patient preferences in decision making is limited but gaining attention.
- Some situations and decisions are more sensitive to patient preferences than others.
- Patient centeredness in design, conduct and communication of results is key.
- In method selection and instrument design many factors can affect validity.
- Capturing preference heterogeneity greatly increases the value of patient preference studies.

https://doi.org/10.1016/j.drudis.2018.09.015

Giving Patients' Preferences a Voice in Medical Treatment Life Cycle: The PREFER Public–Private Project

de Bekker-Grob, Esther; Berlin, Conny; Levitan, Bennet; Raza, Karim et al., Patient, 2019, Volume 10, Issue 3, pp 263–266

ABSTRACT

The patient perspective is important in all medical research, particularly in developing new treatments (i.e., drugs, medical devices, and vaccines). Treatments are developed for patients, and there is an emerging consensus that patients should be involved at crucial decision points in the treatment life cycle. As such, taking into consideration the patient voice has become increasingly important not only for the companies that develop new treatments but also for the authorities that assess, regulate, and decide which treatments are effective, safe, well-tolerated, and cost effective.

In general, stakeholders (i.e., industry, regulatory authorities, health technology assessment [HTA] bodies, reimbursement agencies, clinicians, and patient organizations) all agree about the importance of incorporating patients' preferences, needs, and perspectives into decision making and the need to provide more avenues for patient engagement. However, there is little guidance on incorporating scientifically valid preference measurements into the treatment development life cycle or into regulatory and reimbursement decision-making processes regarding medical treatments. Important questions include the following: What is an appropriate structured approach to assess and use patient preferences during the development, approval, and post-approval phases of medical products? What kind of qualitative and quantitative methods exist to obtain insight into patient preferences? What level of validity, representativeness, and robustness is necessary? Which preference-measurement method should be used in what key decision points in the medicinal product life cycle? How will these patient preference approaches satisfy the needs of the different stakeholders, specifically regulatory, HTA, and reimbursement bodies, and feed into their existing decision-making processes? To what extent can we identify generic approaches to preference elicitation as opposed to disease- or disease area-specific approaches? How transferable are patient-preference data from country to country?

The answers to these questions should accommodate the requirements of different stakeholders and decision makers in a medicine's life cycle. Therefore, combining a multi-disciplinary approach with a consortium of various stakeholders is essential, allowing these urgent and relevant questions to be answered and giving patients' preferences appropriate roles in the treatment life cycle. PREFER (Patient Preferences in Benefit and Risk Assessments during the Treatment Life Cycle) is a public–private research initiative that has recently been launched to tackle these challenges.

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