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PATIENT PREFERENCES

PUBLICATIONS

2017 - 2021

The Patient Preferences in Benefit-Risk Assessments during the Drug Life Cycle (PREFER) project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 115966. This Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and the European Federation of Pharmaceutical Industries and Associations (EFPIA). This booklet and its contents reflects the PREFER project's view and not the view of IMI, the European Union or EFPI

Publications from the PREFER project

PREFER is developing recommendations for when and how patient preferences can contribute to industry, regulatory and HTA decision making throughout the medical product life cycle. We include patient stakeholders at every level of the project. This pdf booklet includes a list of our publications in chronological order (latest first), with abstracts and links to full text. The list was updated on 23 September 2021.

Contents

Key Determinants of Health-Related Quality of Life Among Advanced Lung Cancer Patients: A Qualitative Study in Belgium and Italy

Janssens, Rosanne; Arnou, Reinhard; Schoefs, Elise; Petrocchi, Serena et al. Frontiers in Pharmacology, 2021, Volume 4, Article 710518

ABSTRACT

Background: The lung cancer (LC) treatment landscape has drastically expanded with the arrival of immunotherapy and targeted therapy. This new variety of treatment options, each with its own characteristics, raises uncertainty regarding the key aspects affecting patients' health-related quality of life (HRQL). The present qualitative study aimed to investigate how LC patients perceive their HRQL and the factors that they consider to be most influential in determining their HRQL.

Methods: This qualitative research incorporates four focus group discussions, with six LC patients in each group. In total, 24 stage III and IV LC patients were included in the discussions, with Italian (*n* = 12) and Belgian (*n* = 12) patients, age range: 42–78, median age = 62 (IQR = 9.3 years), SD = 8.5; 62% men. Using thematic analysis, transcripts and notes from the FGDs were analyzed using NVivo software (edition 12).

Results: Three main themes capturing determinants of HRQL were identified. First, patients agreed on the importance of physical aspects (symptoms and side-effects) in determining their HRQL. In particular, skin conditions, nausea, fatigue, risk of infections, sensory abnormalities, pain, and changes in physical appearance were highlighted. Second, patients worried about psychological aspects, negatively impacting their wellbeing such as uncertainties regarding their future health state, and a lower degree of autonomy and independence. Third, patients underlined the importance of social aspects, such as communication with healthcare providers and social interaction with friends, family and peers.

Conclusion: This study demonstrates that physical, psychological, and social aspects are key factors driving LC patients' HRQL. Gaining a better understanding of how LC patients perceive their HRQL and how it is affected by their illness and therapy will aid patient-centric decision-making across the drug life cycle, by providing stakeholders (drug developers, regulators, reimbursement bodies, and clinicians) insights about the treatment and disease aspects of importance to LC patients as well as the unmet needs LC patients may have regarding available treatment modalities. Finally, this study underscores a need for individual treatment decision-making that is considerate of uncertainties among LC patients about their future health state, and ways for improving communication between healthcare providers and patients to do so.

https://doi.org/10.3389/fphar.2021.710518

Patient preferences for gene therapy in haemophilia: Results from the PAVING threshold technique survey

van Overbeeke, Eline; Hauber, Brett; Michelsen, Sissel; Peerlinck, Kathelijne et al., Haemophilia, online 01 September 2021

ABSTRACT

Objectives: The aim of the Patient preferences to Assess Value IN Gene therapies (PAVING) study was to investigate trade-offs that adult Belgian people with haemophilia (PWH) A and B are willing to make when choosing between prophylactic factor replacement therapy (PFRT) and gene therapy.

Methods: The threshold technique was used to quantify the minimum acceptable benefit (MAB) of a switch from PFRT to gene therapy in terms of 'Annual bleeding rate' (ABR), 'Chance to stop prophylaxis' (STOP), and 'Quality of life' (QOL). The design was supported by stakeholder involvement and included an educational tool on gene therapy. Threshold intervals were analysed using interval regression models in Stata 16.

Results: A total of 117 PWH completed the survey. Mean thresholds were identified for all benefits, but substantial preference heterogeneity was observed; especially for the STOP thresholds, where the distribution of preferences was bimodal. Time spent on the educational tool and residence were found to impact MAB thresholds. The most accepted (88% of PWH) gene therapy profile investigated in this study comprised of zero bleeds per year (vs. six for PFRT), 90% chance to stop prophylaxis, no impact on QoL, and 10 years of follow-up on side effects (vs. 30 for PFRT).

Conclusions: Results from this study proved the value of educating patients on novel treatments. Moreover, preference heterogeneity for novel treatments was confirmed in this study. In gene therapy decision-making, preference heterogeneity and the impact of patient education on acceptance should be considered.

https://doi.org/10.1111/hae.14401

Taking into Account Patient Preferences: A Consensus Study on the Assessment of Psychological Dimensions Within Patient Preference Studies

Russo, Selena; Monzani, Dario; Pinto, Cathy Anne; Vergani, Laura et al., Patient Preference and Adherence, 2021. Volume 15, pp 1331–1345

ABSTRACT

Patient preferences are gaining recognition among key stakeholders involved in benefit-risk decision-making along the medical product lifecycle. However, one of the main challenges of integrating patient preferences in benefit-risk decision-making is understanding differences in patient preference, which may be attributable to clinical characteristics (eg age, medical history) or psychosocial factors. Measuring the latter may provide valuable information to decision-makers but there is limited guidance regarding which psychological dimensions may influence patient preferences and which psychological instruments should be considered for inclusion in patient preference studies. This paper aims to provide such guidance by advancing evidence and consensus-based recommendations and considerations. Findings of a recent systematic review

on psychological constructs having an impact on patients' preferences and health-related decisions were expanded with input from an expert group (n = 11). These data were then used as the basis for final recommendations developed through two rounds of formal evaluation via an online Delphi consensus process involving international experts in the field of psychology, medical decision-making, and risk communication (n = 27). Three classes of recommendations emerged. Eleven psychological constructs reached consensus to be recommended for inclusion with the strongest consensus existing for health literacy, numeracy, illness perception and treatment-related beliefs. We also proposed a set of descriptive and checklist criteria to appraise available psychological measures to assist researchers and other stakeholders in including psychological assessment when planning patient preference studies. These recommendations can guide researchers and other stakeholders when designing and interpreting patient preference studies with a potential high impact in clinical practice and medical product benefit-risk decision-making processes.

https://doi.org/10.2147/PPA.S261615

Patient Preferences for Multiple Myeloma Treatments: A Multinational Qualitative Study

Janssens, Rosanne; Lang, Tamika; Vallejo, Anna; Galinsky, Jayne et al. Frontiers in Medicine, 2021, Volume 8, Article 686165

ABSTRACT

Background: Investigational and marketed drugs for the treatment of multiple myeloma (MM) are associated with a range of characteristics and uncertainties regarding long term side-effects and efficacy. This raises questions about what matters most to patients living with this disease. This study aimed to understand which characteristics MM patients find most important, and hence should be included as attributes and levels in a subsequent quantitative preference survey among MM patients.

Methods: This qualitative study involved: (i) a scoping literature review, (ii) discussions with MM patients (n = 24) in Belgium, Finland, Romania, and Spain using Nominal Group Technique, (iii) a qualitative thematic analysis including multi-stakeholder discussions.

Results: MM patients voiced significant expectations and hopes that treatments would extend their lives and reduce their cancer signs and symptoms. Participants however raised concerns about life-threatening side-effects that could cause permanent organ damage. Bone fractures and debilitating neuropathic effects (such as chronic tingling sensations) were highlighted as major issues reducing patients' independence and mobility. Patients discussed the negative impact of the following symptoms and side-effects on their daily activities: thinking problems, increased susceptibility to infections, reduced energy, pain, emotional problems, and vision problems. MM patients were concerned with uncertainties regarding the durability of positive treatment outcomes, and the cause, severity, and duration of their symptoms and side-effects. Patients feared short-term positive treatment responses complicated by permanent, severe side-effects and symptoms.

Conclusions: This study gained an in-depth understanding of the treatment and disease-related characteristics and types of attribute levels (severity, duration) that are most important to MM patients. Results from this study argue in favor of MM drug development and individual treatment decision-making that focuses not only on extending patients' lives but also on addressing those symptoms and side-effects that significantly impact MM patients' quality of life. This study

underscores a need for transparent communication toward MM patients about MM treatment outcomes and uncertainties regarding their long-term efficacy and safety. Finally, this study may help drug developers and decision-makers understand which treatment outcomes and uncertainties are most important to MM patients and therefore should be incorporated in MM drug development, evaluation, and clinical practice.

https://doi.org/10.3389/fmed.2021.686165

Treatment preferences for preventive interventions for rheumatoid arthritis: protocol of a mixed methods case study for the Innovative Medicines Initiative PREFER project

Falahee, Marie; Simons, Gwenda; DiSantostefano, Rachael; Valor Méndez, Larissa et al. BMJ Open, 2021, Volume 11, Issue 4

ABSTRACT

Introduction: Amidst growing consensus that stakeholder decision-making during drug development should be informed by an understanding of patient preferences, the Innovative Medicines Initiative project 'Patient Preferences in Benefit-Risk Assessments during the Drug Life Cycle' (PREFER) is developing evidence-based recommendations about how and when patient preferences should be integrated into the drug life cycle. This protocol describes a PREFER clinical case study which compares two preference elicitation methodologies across several populations and provides information about benefit–risk trade-offs by those at risk of rheumatoid arthritis (RA) for preventive interventions.

Methods and analysis: This mixed methods study will be conducted in three countries (UK, Germany, Romania) to assess preferences of (1) first-degree relatives (FDRs) of patients with RA and (2) members of the public. Focus groups using nominal group techniques (UK) and ranking surveys (Germany and Romania) will identify and rank key treatment attributes. Focus group transcripts will be analysed thematically using the framework method and average rank orders calculated. These results will inform the treatment attributes to be assessed in a survey including a discrete choice experiment (DCE) and a probabilistic threshold technique (PTT). The survey will also include measures of sociodemographic variables, health literacy, numeracy, illness perceptions and beliefs about medicines. The survey will be administered to (1) 400 FDRs of patients with RA (UK); (2) 100 FDRs of patients with RA (Germany); and (3) 1000 members of the public in each of UK, Germany and Romania. Logit-based approaches will be used to analyse the DCE and imputation and interval regression for the PTT.

Ethics and dissemination: This study has been approved by the London-Hampstead Research Ethics Committee (19/LO/0407) and the Ethics Committee of the Friedrich-Alexander-Universität Erlangen-Nürnberg (92_17 B). The protocol has been approved by the PREFER expert review board. The results will be disseminated widely and will inform the PREFER recommendations.

https://doi.org/10.1136/bmjopen-2020-045851

Does been exposed to an educational tool influence patient preferences? The influence of an educational tool on patient preferences assessed by a discrete choice experiment.

Schölin Bywall, Karin; Veldwijk, Jorien; Hansson, Mats G.; Baecklund, Eva et al., Patient Education and Counseling, online 10 March 2021

ABSTRACT

Objectives: There is an increased interest in patient preferences informing the development and authorisation of medical products. A requirement for robust and meaningful results of such studies is that patients adequately understand the risks and benefits associated with treatments for which their preferences are elicited. This study aims to determine the influence of an educational tool, compared with traditional written information on patient preferences elicited in a discrete choice experiment (DCE).

Methods: Treatment preferences of Swedish patients with rheumatoid arthritis (RA) were assessed using a DCE. Patients were recruited via clinics, a research panel, and the Swedish Rheumatism Association. Respondents received training materials either as plain written text or as an online educational tool. The educational tool was designed to enhance understanding of the written text by using graphics, pictograms, icon arrays, spoken text, and click-on functions. Data were analysed using random parameter logit models.

Results: 675 patients with RA were included in the analysis. The patients received either a written information (n = 358) or information via an educational tool (n = 317). Respondents receiving the educational tool placed relatively more importance on all included side effects in their decision making, compared to respondents receiving the written text, who placed greater importance on treatment effectiveness and administration methods.

Conclusion: Compared to the respondents receiving the written text, the decisions of respondents receiving the educational tool were more influenced by medication side effects. Further research is needed to provide guidance on how and when to use educational tools to inform and elicit patients' preferences.

Practice implications: The ways in which attributes are presented to patients significantly impacts preferences measured in a DCE.

https://doi.org/10.1016/j.pec.2021.03.013

Methodological Priorities for Patient Preferences Research: Stakeholder Input to the PREFER Public–Private Project

Smith, Ian P.; DiSantostefano, Rachael L.; de Bekker-Grob, Esther W.; Levitan, Bennet et al., Patient, 2021, Volume 14, pp 449–453

ABSTRACT

Patient advocacy groups, regulatory agencies, and industry have increasingly advocated for patient engagement in decisions across the medical product lifecycle (MPLC). Among the array of approaches to obtain patient input, an increasingly popular and important approach is the use of patient preferences, including an understanding of which endpoints are most important to patients and of the patient perspective on benefit–risk (B-R) trade-offs when making treatment and

reimbursement decision. This push to involve patient preference information throughout the MPLC has resulted in a growing body of knowledge and experience in this field, which in turn has stimulated a growing interest in how best to conduct patient preference studies. Despite the increasing frequency with which patient preference studies are conducted, there remain many unanswered questions regarding how to incorporate scientifically valid preference measurements into MPLC decision making regarding medical treatments, including development, regulatory and reimbursement decisions. Previous groups such as the Medical Device Innovation Consortium (MDIC) have worked to address these issues but many questions remain. To answer some of these questions, the European public-private partnership PREFER ('Patient Preferences in Benefit and Risk Assessments during the Drug Lifecycle') was launched in 2016.

PREFER is a 5-year project, funded jointly by the Innovative Medicines Initiative (IMI) 2 (EU Horizon 2020) and the European pharmaceutical industry [represented by the European Federation of Pharmaceutical Industries and Associations (EFPIA)]. PREFER aims to strengthen patient-centric decision making throughout the MPLC by developing evidence-based recommendations to fill the gaps in knowledge regarding the methodological aspects of patient preference studies.

http://dx.doi.org/10.1007/s40271-021-00502-6

Patient Preferences to Assess Value IN Gene Therapies: Protocol Development for the PAVING Study in Hemophilia

van Overbeeke, Eline; Hauber, Brett; Michelsen, Sissel; Goldman, Michel et al., Frontiers in Medicine, 2021, Volume 8, Article 595797

ABSTRACT

Introduction: Gene therapies are innovative therapies that are increasingly being developed. However, health technology assessment (HTA) and payer decision making on these therapies is impeded by uncertainties, especially regarding long-term outcomes. Through measuring patient preferences regarding gene therapies, the importance of unique elements that go beyond health gain can be quantified and inform value assessments. We designed a study, namely the Patient preferences to Assess Value IN Gene therapies (PAVING) study, that can inform HTA and payers by investigating trade-offs that adult Belgian hemophilia A and B patients are willing to make when asked to choose between a standard of care and gene therapy.

Methods and Analysis: An eight-step approach was taken to establish the protocol for this study: (1) stated preference method selection, (2) initial attributes identification, (3) stakeholder (HTA and payer) needs identification, (4) patient relevant attributes and information needs identification, (5) level identification and choice task construction, (6) educational tool design, (7) survey integration, and (8) piloting and pretesting. In the end, a threshold technique survey was designed using the attributes "Annual bleeding rate," "Chance to stop prophylaxis," "Time that side effects have been studied," and "Quality of Life."

Ethics and Dissemination: The Medical Ethics Committee of UZ KU Leuven/Research approved the study. Results from the study will be presented to stakeholders and patients at conferences and in peer-reviewed journals. We hope that results from the PAVING study can inform decision makers on the acceptability of uncertainties and the value of gene therapies to patients.

https://doi.org/10.3389/fmed.2021.595797

Patient Preferences in Rare Diseases: A Qualitative Study in Neuromuscular Disorders to Inform a Quantitative Preference Study

Jimenez-Moreno, A. Cecilia; van Overbeeke, Eline; Pinto, Cathy Anne; Smith, Ian et al., Patient, 2021, Volume 14, pp 601–612

ABSTRACT

Introduction: It has become increasingly important to include patient preference information in decision-making processes for drug development. As neuromuscular disorders represent multisystem, debilitating, and progressive rare diseases with few treatment options, this study aimed to explore unmet health care needs and patient treatment preferences for two neuromuscular disorders, myotonic dystrophy type 1 (DM1) and mitochondrial myopathies (MM) to inform early stages of drug development.

Methods: Fifteen semi-structured interviews and five focus group discussions (FGDs) were held with DM1 and MM adult patients and caregivers. Topics discussed included (1) reasons for study participation; (2) disease signs/symptoms and their impact on daily lives; (3) top desired benefits; and (4) acceptability of risks and tolerance levels for a hypothetical new treatment. Data were analyzed following a thematic 'code' approach.

Results: A total of 52 participants representing a wide range of disease severities participated. 'Muscle strength' and 'energy and endurance' were the disease-related unmet needs most often mentioned. Additionally, improved 'balance', 'cognition' and 'gut function' were the top desired treatment benefits, while 'damage to the liver, kidneys or eyes' was the most concerning risk. Factors influencing their tolerance to risks related to previously having experienced the risk and differentiation between permanent and temporary risks. A few differences were elicited between patients and caregivers.

Conclusions: This qualitative study provided an open forum to elicit treatment-desired benefits and acceptable risks to be established by patients themselves. These findings can inform decisions for developing new treatments and the design of clinical trials for DM1 and MM.

https://doi.org/10.1007/s40271-020-00482-z

What Matters Most to Lung Cancer Patients? A Qualitative Study in Italy and Belgium to Investigate Patient Preferences

Petrocchi, Serena; Janssens, Rosanne; Oliveri, Serena; Arnou, Reinhard et al., Frontiers in Pharmacology, 2021, Volume 12, Article 602112

ABSTRACT

Background: The potential value of patient preference studies has been recognized in clinical individual treatment decision-making between clinicians and patients, as well as in upstream drug decision-making. Drug developers, regulators, reimbursement and Health Technology Assessment (HTA) bodies are exploring how the use of patient preference studies could inform drug development, regulatory benefit risk-assessment and reimbursement decisions respectively. Understanding patient preferences may be especially valuable in decisions regarding Non-Small Cell Lung Cancer (NSCLC) treatment options, where a variety of treatment options with different characteristics raise uncertainty about which features are most important to NSCLC patients. As

part of the Innovative Medicines Initiative PREFER project, this qualitative study aimed to identify patient-relevant lung cancer treatment characteristics.

Methods: This study consisted of a scoping literature review and four focus group discussions, 2 in Italy and 2 in Belgium, with a total of 24 NSCLC patients (Stages III-IV). The focus group discussions sought to identify which treatment characteristics patients find most relevant. The discussions were analyzed thematically using a thematic inductive analysis.

Results: Patients highlighted themes reflecting: 1) positive effects or expected gains from treatment such as greater life expectancy and maintenance of daily functioning, 2) negative effects or adverse events related to therapy that negatively impact patients' daily functioning such as fatigue and 3) uncertainty regarding the duration and type of treatment effects. These overarching themes were consistent among patients from Belgium and Italy, suggesting that treatment aspects related to efficacy and safety as well as the psychological impact of lung cancer treatment are common areas of concern for patients, regardless of cultural background or country.

Discussion: Our findings illustrate the value of using qualitative methods with patients to identify preferred treatment characteristics for advanced lung cancer. These could inform a subsequent quantitative preference survey that assesses patient trade-offs regarding treatment options.

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Patient Preferences for Lung Cancer Treatment: A Qualitative Study Protocol Among Advanced Lung Cancer Patients

Durosini, Ilaria; Janssens, Rosanne; Arnou, Reinhard; Veldwijk, Jorien et al., Frontiers In Public Health, 2021, Volume 9, Article 622154

ABSTRACT

Introduction: Lung cancer is the deadliest and most prevalent cancer worldwide. Lung cancer treatments have different characteristics and are associated with a range of benefits and side effects for patients. Such differences may raise uncertainty among drug developers, regulators, payers, and clinicians regarding the value of these treatment effects to patients. The value of conducting patient preference studies (using qualitative and/or quantitative methods) for benefits and side effects of different treatment options has been recognized by healthcare stakeholders, such as drug developers, regulators, health technology assessment bodies, and clinicians. However, evidence-based guidelines on how and when to conduct and use these studies in drug decision-making are lacking. As part of the Innovative Medicines Initiative PREFER project, we developed a protocol for a qualitative study that aims to understand which treatment characteristics are most important to lung cancer patients and to develop attributes and levels for inclusion in a subsequent quantitative preference survey.

Methods: The study protocol specifies a four-phased approach: (i) a scoping literature review of published literature, (ii) four focus group discussions with stage III and IV Non-Small Cell Lung Cancer patients, (iii) two nominal group discussions with stage III and IV Non-Small Cell Lung Cancer patients, and (iv) multi-stakeholder discussions involving clinicians and preference experts.

Discussion: This protocol outlines methodological and practical steps as to how qualitative research can be applied to identify and develop attributes and levels for inclusion in patient preference studies aiming to inform decisions across the drug life cycle. The results of this study are intended to inform a subsequent quantitative preference survey that assesses patient trade-offs regarding lung cancer treatment options. This protocol may assist researchers, drug developers, and decision-makers in designing qualitative studies to understand which treatment aspects are most valued by patients in drug development, regulation, and reimbursement.

http://dx.doi.org/10.3389/fpubh.2021.622154

A study protocol for quantifying patient preferences in neuromuscular disorders: a case study of the IMI PREFER Project

Jimenez-Moreno, Aura Cecilia; Pinto, Cathy Anne; Levitan, Bennett; Whichello, Chiara et al., Wellcome Open Research, online 23 October 2020

ABSTRACT

Objectives: Patient preference studies are increasingly used to inform decision-making during the medical product lifecycle but are rarely used to inform early stages of drug development. The primary aim of this study is to quantify treatment preferences of patients with neuromuscular disorders, which represent serious and debilitating conditions with limited or no treatment options available.

Methods: This quantitative patient preferences study was designed as an online survey, with a cross-over design. This study will target two different diseases from the neuromuscular disorders disease group, myotonic dystrophy type 1 (DM1) and mitochondrial myopathies (MM). Despite having different physio-pathological pathways both DM1 and MM manifest in a clinically similar manner and may benefit from similar treatment options. The sample will be stratified into three subgroups: two patient groups differentiated by age of symptom onset and one caregivers group. Each subgroup will be randomly assigned to complete two of three different preference elicitation methods at two different time points: Q-methodology survey, discrete choice experiment, and best-worst scaling type 2, allowing cross-comparisons of the results across each study time within participants and within elicitation methods. Additional variables such as sociodemographic, clinical and health literacy will be collected to enable analysis of potential heterogeneity.

Ethics and Dissemination: This study protocol has undergone ethical review and approval by the Newcastle University R&D Ethics Committee (Ref: 15169/2018). All participants will be invited to give electronic informed consent to take part in the study prior accessing the online survey. All electronic data will be anonymised prior analysis. This study is part of the Patient Preferences in Benefit-Risk Assessments during the Drug Life Cycle (IMI-PREFER) project, a public-private collaborative research project aiming to develop expert and evidence-based recommendations on how and when patient preferences can be assessed and used to inform medical product decision making.

https://doi.org/10.12688/wellcomeopenres.16116.1

An overview of critical decision-points in the medical product lifecycle: Where to include patient preference information in the decision-making process?

Whichello, Chiara; Schölin Bywall, Karin; Mauer, Jonathan; Watt, Stephen et al., Health Policy, 2020, Volume 124, Issue 12, pp 1325–1332

ABSTRACT

Background: Patient preference (PP) information is not effectively integrated in decision-making throughout the medical product lifecycle (MPLC), despite having the potential to improve patients' healthcare options. A first step requires an understanding of existing processes and decision-points to know how to incorporate PP information in order to improve patient-centric decision-making.

Objectives: The aims were to: 1) identify the decision-making processes and decision-points throughout the MPLC for industry, regulatory authorities, and reimbursement/HTA, and 2) determine which decision-points can potentially include PP information.

Methods: A scoping literature review was conducted using five scientific databases. Semi-structured interviews were conducted with representatives from seven European countries and the US, including industry (n = 24), regulatory authorities (n = 23), reimbursement/HTA (n = 23). Finally, validation meetings with key stakeholders (n = 11) were conducted.

Results: Six critical decision-points were identified for industry decision-making, three for regulatory decision-making, and six for reimbursement/HTA decision-making. Stakeholder groups agreed that PP information is not systematically integrated, either as obligatory information or pre-set criteria, but would benefit all the listed decision-points in the future.

Conclusion: Currently, PP information is not considered as obligatory information to submit for any of the MPLC decision-points. However, PP information is considered an important component by most stakeholders to inform future decision-making across the MPLC. The integration of PP information into 15 identified decision-points needs continued discussion and collaboration between stakeholders.

http://dx.doi.org/10.1016/j.healthpol.2020.07.007

Patient preferences on rheumatoid arthritis second-line treatment: a discrete choice experiment of Swedish patients

Schölin Bywall, Karin; Kihlbom, Ulrik; Hansson, Mats; Falahee, Marie et al., Arthritis Research & Therapy, 2020, Volume 22, Article 288

ABSTRACT

Background: Preference assessments of patients with rheumatoid arthritis can support clinical therapeutic decisions for including biologic and targeted synthetic medicines to use. This study assesses patient preferences for attributes of second-line therapies and heterogeneity within these preferences to estimate the relative importance of treatment characteristics and to calculate the minimum benefit levels patients require to accept higher levels of side effects.

Methods: Between November 2018 to August 2019, patients with rheumatoid arthritis were recruited to a survey containing demographic and disease-related questions as well as a discrete choice experiment to measure their preferences for second-line therapies using biologics or Janus kinases inhibitors. Treatment characteristics included were route of administration, frequency of use, probability of mild short-term side effects, probability of side effects changing appearance, probability of psychological side effects, probability of severe side effects and effectiveness of treatment.

Results: A total of 358 patients were included in the analysis. A latent class analysis revealed three preference patterns: (1) treatment effectiveness as the single most important attribute, (2) route of administration as the most important attribute, closely followed by frequency of use and psychological side effects and (3) severe side effects as the most important attribute followed by psychological side effects. In addition, disease duration and mild side effects influenced the patients' choices.

Conclusion: Respondents found either effectiveness, route of administration or severe side effects as the most important attribute. Patients noting effectiveness as most important were more willing than other patients to accept higher risks of side effects.

http://dx.doi.org/10.1186/s13075-020-02391-w

Patient perspectives regarding gene therapy in haemophilia: Interviews from the PAVING study

van Overbeeke, Eline; Michelsen, Sissel; Hauber, Brett; Peerlinck, Kathelijne et al., Haemophilia, 2020, Volume 27, pp 129–136

ABSTRACT

Introduction: Exploring patient perceptions regarding gene therapies may provide insights about their acceptability to patients. Objective: To investigate opinions of people with haemophilia (PWH) regarding gene therapies. Moreover, this study aimed to identify patient-relevant attributes (treatment features) that influence PWH's treatment choices. Methods: Semi-structured individual interviews were conducted with Belgian PWH, types A and B. A predefined interview guide included information sections and open, attribute ranking and case questions. Qualitative data were organized using NVivo 12 and analysed following framework analysis. Sum totals of scores obtained in the ranking exercise were calculated per attribute. Results: In total, 20 PWH participated in the interviews. Most participants demonstrated a positive attitude towards gene therapy and were very willing (40%; n = 8) or willing (35%; n = 7) to receive this treatment. The following five attributes were identified as most important to PWH in making their choice: annual bleeding rate, factor level, uncertainty of long-term risks, impact on daily life, and probability that prophylaxis can be stopped. While participants were concerned about the uncertainty regarding long-term safety, most participants were less concerned about uncertainty regarding long-term efficacy. Conclusions: This qualitative study showed that most PWH have a positive attitude towards gene therapy and that besides efficacy, safety and the related uncertainties, also impact on daily life is important to patients. The identified patient-relevant attributes may be used by regulators, health technology assessment bodies and payers in their evaluation of gene therapies for haemophilia. Moreover, they may inform clinical trial design, pay-for-performance schemes and real-world evidence studies.

http://dx.doi.org/10.1111/hae.14190

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

van Overbeeke, Eline; Forrester, Valérie; Simoens, Steven; Huys, Isabelle, Patient, 2021, Volume 14, pp 119–128

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.

https://doi.org/10.1007/s40271-020-00449-0

Appraising patient preference methods for decision-making in the medical product lifecycle: an empirical comparison

Whichello, Chiara; Levitan, Bennet; Juhaeri, Juhaeri; Patadia, Vaishali et al., BMC Medical Informatics and Decision Making, 2020. Volume 20, Article 114

ABSTRACT

Background: Incorporating patient preference (PP) information into decision-making has become increasingly important to many stakeholders. However, there is little guidance on which patient preference assessment methods, including preference exploration (qualitative) and elicitation (quantitative) methods, are most suitable for decision-making at different stages in the medical product lifecycle (MPLC). This study aimed to use an empirical approach to assess which attributes of PP assessment methods are most important, and to identify which methods are most suitable, for decision-makers' needs during different stages in the MPLC.

Methods: A four-step cumulative approach was taken: 1) Identify important criteria to appraise methods through a Q-methodology exercise, 2) Determine numerical weights to ascertain the relative importance of each criterion through an analytical hierarchy process, 3) Assess the performance of 33 PP methods by applying these weights, consulting international health preference research experts and review of literature, and 4) Compare and rank the methods within taxonomy groups reflecting their similar techniques to identify the most promising methods.

Results: The Q-methodology exercise was completed by 54 stakeholders with PP study experience, and the analytical hierarchy process was completed by 85 stakeholders with PP study experience. Additionally, 17 health preference research experts were consulted to assess the performance of the PP methods. Thirteen promising preference exploration and elicitation methods were identified as likely to meet decision-makers' needs. Additionally, eight other methods that decision-makers might consider were identified, although they appeared appropriate only for some stages of the MPLC.

Conclusions: This transparent, weighted approach to the comparison of methods supports decision-makers and researchers in selecting PP methods most appropriate for a given application.

https://doi.org/10.1186/s12911-020-01142-w

Patient Centricity in Patient Preference Studies: The Patient Perspective

van Overbeeke, Eline; Vanbinst, Inès; Jimenez-Moreno, Aura Cecilia, Huys, Isabelle, Frontiers in Medicine, 2020, Volume 7, Article 93

ABSTRACT

Objectives: A factor contributing to the value of patient preference studies is patient centricity. This study aimed to explore how patients want to be involved in the design and conduct of patient preference studies. In addition, we investigated patients' expectations regarding the communication of study results back to patients.

Methods: Semi-structured interviews were conducted with patient representatives within three different disease areas: rheumatic diseases, cancer, and neuromuscular disorders. For each disease area, interviews were conducted with interviewees from Belgium, the Netherlands and the United Kingdom. Interviews followed a predefined interview guide covering topics relating to timing, level, and requirements for patient involvement in patient preference studies, as well as communication of results. Interviews were audio-recorded, transcribed and analyzed using framework analysis in NVivo 12.

Results: A total of 14 interviews were conducted. Some interviewees believed that patients should be involved in all steps of a patient preference study. Patient involvement seemed most valuable during the design phase to support defining research questions and instrument design. During analysis, patients can be involved for optimal interpretation of results. Most interviewees mentioned that patient involvement should be on the level of advice or collaboration, not control. Interviewees expressed requirements for patient involvement relating to the knowledge of the involved patient, time investment, compensation and other incentives. Regarding communication of results, most interviewees wished to receive a brief and lay summary of the results, followed by a detailed explanation of both individual and average results accompanied by visuals.

Conclusions: Patient involvement in patient preference studies could increase question comprehension by study participants and ensure correct interpretation of results by researchers. Patients want to be involved as advisors or collaborators, and considering their personal situation as well as establishing agreements on roles, time involvement and compensation early on will result in a most optimal partnership.

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Design, Conduct, and Use of Patient Preference Studies in the Medical Product Life Cycle: A Multi-Method Study

van Overbeeke, Eline; Janssens, Rosanne; Whichello, Chiara, Schölin Bywall, Karin et al., Frontiers in Pharmacology, 2019, Volume 10, Article 1395

ABSTRACT

Objectives: To investigate stakeholder perspectives on how patient preference studies (PPS) should be designed and conducted to allow for inclusion of patient preferences in decision-making along the medical product life cycle (MPLC), and how patient preferences can be used in such decision-making.

Methods: Two literature reviews and semi-structured interviews (n = 143) with healthcare stakeholders in Europe and the US were conducted; results of these informed the design of focus group guides. Eight focus groups were conducted with European patients, industry representatives and regulators, and with US regulators and European/Canadian health technology assessment (HTA) representatives. Focus groups were analyzed thematically using NVivo.

Results: Stakeholder perspectives on how PPS should be designed and conducted were as follows: 1) study design should be informed by the research questions and patient population; 2) preferred treatment attributes and levels, as well as trade-offs among attributes and levels should be investigated; 3) the patient sample and method should match the MPLC phase; 4) different stakeholders should collaborate; and 5) results from PPS should be shared with relevant stakeholders. The value of patient preferences in decision-making was found to increase with the level of patient preference sensitivity of decisions on medical products. Stakeholders mentioned that patient preferences are hardly used in current decision-making. Potential applications for patient preferences across industry, regulatory and HTA processes were identified. Four applications seemed most promising for systematic integration of patient preferences: 1) benefit-risk assessment by industry and regulators at the marketing-authorization phase; 2) assessment of major contribution to patient care by European regulators; 3) cost-effectiveness analysis; and 4) multi criteria decision analysis in HTA.

Conclusions: The value of patient preferences for decision-making depends on the level of collaboration across stakeholders; the match between the research question, MPLC phase, sample, and preference method used in PPS; and the sensitivity of the decision regarding a medical product to patient preferences. Promising applications for patient preferences should be further explored with stakeholders to optimize their inclusion in decision-making.

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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, 2019, Volume 19, Article 189

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.

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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, 2019, Volume 10, Article 1009

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.

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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, Volume 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.

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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.

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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

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.

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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, pp 57–68

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.

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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

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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