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DOI:
10.1007/s40271-017-0222-3

Document Version
Peer reviewed version

Citation for published version (Harvard):
Raza, K 2017, 'Giving Patients' Preferences a Voice in Medical Treatment Life Cycle: The PREFER Public–Private Project', *Patient*, vol. 10, no. 3, pp. 263-266. https://doi.org/10.1007/s40271-017-0222-3

Giving patients' preferences a voice in medical treatment lifecycle: the PREFER public-private project

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Acknowledgement

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 EFPIA.

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This text and its contents reflects the PREFER project's view and not the view of IMI, the European Union, EFPIA, or the authors' respective organisation(s).

Words: 1,752

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 not only become increasingly important 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 [1,2].

In general, stakeholders (i.e. industry, regulatory authorities, health technology assessment (HTA) bodies, reimbursement agencies, clinicians and patient organisations) all agree about the importance of incorporating patients' preferences[1], 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 lifecycle, or into regulatory and reimbursement decision making processes regarding medical treatments. Important questions include: 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 is patient preference data from country to country?

[1] Roughly, preferences reflect patients' values and characterize the relative importance that patients associate with the expected benefits, possible harms and other aspects of treatment [3].

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 - a public private research initiative – has recently been launched to tackle these challenges [4].

1. PREFER: what is it?

PREFER is an acronym for the research project 'Patient Preferences in Benefit and Risk Assessments during the Treatment Life Cycle' [4]. 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 programme and the European pharmaceutical industry represented by EFPIA (the European Federation of Pharmaceutical Industries and Associations). The consortium of PREFER includes 33 partners: 10 academic institutions from different European countries, 16 pharmaceutical companies from the US and Europe, 4 national and international patient organisations, 1 HTA body and 2 small and medium sized enterprises, all adding their experience and perspectives to the project.

PREFER builds upon the experiences and outcomes of previous initiatives, e.g. from the Food and Drug Administration (FDA), European Medicines Agency (EMA), previous IMI projects like Pharmacoepidemiological Research on Outcomes of Therapeutics (PROTECT), European Patients Academy on Therapeutic Innovation (EUPATI), and the Medical Device Innovation Consortium (MDIC) [5-8]. Compared to these initiatives, PREFER takes a broader approach, engaging competences and perspectives from a wide set

of stakeholders. It is focused on formal requirements assessment from the perspective of each stakeholder group, case studies to inform practical recommendations on when and how to perform studies to elicit patient preferences, and how these can address the requirements and inform regulatory authorities, pharmaceutical companies, HTA bodies and reimbursement agencies.

2. PREFER: what are the aims, objectives and deliverables?

The main aim of PREFER is to strengthen patient-centric decision making throughout the life cycle of medicinal treatments by developing expert and evidence-based recommendations on how patient preferences should be assessed and inform decision making. To reach this main aim, PREFER is divided into 3 parts, each having its own general objectives.

Part A ('Patient preferences: why, when and how (i.e. methodology)') will (i) obtain insights from all stakeholders regarding their key needs, expectations, desires and concerns about the assessment and use of patient preferences in medical decision processes. Hereto a scope literature review, in-depth interviews and focus groups will be performed with all stakeholders from different countries until data saturation and validation has been reached; and (ii) identify and appraise qualitative and quantitative methods for preference elicitation, considering also educational and psychological tools (including serious games) that can be integrated into preference elicitation methods and understanding the drivers of preferences. Systematic reviews in combination with input from international health preference experts will be used to reach consensus about which methods for what key decision are suitable to be tested in Part B.

Part B ('Testing preference elicitation methods in clinical case studies') will use methods developed and identified in Part A to (i) test and evaluate several methods for preference elicitation in clinical case studies by focusing on different decision points in the

treatment development and approval process, covering a range of diseases (common to uncommon), age groups affected, taking into account the cultural and sociological diversity within the European Union, and (ii) conduct computer simulation studies to both contribute to smarter design of the case studies and exploring the sensitivity of preference studies. These case studies and simulations will be designed to address issues raised in the requirements gathering step in part A. The outputs will be evaluated together with stakeholders to ensure a 'fit' with their decision-making processes.

Part C ('Developing recommendations') will (i) generate recommendations on patient-preference elicitation to inform decision making during the medical treatment life cycle using the results from Part A and B of the PREFER project, and (ii) support the development of guidelines for the design, conduct, analysis and reporting of patient-preference studies.

3. PREFER: why is it necessary?

Over the last decade, the patient voice has become heard more often in e.g. research funding bodies, institutional review boards, technology appraisal committees, regulatory and HTA body assessment panels, reimbursement decision makers, and the development of outcome measures. Although some pharmaceutical companies perform patient preference research alongside their early development and registration studies, explicitly and deliberatively taking account of patient preferences is uncommon along medicinal product life cycle. Moreover, this patient preference research (qualitative and quantitative) may be used for internal company decision-making, but is not always shared with the regulatory and HTA/reimbursement bodies, or even published of in peer-reviewed journals. For patient-preference studies that are used within the medical treatment life cycle, standards on the design, conduct, analysis, and use of the findings in decision-making are lacking. This is

critical, since conducting patient-preference elicitation studies is often time-consuming, expensive, and may be burdensome to patients. Additionally, while one division of one regulatory agency, FDA Centre for Devices and Radiological Health, has issued some guidance on using patient preferences to support medical device applications, most industry, regulatory authorities, HTA bodies, and reimbursement agencies have key uncertainties regarding the validity, representativeness, and robustness of preference studies to inform deliberative decision making. What is currently missing is a shared understanding among all key stakeholders of (i) what constitutes a methodologically-sound patient preference study; (ii) how the results from such a study can be incorporated in the decision-making processes of industry, regulatory authorities, HTA bodies, and reimbursement agencies; and (iii) at which stage(s) in the product development process and life cycle this information can best be collected. Another current gap is the lack of information on how patient-preference elicitation should be conducted in different patient populations, with acute and chronic diseases, and with more common as well as less common diseases. The recommendations that will be developed in PREFER are intended to address these outstanding questions. These recommendations will likely result in improved adaptation of medical treatments to patients' needs and wishes which in turn will benefit satisfaction and health outcomes. Due to a strong joint interest in the topic of this project and in developing recommendations with broad acceptance numerous stakeholders provide contributions to this project via patients, regulatory, HTA / reimbursement and scientific advisory groups.

4. PREFER: what are the ambitions and impact?

A broad array of (combinations of) patient preference methods will be tested prospectively in several empirical and simulation case studies. The availability of large patient cohorts will enable us to test new methods or deviations from existing methods in a randomized manner,

by comparing well-known methods and newer ones. Based on discussion with stakeholders, suitable methods will be tested and their contributions to inform decision making will be discussed in recommendations adapted to the needs of all relevant stakeholders. The recommendations from PREFER are expected to lead to changes in practices, in that stakeholders will systematically consider whether a preference study would add value at key decision points in the medical treatment life cycle; if so, PREFER recommendations would be available to follow. Patients and patient organisations may become more involved into decision making at key decision points and develop closer relationships with industry, regulatory authorities, HTA bodies, and reimbursement agencies. Ideally, these bodies will systematically consider including the patient's perspective in their decision-making processes. The output will not only be used by these decision makers, but will also serve as an additional source of information to adjust or discontinue medical treatment development processes and inform health care providers and patients. Some particular insights expected are guidelines for identification of subgroups of patients with distinct preferences and an understanding of the existence of cultural and economic-based differences in preferences. Support for the communication of preferences for relative benefits and risks of medical treatment – all of which will help foster the implementation of patient-centred medicine.

In summary, the ambition of PREFER is to develop a systematic approach for considering the use of patient preferences across the medical treatment life cycle. The strongest demonstration of the value of PREFER will come from acceptance of its recommendations by all stakeholders. The ideal achievement of the PREFER project would be a global, harmonised approach to the use of patient-preference studies by industry, regulatory authorities, HTA bodies, and reimbursement agencies. The horizon after the PREFER project is a world where collecting evidence on patient preferences about the relative benefits and risks of medical treatments is considered systematically, alongside

traditional type of evidence such as efficacy, safety/adverse events, quality of life and economic evidence.

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