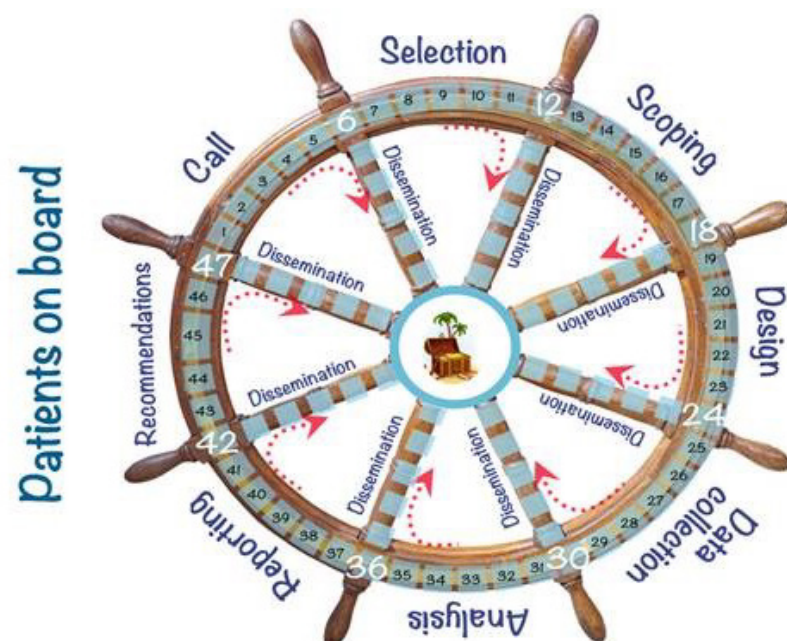


POSITION OF KCE ON PATIENT INVOLVEMENT IN HEALTH CARE POLICY RESEARCH



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IRINA CLEEMPUT, MARIE DAUVVIN, LAURENCE KOHN, PATRIEK MISTIAEN, WENDY CHRISTIAENS, CHRISTIAN LÉONARD



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Disclaimer:

- **The external experts were interviewed as part of the (qualitative) data collection for this study. They did not co-author the scientific report and do not necessarily agree with its content.**
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LIST OF ABBREVIATIONS

ABBREVIATION	DEFINITION
AAL	Active Assisted Living
ABPI	Association of the British Pharmaceutical Industry
AIDS – HIV	Acquired Immuno-Deficiency Syndrome – Human Immunodeficiency Virus
BS – MB	Belgisch Staatsblad – Moniteur Belge – Belgian Official Journal
CAAMI – HZIV	Caisse Auxiliaire d'Assurance Maladie-Invalidité – Hulpkas voor Ziekte- en Invaliditeitsverzekering
CADTH	Canadian Agency for Drugs and Technologies in Health
CBPAR	Community-based participatory (action) research
CEBAM	Belgian Centre for Evidence-Based Medicine
CHMP	Committee for Medicinal Products for Human use
CIHR	Canadian Institutes of Health Research
COPD	Chronic Obstructive Pulmonary Disease
EBP	Evidence Based Practice
ED	Early Dialogue
EEA	European Economic Area
EUPATI	European Patient Academy on Therapeutic Innovation
EJPRD	European Joint Program on Rare Diseases
EMA	European Medicines Agency
EOS	Excellence Of Science
EU	European Union
EULAR	European League Against Rheumatism
EUnetHTA	European Network for Health Technology Assessment
EUPATI	European Patients' Academy
FAGG – AFMPS – FAMHP	Federaal Agentschap voor Geneesmiddelen en Gezondheidsproducten – Agence Fédérale des Médicaments et des Produits de Santé – Federal Agency for Medicines and Health Products
FDA	Food and Drug Administration



FOG	Frailty Oversight Group
F.R.S. - FNRS	Fonds de la Recherche Scientifique
FTE	Full-time equivalent
FWO	Fonds voor Wetenschappelijk Onderzoek
G-BA	Gemeinsamer Bundesausschuss - German Federal Joint Committee
GCP	Good Clinical Practice
G-I-N	Guidelines International Network
GIPA	Greater Involvement of People Living with HIV and AIDS
GRADE	Grading of Recommendations, Assessment, Development and Evaluations
HIQA	Health Information and Quality Authority
HSR	Health Services Research
HTA	Health Technology Assessment
HTAi	Health Technology Assessment international
INAHTA	International Network of Health Technology Assessment Agencies
INNOVIRIS	Institut Bruxellois de la Recherche et de l'Innovation Sociale – Instituut ter Bevordering van het Wetenschappelijk Onderzoek en de Innovatie van Brussel – Institute for the promotion of scientific research and innovation in Brussels
IPF	Idiopathic Pulmonary Fibrosis
IQWiG	Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen
JLA	James Lind Alliance
KBF	King Baudouin Foundation
LUSS	La Ligue des Usagers des Services de Santé
NASH	Non Alcoholic Steatosis Hepatitis
NICE	National Institute for Health and Care Excellence
NIHDI	National Institute for Health and Disability Insurance
NIHR	National Institute for Health Research
OHTAC	Ontario Health Technology Advisory Committee



PAQS	Plateforme pour l'Amélioration de la Qualité et de Sécurité des Soins – Platform for the improvement of the quality and safety of healthcare
PCORI	Patient-Centred Outcomes Research Institute
PFMD	Patient Focused Medicines Development
PIP	Patient Involvement Programme
PPI	Public and Patient Involvement
PREM	Patient-Reported Experience Measure
PROM	Patient-Reported Outcome Measure
PRT	Patienten Rat & Treff
PSP	Priority Setting Partnership
RedETS	Spanish Network of Agencies for Assessing National Health System Technologies and Performance
SAHMRI	South Australian Health and Medical Research Institute
SAM	Subsaharan African Migrants
SIDA	Syndrome d'Immuno-Déficience Acquise - AIDS
TBM	Toegepast Biomedisch onderzoek met een primair Maatschappelijke finaliteit - Applied biomedical research with a primary societal finality
ULB	Université Libre de Bruxelles
US	United States
SMC	Scottish Medicines Consortium
SPOR	Strategy for Patient Oriented Research
UK	United Kingdom
VCS	Voluntary and Community Sector
VIVEL	Vlaams Instituut voor de Eerste Lijn – Flemish Institute for First Line Healthcare
VOKA	Vlaams netwerk van ondernemingen – Flemish network for enterprises
VPP	Vlaams Patiëntenplatform
ZonMW	Dutch Organisation for Health Research and Development



■ SCIENTIFIC REPORT

1 BACKGROUND AND SCOPE

It is increasingly recognized that involving patients in health policy research can be valuable. This idea is not new, but best practices for patient involvement are not yet very well established.

Patients are in a unique position to contribute an essential perspective to health policy research as they know what it means to live with the condition. Besides contributing to research as 'carriers of data', informing researchers about the symptoms and adverse events that matter most to them and have the greatest impact on their lives, patients may also contribute to aspects related to the research development plan. This would be more in the role of co-researcher than as unit of research.

However, it is recognised that patients or patient representatives also have or might have a conflict of interest. Transparency with respect to potential conflicts of interest is important, but does not take away the question of how to weigh the contributions of patients or patient representatives relative to other stakeholders' contributions in the research process.

Several agencies have developed experience with patient involvement, in several domains of health policy research: the Food and Drug Administration (FDA) and the European Medicines Agency (EMA) for assessments to prepare regulatory decisions regarding pharmaceutical products, several HTA agencies for preparing reimbursement decisions (CADTH, NICE, G-BA, HIQA ...) and also EUnetHTA for its joint or collaborative assessments. Belgium lags behind in terms of patient involvement in health policy research. KCE includes representatives of patient organisation umbrella organisations in its Board, and involves patients in some of its HTA or HSR reports, but not systematically. Patient consultation or consultation of the target population is required for submissions for the KCE Trials programme, but it has not yet been specified how this should be done. Involvement of patients in GCP guidelines is more established.



This position paper aims to explain KCE's position with respect to patient involvement in health policy research. Structural and organizational requirements supporting this position are also described. In a subsequent step, KCE will develop a process book for patient involvement, in line with the stated position. For this, it will be able to build on practical tools and guidance already developed elsewhere, e.g. by PARADIGM, ZonMw; INVOLVE, etc. (see chapter 11).

The focus is on the involvement of *patients* in research, i.e. people affected by the disease under consideration or their representatives, rather than lay people from the general public. However, for some topics, e.g. prevention, 'patients' can include people not (yet) affected by a disease.

Patient involvement in research is defined as doing research 'with' and 'by' patients who use services rather than 'to', 'about' or 'for' them. It could encompass, for example, involvement in the choice of research topics, helping to define the scope of a study, assisting in the design, carrying out the research, or actively disseminate the findings of the research projects.²

In 2012, KCE published a report on the feasibility and acceptability of different models for patient and citizen participation in health policy.³ The current position paper takes the findings of this project into account, in order to be in line with the stakeholders' views on the potential role of patients in their decision-making processes, but is not limited by it. As the focus is on patient involvement in *research*, which may precede decision making and inform it, the stakes may be slightly different than when we talk about patient involvement in decision-making processes.

To nourish our position statement, we performed a scoping review^a of the literature on patient involvement (see Appendix 1 for a description of the methodology), described a few patient involvement initiatives in research from other countries, and examined existing examples of patient involvement in research in Belgium. Because the position should be supported by the entire organization, we also measured the current patient involvement culture at KCE, i.e. the openness/resistance towards patient involvement, and assessed the extent to which patients have been involved in KCE research in the past.

^a A scoping review rapidly maps, summarizes and disseminates research findings in a particular area to inform future work.⁴⁴ Joss, N., A. Cooklin, and B. Oldenburg, *A scoping review of end user involvement in disability research*. Disabil Health J, 2016. 9(2): p. 189-96. The objective was not to

perform a full systematic review of the literature on patient involvement in healthcare policy research, but rather to learn about the definitions, rationales, methods, and impact of patient involvement in research. A classification of references according to these categories of interest is included in the Appendix.



2 DEFINITIONS

2.1 Involvement, engagement, participation

Several classifications of levels of involvement have been suggested in literature. Previous KCE research made a comprehensive overview of terms and definitions related to patient involvement in health policy decision making.³ From this, we learnt that the concept of patient involvement is poorly defined in literature. The term 'involvement' is used interchangeably with a number of other terms, such as participation, engagement or even empowerment, even if empowerment is rather a consequence of involvement than a level of involvement.

We decided to apply the terminology used by INVOLVE, an initiative of the National Institute for Health Research to structure patient and public involvement in research in the UK. INVOLVE defines involvement, engagement and participation as follows:^{5, 6}

- **Involvement:** Research which is done with or by patients and the public, rather than to, for or about them. It is an active partnership between researchers and patients and the public. Patients and the public are involved in key decisions throughout the research project life cycle.
- **Participation:** Where people give their informed consent to take part in a research study. For example, take trial drugs, try a new procedure or type of care, fill in questionnaires or be interviewed about their experiences. They are usually called study participants
- **Engagement:** Where we share information and knowledge about research with the public who we listen to and learn from as part of the process. The public can ask questions and debate results of the research.

The focus of this report is on involvement and engagement. Patient involvement in health policy research encompasses several intensities of engagement. We apply the operational definitions as developed by Hughes et al. for public involvement in research and adapted them to the more narrow focus of patient involvement:⁷

- **Targeted consultation** implies involvement where patients are contacted and consulted on specific aspects of the research study. They may be approached to provide feedback on a summary, or on the wording of a research survey or questionnaire; or to comment on or provide support for a research proposal. Typically the patients involved are already active in research or are members of patient groups with which the researcher has contacts. Targeted consultation is often limited to specific requests and tasks where patients are otherwise not involved. The patients involved may not receive much information regarding subsequent progress, outputs or impact.⁷ Targeted consultation can be about any aspect of the research process, from identifying topics for research, through thinking about the implications of the research findings.
- **Embedded consultation** is a level of involvement where patients are consulted regularly throughout the entire research process, from giving feedback on research ideas and proposals through to the dissemination of findings. Typically involvement includes patient representation on research steering or advisory groups; regular consultation with a patients advisory group or patient organisations; or methods of consulting with a range of people at different stages of a study. Typical for embedded consultation is that patients are consulted on a regular basis, and a range of methods are used. It is strengthened when involving a number of people with a range of views, experiences and perspectives and when not relying on one person or lay representative. In this model, the research team still has ownership and control over the research study but engages in meaningful consultation with others.⁷




- **Collaboration and co-production** implies involving patients in the research team, either as researchers/co- authors or as contributors to key decisions regarding research processes and findings. Typically this includes contributing to decisions about the tools used, choice and wording of research questions, how data are analysed, how research findings are presented and how results are implemented.⁷ Members may engage in the study in different ways depending on their areas of expertise and experiences but each role is given equal value and weighting.⁸
- **User-led involvement or coordination** implies that patients, academics and practitioners work together systematically across all areas of the research cycle. Patients are supported to take the lead in directing the nature and direction of a research study. Typically, people with lived experience are involved in generating ideas, proposals, funding bids, publishing and presenting the findings and are likely to be involved in conducting the research by interviewing participants or facilitating focus groups.⁷ The research is actively controlled, directed and managed by patients and/or patient organisations. Patients decide on the scope, research questions, design, planning and reporting.

Whilst advocates of user-led research consider this as a hierarchical ladder (similar to Arnstein's ladder of citizen involvement published in 1969), INVOLVE advises against viewing these approaches as hierarchical levels.⁹ More important is the quality of the relationships built between patients and researchers, parity of participation and impact of patient involvement. INVOLVE pleads for a flexible approach, whereby the value and relevance of different approaches to patient involvement is examined and different approaches can be applied in one project.^{6, 10}

If we integrate the contribution of Manafo et al.¹¹ to the conceptual literature on patient involvement, an interesting matrix can be constructed. Manafo et al. describe the role of the researcher for each patient involvement approach. Integrating this with the previous approaches leads to Table 1.

**Table 1 – Patient and researcher roles in research depending on the level of involvement (adapted from Manafo et al.¹¹)**

	Learn/Inform	Targeted consultation	Embedded consultation	Collaboration	User-led involvement
Patient	To ask questions and learn about how to get more involved	To provide feedback and advice on specific research activities	To work directly with a research team throughout the project	To partner on equal footing with researchers in all aspects of research	To make decisions and lead research activities
Researcher	To provide information, listen, and answer questions honestly	To seek your input on an ad hoc basis	To include patients as standing members of an advisory group	To partner equally with you as team members	To follow your lead and support your decisions
					
How can this be done?	Through orientation and information sessions, and media campaigns in an open atmosphere for sharing	Through scientific cafés, focus groups, priority-setting activities, and as members of ad hoc working groups or expert panels	Patients as members of standing working groups and research advisory committees	Patients as co-investigators and research partners, and as members of research steering committees	Through patient or community steering committees and patients as principal investigators

Source: Adapted from Manafo et al.¹¹



2.2 Patients, patient representatives, caregivers etc.

Most of the literature on patient involvement in research, considers both public and patient involvement. In this report, we focus on the involvement of patients, rather than the involvement of citizens or the general public. 'Citizens' refer to individuals selected to represent the interests of the wider community, whereas 'patients' refer to people with a legitimate, personal interest in a healthcare issue (e.g. use of a health technology, healthcare services).¹² The primary objective of this report is to support KCE's position statements about patient involvement **to improve the assessment of patient-related issues in its research**. More specifically, it wants to know how the quality of its research on patient-related issues (e.g. impact of a treatment on quality of life, patient priorities, etc.) can be involved by involving patients in decisions about the scope, design and execution of research projects. Citizens can be involved to learn about the aspects that are relevant for the society as a whole, but this is a different kind of perspective which might require a different approach to involvement.

We consider different groups of people that could represent patients in a research project, possibly in different roles (as experts, as patient advocates, as healthcare users):

- **Experts by experience**, i.e. individuals with a specific condition or having recovered from a specific condition, or having experience with the healthcare system (e.g. through pregnancy and delivery). These individuals have valuable experiential knowledge about a specific illness or condition or treatment.¹³ Even if patients are newly diagnosed and therefore do not have experiential knowledge about a disease, they can have valuable insights into the diagnostic procedure. Hence, patients do not have to be long-term patients. These patients speak on their own behalf when involved in a research process, based on their personal experience.
- **Relatives or informal caregivers**, i.e. (non-organisational, individual) representatives of patients as defined above, if the patients are unable to express themselves (e.g. caregivers, the parent of a sick child).
- **Patient-experts**, i.e. experts by experiences with knowledge about and expertise in scientific approaches for policy research (trained or acquired through frequent involvement in research projects).¹⁴ They understand the scientific language, know what is expected from them in the process of the research project, know how to accurately respond to queries and give input.
- **Patient representatives**, i.e. people who do not necessarily have the health problem, but speak on behalf of patients with a specific health condition. Depending on the context, this could also be people who bring the collective voice of specific, affected communities.¹⁵ They can make a significant contribution to understanding the patients' perspectives, especially in a context where patients are unable to communicate their values, needs, and preferences.¹⁶ There are different types of patient representatives: representatives of specific patient groups (e.g. cancer survivors, haemophilia patients) and representatives of patients in general (e.g. collaborators of an umbrella organisation of patient organisations).
- **Patient organisations** have been described as "entities that produce and mobilise knowledge about a condition (experiential and credential knowledge) to make things happen in their disease area".¹⁷ They have a membership of individual patients and/or relatives and/or informal caregivers. Representatives of patient associations are often advocates, defending the interests of the patient population they represent, but they can also contribute to a research process by bringing in the collective patient perspective, rather than their own exclusive experience, because they can consult their membership.¹⁸
- Belgium has three **umbrella organisations of patient associations**, like *Vlaams Patiëntenplatform (VPP)*, *La Ligue des Usagers des Services de Santé (LUSS)* and the *Patienten Rat & Treff (PRT)*. Their role is multiple: they can represent the interests of patients in general, but they also support representatives of their member organisations in their activities. As such, they can support or assist patient representatives in patient involvement activities, without actively influencing these patient representatives' perspectives or viewpoints.



- Representatives of **sickness funds** are considered to speak on behalf of healthcare service users, which can be patients suffering from a health condition but also general public using and paying for healthcare services.

In the position paper, we use the general term ‘patient’ to encompass all these different groups of people that can provide patient-relevant input in the research process, even though we recognize that the different types of patient representatives could play different roles. The identification of which type of representative should be involved in each stage of the research process will be further explored in the (yet to be developed) KCE process book.

2.3 Patient involvement versus qualitative research about patient-related issues

Patient involvement activities should not be perceived as being the same as or a substitute for qualitative research about patient-related issues.

In qualitative research about patient-related issues, patient's experiences are sought and used as data. Patient involvement means building up a partnership with patients in research, where their views and experiences contribute to decisions about, for instance, the research agenda, design, conduct and reporting of results.¹⁹ The main difference is thus that in patient involvement there is a two-way sharing of knowledge, views and perspectives within a research team that can contribute to what is studied and how it is studied²⁰, whereas in qualitative research, patients are the study participants providing data which are analysed by the researchers.

However, patient involvement and qualitative research can be combined, and the same patients can be involved in both activities. When the involvement consists of targeted or embedded consultation, for instance, qualitative research techniques can be used to collect viewpoints of patients involved in the research process.

An interesting example is provided by Morgan et al. (2016), in a study about involving harder-to-reach populations as research partners in health research.²⁰ They use a multi-disciplinary mixed methods approach, where

they combined patient involvement and qualitative research -with some overlap- to inform the design of incentive trials for smoking cessation in pregnancy and breastfeeding. It was found that women involved voiced different perspectives from those captured within the qualitative dataset. Patient involvement in the qualitative research design helped to interpret systematic review findings and construct vignettes for use in the qualitative data analysis. Involvement of harder-to-reach women assisted with recruitment to improve sample diversity in the formal qualitative dataset and with the translation of theory and findings presented in a researcher generated logic model into a lay tool.²⁰ However, it should also be noted that the researchers experienced overlap and movement between patient involvement and qualitative research, with groups or individuals playing multiple roles. Some participants were on the one hand influencing research decisions, making substantive contributions to research processes, interpreting results as part of the patient involvement activities and on the other hand generating data as part of the qualitative research activities. The authors concluded that this created “a need to negotiate boundaries between the researchers and the researched where the lines between objective observation and subjective participation required careful consideration”.²⁰

The key similarities and differences between patient involvement and qualitative research as identified by the authors are presented in Table 2.



Table 2 – Key similarities and differences between public and patient involvement (PPI) and qualitative research according to Morgan et al.²⁰ – emphasis added

	Similarities	Differences
Why	Both PPI and qualitative research aim to incorporate deeper understanding of the research problem and ensure greater relevance of the findings to society. Both were used to gather information to help in the design of an intervention and potential clinical trial.	PPI involves non-researchers and non-clinicians in research to inform study design and conduct. Qualitative research involves collecting data from participants to answer the research question(s).
Who	Both PPI and qualitative research can include representatives of the target population of the study.	PPI might only include representatives of patients or the public in general rather than the target population and representatives might be trained in PPI . PPI representatives are usually fewer in number than researchers or research participants. Qualitative research might seek to include broader perspectives and disconfirming data from as diverse a sample as possible.
What	Both PPI and qualitative research (with consent) can collect data using traditional methods such as recorded discussions, interactive sessions, and activities. Any collection of data for research purposes, audio-recording or subsequent use of quotations requires research ethics committee approval (http://www.hra.nhs.uk/).	PPI is predominantly involvement in the tasks of research and is a two way exchange of knowledge that influences study design, whereas qualitative research is predominantly for advancing understanding and thus involves the researchers being informed by the participants. Qualitative research requires research ethics committee approval whereas PPI usually does not.
Where	Both PPI and qualitative research can take place in a range of settings , including Universities or public spaces and either face-to-face, by telephone or using remote audio-visual technology.	PPI tends to involve inviting representatives to join research team meetings in academic settings , but can include researchers going out into the community . The setting for qualitative research takes into account participant preferences and where is best for the data collection.
When	Both PPI and qualitative research can involve single or serial interactions or meetings .	PPI is more likely to take place over an extended period and involve multiple meetings . Qualitative research is more likely to involve a one-time data collection session.
How	Both PPI and qualitative research might employ similar purposive sampling approaches to represent specific populations.	PPI is more likely to draw on established networks of people interested in contributing to research . Qualitative research designs vary based on the aims of the study, e.g. snowball, stratified, theoretical, and purposive and convenience sampling.

Source: Morgan et al.²⁰



3 RATIONALES FOR PATIENT INVOLVEMENT IN HEALTH POLICY RESEARCH

3.1 Reasons for involving patients in policy research

There might be various reasons or justifications for involving patients in health policy research.

NIHR director Davies stated:

*“No matter how complicated the research, or how brilliant the researcher, patients and the public always offer **unique, invaluable insights**. Their advice when designing, implementing and evaluating research invariably makes studies **more effective, more credible and often more cost-effective**.”⁵*

From the literature, we identified five possible reasons for patient involvement: democratic, legitimacy, scientific, instrumental and developmental reasons (Table 3).^{16, 21}

Table 3 – Reasons for involving patients in health policy research

Justification	Clarification
Democratic	Patients have the basic right to participate in research that might eventually impact their lives.
Legitimacy (procedural and content-related)	Patients and consumers should be involved with equal credibility as other experts and participants. This should lead to more informed, transparent and accountable decisions during the research project, incorporating social values and ethics, as well as patients' values, priorities, lived experiences, and relevant outcomes.
Scientific (instrumental)	Patient involvement can help to make better-informed, higher quality decisions during the research process. Patients contribute their unique perspective to the scientific process: e.g. formulation of informed consent form, use of instruments for data collection, choice of outcomes to investigate.
Developmental	Increasing public understanding of health policy research, and strengthening the public's and patients' capacity to contribute.

Adapted from OHTAC¹⁶, RedETS²¹ and the HTA Network Patients and Consumers Stakeholder pool²²

The **democratic** rationale describes patient involvement as a basic right of patients to participate in processes and decisions that may have an impact on their life.⁶ Participation is a value in itself, a moral principle and a right, besides strengthening the autonomy of patients, control and empowerment of those who will eventually be affected by the research.²¹

Legitimacy of health policy research is associated with procedural as well as content-related requirements. Procedural requirements include transparency of the research process, publication of the research procedures, opportunities for appeal and revisability of conclusions, enforceability of the procedural legitimacy conditions.²² Content-related requirements refer to the inclusion of relevant data and information.²² Patients help to legitimize the research process, e.g. by ensuring that the processes are transparent and understandable for patients or by using the



appeal mechanisms, but also help to legitimize the content of the research project by ensuring that patient-relevant aspects are taken into account in the design, conclusions and recommendations, verifying the basis for the conclusions and the way recommendations are drawn from the scientific evidence and formulated. The goal of patient involvement is in this case to improve the transparency and accessibility of decision processes during a research project in order to obtain more support for the eventual study conclusion.^{21, 23}

From a **scientific** perspective, the reason for involving patients could be to get their expert view on the scope, design, instruments used for data collection, etc. For example, informed consent forms are to be adapted to the cognitive abilities of patients participating in research, in order to ensure that patients really are informed.¹⁹ Patient involvement is in this case **instrumental** in the research process: they can improve the efficiency of the research process, the quality of the research and effectiveness of the communication of the research results.^{6, 21, 23}

The Patients and Citizens Involvement Interest Group of HTAi has performed extensive research on the underlying values of patient and citizen involvement in HTA around the world. Their conclusions are in line with the previous list of possible rationales, but are framed somewhat differently (Table 4). Relevance corresponds to the content-criterion of the legitimacy reason and the scientific reason for patient involvement, fairness and equity to the democracy reason for patient involvement and capacity building to the developmental reason for patient involvement in Table 4.

Table 4 – Values for patient involvement in HTA according to the Patients and Citizens Involvement Interest Group of HTAi²⁴

Value	Clarification
Relevance	Patients have knowledge, perspectives and experiences that are unique and contribute to essential evidence for HTA.
Fairness	Patients have the same rights to contribute to the HTA process as other stakeholders and have access to processes that enable effective engagement.
Equity	Patient involvement facilitates those affected by the HTA recommendations/decision to participate in the HTA; contributing to the transparency, accountability and credibility of the decision-making process.
Capacity building	Patient involvement processes address barriers to involving patients in HTA and build capacity for patients and HTA organizations to work together.

Source: <https://htai.org/interest-groups/pcig/values-and-standards/>

Other classifications of rationales have been described in literature (e.g. Smith et al. 2008²⁵). For example, some authors refer to the movement of **consumerism**, where patients get more choice about how their care is provided, and service providers are more responsive to these choices. This movement has stimulated community involvement, and community-based participatory research. Other rationales emerge from the movement towards patient-centred care, and growing public concerns and expectations about research.²⁵ In **community-based participatory action research** the rationale is to increase effectiveness and sustainability of community/public health interventions by involving the beneficiaries of the interventions.²⁶ In this case, patient and public participation is closely related to **empowerment** of citizens and patients.



A few critical side-notes should be made. Firstly, as Tritter states it: “Involvement is supposed to promote self-efficacy, develop social capital and create accountability, but there is little published evidence of the impact that involvement activities have made on those involved, or on the delivery, or outcomes of healthcare services”.²⁷ This means that many of these rationales are “common sense”, but little evidence exists on the actual impact for patients.

Secondly, the involvement of patients in health policy research requires a normative framework that translates into specific rules and procedures, to avoid that the involvement of patients becomes purely cosmetic or tokenistic.²¹ Hence, believing in the possible value of patient involvement in health policy research might not be sufficient to realise effective and meaningful patient involvement.

3.2 Goals of patient involvement in health policy research

Involving patients in health policy research might have multiple goals. The concrete goal often depends on the research phase. Research phases are roughly the same across policy study types, be it health technology assessment (HTA), health services research (HSR), good clinical practice guidelines (GCP) or clinical trials, even though some phases might be more relevant or elaborated for some types than others. Table 5 gives a snapshot of possible goals of patient involvement by research phase described in the literature. This is not an exhaustive list but gives at least an idea of why patients could be involved in each research phase

Table 5 – Goals of patient involvement by research phase

Research phase	Goal of patient involvement
Identifying research topics	Identify research topics important to patients ²⁸
Prioritising research topics	Match to patient priorities ²⁹ Help to ensure the research proposed is ethical ²⁸
Scoping	Enriched understanding of health issues, especially politicized and sensitive issues ³⁰ Clarify the research question and affirm its importance ^{28, 30} Define patient-relevant outcome measures ²⁸ Enhanced local ownership of research enhances community or organizational readiness to implement research protocol ³⁰
Design	Improve the study design, recruitment, data collection procedures and analysis, data interpretation and translation, and dissemination of clinical trials. ²⁹⁻³¹ Ensure the methods selected are appropriate for patients ²⁸ Aid in designing a detailed protocol ²⁸
Data collection	Create/adapt/review instruments for data collection ²⁸ (e.g. readability, understandability, but also adaptation of instruments that are culturally sensitive to the target population ³²) Assist in writing the patient information and consent forms ²⁸ Define appropriate/feasible timing and frequency of data collection for potential participants ³³ Assist in creating a recruitment strategy ²⁸



	Improve representativeness of the sample in clinical studies (patient involvement might help to increase awareness and participation of underrepresented groups + increase retention in longitudinal studies) ^{33, 34} Assist in conducting interviews and surveys ²⁸
Data analysis	Provide the context, which helps to carry out the technical role of statisticians effectively (meaningful effect size, issues with measurement of variables, confounding, potential predictors of an outcome (important for model building)) ^{30, 33} Assist the research team in developing themes from data ²⁸ Enriched interpretation of quantitative and qualitative research results from the integration of multiple perspectives ^{28, 30}
Reporting	Advise on the appropriateness of the reporting ²⁸ Ensure more meaningful and understandable reporting for patients and the community ³⁴ Produce summary of findings ²⁸
Recommendations	Advise on the appropriateness of the recommendations (feasibility, acceptability, formulation...) ³⁰
Dissemination	Increase acceptance of the findings of research ³⁵ Advice on different avenues for disseminating results ^{28, 30} Jointly present the findings with researchers ²⁸ Write information for local patient groups/hospitals/... ²⁸ Assist in getting results published on relevant organization web-sites ²⁸ Help distribute results within their informal networks ²⁸

There has been some debate in literature about the appropriateness of the involvement of patients for some of these purposes. For example, Sandman et al. (2017) state that for the identification of the relevant outcomes that need to be studied or the relevant patient-related aspects of a health policy problem, published patient-based evidence should be used rather than patient involvement.³⁶ One reason is that there might be disagreement between patients of the same patient group regarding the relevant aspects to be included in the research design or objectives. Patients are diverse and differentiate with respect to their preferences and perspectives. A second reason is that patients' preferences are by definition subjective. There is no clear answer as to how these subjective preferences should be weighted against other aspects that need to be taken into account in a research project. A third reason is that the effectiveness of a policy research project, in terms of drawing clear conclusions and formulating clear-cut

recommendations, might be reduced by the inclusion of subjective patient perspectives and preferences.



4 PHILOSOPHICAL AND ANTHROPOLOGICAL REASONS FOR TAKING THE PATIENT VOICE INTO CONSIDERATION IN HEALTHCARE RESEARCH

As discussed in the previous chapter, there are a number of instrumental or pragmatic reasons for involving patients in health(care) and policy research, be it at the level of fundamental research or applied research. This chapter focusses on the philosophical and anthropological reasons for patient involvement in health policy research – reasons that could be described as ontological. Indeed, beyond any “useful” character of this involvement, any researcher or decision-maker should ask himself what forces him to take account of what the human being, called “the patient”, thinks.

The reasons we will discuss concern philosophical and ethical issues about which the person inevitably has an opinion, a feeling or convictions. The expression of these, if taken into consideration, will have an impact not only on the organisation or financing of healthcare but also on the conception of the human being. So what needs to be done here is to think about what the patient is telling us about the human being when he expresses himself.

The themes we are proposing to tackle are complex and have already been the subject of numerous works, since time immemorial. We will therefore not aim to deal with them exhaustively but rather to recall them and clarify the ways in which they draw on the definition of the human being.

We will broach three questions, three logical conceptual connections, that also represent three ways of referring to the human being when we talk about “care” and about the “what-for” of patient involvement:

- The first connection will be the one that interconnects **freedom**, **responsibility** and **merit**; we will attempt to clarify these concepts and the links they have in the field of health.
- The second connection will take a look at the **normal** and the **pathological**, which are also at the root of the understanding of the human being.
- Finally, and logically following on from the foregoing, we will tackle the dual connection between **recovery** and **improvement**.
- When a person, as a potential patient, expresses himself in respect of these three logical connections, he is not just providing us with information about how to view a care system; he is telling us how he conceives the human being.

It will not be a matter here of taking a stand in favour or against a particular ethic but of setting forth the existing tensions and the underlying challenges behind any philosophical position concerning the themes raised and the way they are connected.

4.1 Freedom, responsibility and merit

The first connection between freedom, responsibility and merit does not affect solely the field of health or healthcare, but it is particularly constitutive of ethical debates in this sector which concerns us all. As the French philosopher Henri Bergson stressed, “Defining freedom would be a very difficult task if you did not want to side with a theory”^{b,37}. So it was that the philosopher preferred to devote his course to the “problem” of the freedom which arises when we think about the feeling of freedom we are filled with when we act. A priori we feel we are taking action freely, since our will is acting: this is the moment of “immediate awareness”, according to Bergson. Then, our “reflective thinking” will cause that much talked-about problem of freedom to appear, forcing us to ask ourselves what it really consists of.

^b Op.cit., p.17



Freedom occupies an important place in economic theory and in philosophy. According to the neoclassical economic approach, freedom is essentially postulated, that is to say we do not seek to find proof of its existence. The incentive-based logic prevailing in it needs the economic player's freedom but the latter is a "datum" within the model.

Health and healthcare are among the fields of human activity in which the question of the existence of freedom is highly pertinent. In fact, the recognition of, on the one hand, the existence of, and, on the other hand, the outlines of the person's freedom, will inevitably determine his possible assumption of responsibility. As Amartya Sen recalls, "freedom is the necessary and sufficient condition for responsibility"^c and "responsibility demands freedom"^{d,38} It is not a question here of establishing the state of the issue with regard to freedom, the very existence of which is the subject of diametrically opposing positions. Indeed, discoveries in physiological and cognitive science may lead to positions that reduce the human act to predetermined responses. For Henri Atlan, "free will is located in holes in determinism and, under the influence of new knowledge, the field of determinism is expanding and the holes are contracting"^{e,39} But we may also, on the other hand, reckon that we "always have the choice".

We all daily experience situations in which we are faced with choices to be made and this experience can be painful, not only because choosing means renouncing, but also and perhaps primarily because it confronts us with the need to discover what we really want deep down inside. In our search for what we are, we are overcome by a feeling of perplexity to the point perhaps of wondering whether what we think we have found in us is really us or if it is the result of a series of stimuli, pieces of information or impressions that are conveyed to us, not to say imposed on us. This internal search confronts us with the limits of our freedom, its outlines, its content, its essence, and its very existence. It is truly "freedom as a being" that is "at stake", fundamental freedom, freedom that is possible for every individual but which is very often

reduced to a weakened potential. It is not just about the freedom to speak or act that we think we have and control when we speak or take action. When you really think about it, that freedom is not necessarily in line with the words and actions that are supposed to render it. In other words, it is not because we express ourselves that we convey our true will or give expression to our fundamental freedom. We know that we are objects and victims of a form of alienation exerted by the prevailing paradigms. The issue of true freedom is already raised with regard to the way we think – that process that seems to us to be particularly natural and that we are convinced is "ours", that it belongs to us and that there, in us, in our mind, we are the masters of the situation.

If we take the examples of the concepts of "health" and "good health", can we claim that they are totally personal to us? If we want to characterise health and list the criteria to be evaluated in order to define it, do we make totally independent choices? We will very quickly be influenced, even insidiously, by images conveying the concept of health and the criteria to be evaluated will be physical appearance, physical strength, activity, hair, eyes, etc. These different criteria will then have to be met in a very particular way for it to be admitted that one's health can be described as "good". Outward appearance will have to meet the canons of beauty of the day, whilst physical strength will have to be substantial and should ideally find expression in leisure activities.

In this respect it is symptomatic that health surveys consider physical activities beneficial to one's health to be solely leisure-time activities. Thus a job involving physical, manual work will not necessarily be regarded as an activity beneficial to the person's health. There is no point in giving endless examples of "formatting" and alienation to reveal this need to rediscover, extend and rebuild our freedom. This is a (re)construction that calls for a veritable process of ontological liberation. This ontological freedom is neither total nor specific: it is not total since we need to take account of certain

^c Op.cit., p.372.

^d Op.cit., p.371.

^e Op.cit., p. 25.



chance circumstances and some form of determinism, particularly that of heredity and genetics but also that of education and the society in which one is born. This is perhaps where we find the greatest difficulty that the individual may encounter when, on being faced with himself, he wonders about what he really is. Let us imagine that a situation of original freedom exists, a state in which an individual has arrived at an age of reason “unsullied” by various influences. What might this “original freedom” consist of? In the absence of references, constraints or restrictions of any kind, freedom is devoid of meaning, content and dimensions. It is precisely through confrontation with external stimuli that the individual will be able to appraise what he thinks is his freedom, which in fact is not ontological in the sense that there were to be an original state in which it “would be”. His freedom is ontological, or at least could be or could become ontological, through a constant reflection enabling the questioning of any external discourse and the adoption, at a particular moment, of an attitude of acceptance, rejection, distrust, vigilance or lucidity that will open the door first to a thought and then to a word or deed, which will convey the “freedom” that has been built. This means that this freedom is built on a day-to-day basis, that it is not subject to rules and that what we do with it is not frozen in time. The reality of the world has become accessible to us in real time but we are no longer able to trust our senses; the reality we take in does not exist. It carries us away into dreams of power, youth and pleasure that slip further away from our grasp the greater our efforts are to make them come true by working long hours or devoting ourselves to moulding the body beautiful.

So ontological freedom will never be total and it will rather be a matter of “maximising” it albeit without dreaming of an inaccessible absolute. This freedom is not more specific or isolated since we all share one and the same humanity, i.e. it is not entirely specific to us. It is customary to refer to human finiteness, suffering and vulnerability to cite this community of humanity. We may also recall, as does Mylène Botbol-Baum,⁴⁰ that this equal humanity also stems from the fact that we all came into the world by the birth given

from a woman^f. However, for the philosopher, we are brought into life beyond that biological event. Above all we have to remember that we have been “created before becoming the creators of our own biography”⁹. So we can say that we share an ontological freedom that we need to make visible and effective in order to become a “subject”.

On this path of ontological liberation, the construction of the “self” is neither exclusively an innate, spontaneous or automatic phenomenon nor exclusively the result of a kind of social constructivism according to which we would not be able to develop a “self” outside interactions with others.⁴¹ The construction of the “self” would rather occupy an intermediate position which leaves room for internal reflection and for the influence of the phenomena of social life. The picture we will paint for ourselves of “good health”, even the notion we will forge in our deepest being, will probably never be completely disconnected from our environment. For example it will never be possible for us to experience all limitations, handicaps and illnesses, and only some of these situations will constitute our own actual experience. Nevertheless, this experience enables us to feel the physical and mental suffering that characterises frailties. Another part is “experienced” through others, for whom we are either objective onlookers carrying out a “cold analysis” or empathetic observers “living through” a “hot analysis”. This body of experiences enables us to delimit the contours of what is “good health” and the degree to which illnesses and diseases whittle away its contours.

The question of freedom has thus been raised, in condensed form. It is specific enough to everyone, whilst at the same time being universal, to justify recourse to patient involvement when the state of this freedom needs to be ascertained, in particular in the field of health. As we mentioned above, there is a close and nuanced relationship between freedom and responsibility; the will to have the patient assume his responsibility with regard to his health or the way to recover health should ideally take account of the above-mentioned nuances. Indeed, if we wish to adopt a fair patient accountability, in which “equals” are made equally aware of their

^f Op.cit., p.38

⁹ Op.cit., p.39.



responsibilities, these equals have to be equal in terms of real freedom. Beyond the many empirical studies attesting to a socio-economic gradient in terms of health, healthcare and risk behaviours, we need to ask ourselves about what patients have actually experienced, and the way in which they perceive their freedom. This does not mean that this freedom is frozen; it will in all probability be possible to make it evolve but it is not certain that incentive-based logics or nudging^{42, 43} will suffice for this. A certain form of care could be more fruitful in this respect.⁴⁴⁻⁴⁸

To conclude as far as this first linkage is concerned, we cannot leave out its third term, that of merit and meritocracy. As long as the patient is postulated as free, he is responsible by the yardstick of this freedom. He could also be deemed to be “deserving”, or consider himself as deserving (or not) his pathology and/or his care. Without tackling possible measures that would attempt to apply this concept of merit if only for instrumental purposes, i.e. not to “penalise” but rather to “educate”, it is essential that we pose ourselves the question as to how the patient experiences this situation. Whilst merit conceived of as compensation in kind or financial compensation for an effort made has already been the subject, implicitly or explicitly, of numerous publications⁴⁹⁻⁵⁶, merit conceived of as the result of an action not requiring anything in return, a free and disinterested action that alone “would deserve” to be asserted,^{57, 58} is less frequent. However, this conception could prove to be fruitful and should again be the subject of an investigation among patients.

4.2 The normal and the pathological

We have already mentioned the importance of real freedom in order to tackle the concepts of health and good health. Implicitly this question touches on the issue of the boundary between the “normal” and the “pathological”. We know that the possibility of offering personalised medicine not only seems

more and more probable but also more and more desirable. Indeed, by combining Big Data, artificial intelligence and genome interpretation, we can imagine a better dovetailing between a particular patient’s pathology and the treatment required. The debate about the possible aporias of this new medicine is beyond the framework of this text but we can nonetheless question the relevance of its “personalised” character. When we examine the concept of normality in terms of health we cannot help but quote Georges Canguilhem and his major work “The normal and the pathological”.⁵⁹ Canguilhem recalls that one can either adopt an “anxious” position towards health and illness and delegate to technology the task of restoring that health or, on the other hand, adopt a dynamic view as in the Greek medicine: a more totalising view based on the fact that man is harmony and balance and that a breaking of this balance is precisely what illness is^h. Illness is then an effort on the part of nature to obtain a new balance, with the organism making an illness in order to cure itselfⁱ. According to Canguilhem, the pathological phenomenon is only perceptible as such, that is to say as an alteration of the normal state, at the level of the whole of the organism and, in the case of man, at the level of the conscious individual in his entirety, where illness turns into a kind of harm^j. For man, being ill really means living on another life and what makes a symptom pathological is its relationship of inclusion into individual behaviour, such that the doctor is faced with a complete and specific individual and not with organs or their functions^k. Obviously, the patient may be wrong about “how” he is “someone else” or “different”, since he does not have the organism’s knowledge or science. However, this does not mean he is wrong when it comes to “in what way” he is “someone else”. He may sense that the pathology is not the mere quantitative extension of the physiological state, and that he is definitely “someone else”^l. Canguilhem evokes in particular the thinking of René Leriche^m for whom “health is the life of organs in silence” and “illness is what disrupts men in the normal course of their lives and in their occupations and

^h Op.cit., p.14.

ⁱ Op.cit., p.15.

^j Op.cit., p.64.

^k Op.cit., p.65.

^l Op.cit., p.66.

^m French surgeon (1879-1955).



in particular what makes them suffer”ⁿ. So it is again the patient’s definition that is important, since there can be disease, anatomical alteration or physiological disorder without the individual being ill. In that case, pain will be what reveals the illness. For Canguilhem, what is defined as “normal” is, in medicine as in philosophy, what is “usual” or what is “ideal”^o and the purpose of treatment is restoration to this usual state^p. He feels that it is because a state is regarded as normal by the patient that treatment will target it and try to reach it. Life is normative: it establishes norms due to the fact that the living being reacts to a problem through an illness and that this conveys the fact that the living being is not indifferent to the conditions in which life is possible. In fact, medicine provides a supplement based on human science to the body’s spontaneous effort to fight. Canguilhem also clarifies the concept of anomaly that should be considered as a purely empirical or descriptive concept, like a statistical discrepancy^q. An anomaly will only be known to science if consciousness perceives it as an obstacle, discomfort or a factor harmful to life^r. Canguilhem is in this way questioning us about forms of life. For him, the status of the anomaly sends us back to the general problem of variability of organisms, since “when living beings diverge from the specific type, we need to ask ourselves whether they are abnormal and are endangering the specific form or whether they are inventors on the road to new forms”^s. He explains that the living being and the environment are not normal when taken separately, but that it is their relationship that makes both one and the other normal: “a living being is normal in a given environment insofar as it is the morphological or functional solution found by life to meet all the demands of the environment”^t. So, for Canguilhem, the normal is not so much the old form as the new form if it

finds the conditions for existence in which it will appear normative, thus relegating all the past forms. Another fundamental aspect of Canguilhem’s thinking concerns the influence of the average on the norm. If human body is a product of social activity, it is not absurd to suppose that the constancy of certain traits, revealed by an average, depends on the conscious or unconscious loyalty to certain norms of life^u. Some populations in the world or some socio-economic, cultural or social categories may adopt customs according to specific circumstances of life. The physiological constants thus recorded reflect a certain average that could become a certain form of norm. We could then wonder whether the members of some groups do not develop “physiological indicators” corresponding to the life they lead^v or are forced to lead. This reflection brings us back to account being taken of differences in lifestyle and level of health according to one’s belonging to different socioeconomic classes before a state of health is declared to be “normal” or not. According to Canguilhem, it is therefore always the individual to whom reference should be made, because such an individual can be “adapted to the duties ensuing from the environment that is specific to him”, in organic conditions that would be unsuitable to those duties in the case of another individual^w. However, we must be wary of inferring from this that Canguilhem advocates a total relativity of the concepts of health and pathology; he accepts that the borderline between the two is vague for a large number of individuals considered simultaneously but it is perfectly accurate for one individual considered at different times^x. He adds that it is the inability to be normative that turns the individual into a patient^y. That is to say, it is his inability to adapt “gently” to different conditions without suffering far-reaching or abrupt ruptures. On the contrary, if he can adopt a different norm, it is in

ⁿ Op.cit., p.68.

^o Op.cit., p.101.

^p Op.cit., p.102.

^q Op.cit., p.109.

^r Op.cit., p.111.

^s Op.cit., p.117.

^t Op.cit., p.120.

^u Op.cit., p.135.

^v Op.cit., p.146.

^w Op.cit., p.155.

^x Op.cit., p.156.

^y Op.cit., p.160.



that sense that he is normative, while the patient has lost degrees of freedom. However, we cannot consider this new norm as a reduction, but rather as a positive experience of innovation on the part of the living being. Thus the illness is not a variation on the dimension of health but rather a new dimension of life, a new health that is not the same as the previous one^z. It is therefore deprivation and modification at the same time. For the individual it is a new life, characterised by new physiological constants and by new mechanisms for obtaining the apparently unchanged results^{aa}. To sum up, the pathological state may not be called abnormal in absolute terms, but abnormal in the relationship with a predetermined situation; being healthy and being normal are therefore not entirely equivalent since the pathological is a kind of normal. To be healthy is not only to be normal in a given situation, but also to be normative in this situation and in other situations that may arise^{bb}. What characterises health is the possibility of going beyond the norm that defines the momentary normal^{cc}. Being in good health is being able to fall ill and recover from it; this is a biological luxury^{dd}. Man only feels he is in good health (which is health) not only when he feels more than normal – i.e. adapted to the environment and its demands – but also normative, capable of following new norms of life^{ee}.

Canguilhem also feels it is medically incorrect to talk of diseased organs, diseased tissue or diseased cells; for every living being there is only illness of the organic whole^{ff}. It is as a “whole” that the biological datum can be termed diseased or not^{gg}. For Canguilhem, curing means giving oneself new norms of life, which are sometimes greater than the previous ones^{hh}. It is

firstly because men feel ill that there is a medicine. It is only secondarily that men know, because there is a medicine, in what way they are illⁱⁱ.

This detour we have made to take in Canguilhem’s thinking will have enabled us to note how important it is to refer to the patient in order to understand the concepts of health and pathology and the degree to which what is “normal” is relative. It will therefore come as no surprise that the means for restoring health are not subject to consensus, not only in time and space as regards the population, but also at the level of the individual.

Let us now tackle the third connection, which takes us to the far reaches of what is normal, not only for the individual but also for an entire population. What does caring and curing mean when the technical possibilities enable a change for the better to be brought about not only for the individual but also for the species? Once again, is it relevant to involve the patient in these kinds of issues?

4.3 Recovery and improvement

We have seen above how complicated it is to establish the distinction between the normal and the pathological. This difficulty is precisely the reason why the patient’s involvement is necessary, and also because of the specific nature of the concepts of health and illness. This being so, once we have agreed about the existence of a health problem, we need to consider the way in which it can be resolved. A priori, the health problem calls for adapted care in order to provide the person with a “normal” life and to

^z Op.cit., p.168.

^{aa} Op.cit., p.163.

^{bb} Op.cit., p.170.

^{cc} Op.cit., p.171.

^{dd} Op.cit., p.173.

^{ee} Op.cit., p.174.

^{ff} Op.cit., p.197. This could be challenged if one considers in particular the case of the 39-year-old female patient who received a lung transplant from a 57-year-old woman who had been a smoker for 30 years. The recipient developed lung cancer and died of it although she had not smoked. See [https://www.lungcancerjournal.info/article/S0169-5002\(18\)30486-0/pdf](https://www.lungcancerjournal.info/article/S0169-5002(18)30486-0/pdf)

^{gg} Op.cit., p.198.

^{hh} Op.cit., p.204.

ⁱⁱ Op.cit., p.205.



“restore” what needs restoring. Sometimes, returning to the pre-pathological state is not possible, and one has to “make do” with only completing part of the path between the pathological state and the pre-pathological state. This is the general context with which patients and care providers are faced on a daily basis. However, we need to consider another scenario, since technological development obliges us to do so. Indeed, “transhumanist”-type projects envisage the improvement of the human species, whether or not accompanied by a sometimes very substantial increase in life expectancy. For example, the founders of Google announce that they are going to “kill death” with the creation of the firm Calico^{jj}.⁶⁰ It is obvious that improved man, reinvented man or eternal man are all anthropological revolutions, in particular in the form of a cyborg liable to “give man the freedom to exist in other parts of the universe without the constraints to which he is subjected by the fact of having evolved on earth”^{kk}.⁶¹ When these various possibilities are considered, it goes basically about defining “the human”.

These questions are of sufficient importance for one to wonder about the processes that could enable them to be debated transparently in society. As soon as the risks have been identified, it is practically impossible to ignore them from that point on. The German philosopher Hans Jonas is very clear in this respect: “if it is a categorical imperative for humanity to exist, any suicidal staking of its existence is categorically prohibited, and the technical risks in which it is involved, even in a very uncertain way, are to be excluded from the outset”^{ll}.⁶² It could thus be put forward that there is a kind of collective responsibility for asking ourselves questions about the existence of these risks and discussing and clarifying them. However, these risks may appear in embryonic form in the researcher’s laboratory, and even if Jonas acknowledges that the lone researcher is no doubt overwhelmed by the

possible assessment of the consequences of his actions, he deems that it is precisely the consequences that above all entail a responsibility^{mm}. Everybody bears a sort of “parental responsibility” with regard to his discoveryⁿⁿ.⁶³ Technological advances do not just impact on our individual health, they can influence the way we view the human being and, in this respect, the researcher is not the only person involved; as human beings, we are all involved. Even if we do not necessarily have to imagine the worst in order to avoid it, as the French philosopher Jean-Pierre Dupuy would ask us to do,⁶⁴ it would be good for us to be part of a culture of permanent debates since all the possibilities that present themselves to man obviously contain questions it would be unwise not to raise.⁶⁵ Whereas the scientist has a role to play in formulating the problems and attempting to resolve them, he does not hold all the keys to success in doing so, particularly on the ethical front. For Jacques Testart, “the biologist can state the number and form of the cells making up the embryo, but this description is not appropriate when it comes to saying what the embryo is with regard to humanity”^{oo}.⁶⁶ Philosophers have long occupied themselves with this field of thought and their contribution is one of the most useful with a view to clarifying the way of involving the patient. According to Michael Sandel, the difficulty of distinguishing recovery and enhancement arises at the moment health is not accorded an intrinsic value, but only a contributory value making it possible to maximise happiness^{pp}.⁶⁷ He feels, moreover, that the advocates of genetic enhancement fail to perceive the moral difference between developing a child’s intellectual abilities through education, and enhancing them by means of genetic engineering^{qq}. For Olivier Rey, the transhumanist imagination is based on the fact that quantitative

^{jj} Op.cit., p. 37.

^{kk} Op.cit., p.78

^{ll} Op.cit., p.29

^{mm} Op.cit., p.36

ⁿⁿ Op.cit., p.11

^{oo} Op.cit., p.64

^{pp} Op.cit., p.38

^{qq} Op.cit., p.59.



developments can bring about qualitative leaps^{rr}.⁶¹ What is more, he recommends that focus not be placed on prospects of this type but rather on what is already being used and here and now calls for an ethical reflection^{ss}. It is not a question of renouncing technology or everything it can provide the human being with in order to reduce his suffering, but of comprehensively documenting, analysing and discussing the effects of its use, particularly when some people are wondering who “in the world of medicine could prohibit us from engineering the human genome with a view to correcting our flaws?”^{tt}.⁶⁸ There is no reason to suppose that technological development will exercise voluntary restraint or that its applications will be confined to restoring the human being’s health without ethical committees having looked into the prospects of improvement. As Luc Ferry stresses, to move from the realm of personal conviction to the law, and from subjective intuition to an obligation for others, reasons are needed that surpass our subjectivity; reasons that take into account the collective, the general interest or even universal values^{uu}.⁶⁹ One of the best ways of taking this collective into account is perhaps quite simply to involve the men and women who make it up, i.e. patients.

4.4 Concluding remarks

We have explored the three logical connections liable to give rise to issues of an anthropological nature. For each of them the importance of these matters is such that one can reasonably think that patient involvement could be fruitful. The result of the involvement should enable us to find out patients’ opinions as to their conception of the human being and therefore as to the best ways of inviting them to contribute to solidarity in the field of healthcare and to assign this care the role that it should consequently have.

^{rr} Op.cit., p.24

^{ss} Op.cit., p.37.

5 WHEN TO INVOLVE PATIENTS IN A RESEARCH PROJECT

At KCE, the annual research program is established after an open call for topic proposals. The received topic proposals are judged on their policy relevance, scope (in or outside the scope of KCE’s remit), and feasibility. Once a topic is selected for research, a project roughly consists of eight phases: scoping, design, data collection, data analysis, reporting, formulation of evidence-based recommendations and dissemination.

Several systematic reviews on patient involvement in research found that engaging patients in all research phases seems feasible in most cases.^{29, 34, 70} During the preparation of clinical trials, patients can provide input on, for instance, inclusion criteria, selection of methods and relevant outcomes, the informed consent procedure and materials. In the execution phase, patients can be involved in the recruitment of participants, the collection of data, the data analysis and the interpretation of findings. Patients can help in the dissemination phase, by involving them in the translation of findings to meaningful messages to their peers.⁷⁰⁻⁷³

Although the involvement of patients is feasible in all phases of health research in principle, the literature is mixed about the actual application of it in real life. Some reviews find that the involvement of patients remains often focused on providing input as consumer referees to protocols (design phase) or reviews (reporting phase) and in assisting with the provision of plain language summaries (reporting phase).⁷⁴ Others state that in practice, patient involvement occurs more often in the prioritisation phase of research topics, patient recruitment and dissemination phase.^{71, 75}, but involvement in the actual design and analysis of interventions is less common.⁷⁵ The European Patients Forum identified most involvement in HTA in Europe in the diffusion and dissemination phase, but much less in the identification and prioritisation of topics.⁷⁶ The opposite conclusion was drawn by Moran

^{tt} Op.cit., p.80

^{uu} Op.Cit., p.231



et al. (2011) based on internal HTA documentation, HTA agency staff interviews and a narrative literature review. They found that public involvement was present in identification and prioritization of topics for HTA, but absent in publication and dissemination.⁷⁷

Some populations (poor people, unemployed, low level of education, illiterate...) are usually considered difficult to reach. However, Domecq et al. (2016) found evidence that even in these populations, patient involvement is feasible.³⁴ Several examples of community-based participatory research (CBPR) focus on such these 'hard-to-reach' populations.⁷⁸⁻⁹⁵ CBPR is a collaborative approach to research engaging the multiple stakeholders, including the public and community providers, who impact, and are impacted by a problem of concern.⁹⁶

Feasibility is one thing, patients' preparedness and readiness to be involved is another. In 2012, the Patient-centred Outcomes Research Institute (PCORI) organized a workshop on how to conduct patient-centred research. The overwhelming interest from patients to participate in the workshop showed that patients are ready to be involved in research.⁹⁷ PCORI is a US-based institute. It is not clear to what extent their experiences with respect to patient preparedness to be involved in research can be extrapolated to Belgium. Besides the mere willingness and interest to participation, also the practical aspects need to be considered. Patients, or patient organisations also need the capacity to participate as partners in a research project.

Patient involvement may be more important for some topics than for others. For example, studies about the financing of hospitals might benefit less from patient involvement than studies about a specific health technology. The level of involvement may also vary depending on the topic. Tools and processes to support (the planning of) patient involvement need be developed. This will be the subject of the KCE process note on patient involvement, which will be developed later.

5.1 Identification and prioritization of research topics

Ideally, topics to be evaluated should take the needs, values and preferences of patients and/or citizens as a whole into account,⁶ not only because it makes the research more relevant for decision making and potentially more impactful, but also because health policy research is often financed through public resources. These resources are obtained from the public at large, hence it seems logical that the public has the right to contribute to the choice of topics to study.

To ensure that all populations are brought into the **identification** of topics for policy research, not only those individuals who identify themselves as 'patients', the call for topic proposals should be broad.⁹⁷ It has also been stressed by PCORI that patients who submit a topic proposal should be kept informed about the state of their proposal in the entire prioritization process. Transparency is important to mitigate disappointment on the part of patients involved. Both conditions are currently fulfilled by KCE, where all citizens, organizations or groups can submit proposals and those who have submitted a proposal all receive a decision letter with, if applicable, an explanation of why a topic was not retained.

An evaluation conducted in the UK by Oliver et al. (2009),⁹⁸ showed that involving patients and the public in the selection and **prioritization** of topics for health technology assessment (HTA) improves control and accountability for those who are affected by the decisions based on the HTA.⁹⁸ In addition, patients contribute to the relevance of the assessments by identifying technologies whose evaluation is most important for society and patients. Patients and citizens often have different and complementary visions to those of researchers and professionals on what issues are priorities for research.^{99, 100} Patient involvement in this phase is considered an ethical duty that rests on democratic arguments.⁷⁷

There are multiple examples of patient involvement in the prioritization of research topics in specific domains. A notable example of patient involvement in research priority setting is that of the James Lind Alliance. The James Lind Alliance is organized in Priority Setting Partnerships, bringing patients, carers and clinicians together to identify and prioritize shared uncertainties about the effects of treatment in specific domains, e.g.



kidney cancer, alcohol-related liver disease, autism and asthma.¹⁰¹ The list of uncertainties reflects the priorities for research in that particular domain. More information on the James Lind Alliance is provided in chapter 11.

The feasibility and challenges related to the methods used by the James Lind Alliance have been studied for the Pressure Ulcer Priority Setting Partnership.¹⁰² This case study showed that it is possible to work in partnership with patient populations consisting mainly of frail elderly, immobile patients with multiple co-morbidities. However, a few challenges were reported as well. First, there was less opportunity for direct dialogue and deliberation than initially hoped because this was the most expensive and time-consuming aspect of the process. Second, for some participants (patients/service users, carers and health professionals) the concept of 'uncertainty' was a difficult term for to get to grips with as a concept in treatment and research. Third, it was observed that all stakeholder find it difficult to acknowledge that some strongly held beliefs about wound care are actually research uncertainties. Fourth, the James Lind Alliance priority setting partnerships do not include health researchers because they are considered to have an own agenda and might therefore be biased. Yet the process involves a variety of research skills. Despite these challenges, it was concluded that the Priority Setting Partnerships do help to open the discussion and explore the gap between patient experience and health professionals' and researchers' understanding of what is most important to perform research on.¹⁰²

Others models for patient involvement in research agenda setting have been developed for other countries. For example, a Dialogue Model was validated for the Netherlands, including six phases: multi-stakeholder exploration, consultation, prioritization of research themes per stakeholder group, integration of different research agendas, programming and implementation.¹⁰³ Evaluation of the application of this model showed, however, that patients involvement in agenda setting is not automatically followed by patient involvement in programming and implementation.¹⁰⁴ More efforts are required from researchers to keep patients on board.

Similar challenges as the one identified by the James Lind Alliance Ulcer Priority Setting Partnership and the Dutch group testing the Dialogue model

were reported by the National Institute for Health Research's Collaboration for Leadership in Applied Health Research and Care for the South-West Peninsula: time constraints, variable quality of questions and initiating and maintaining engagement in the process.¹⁰⁵

The King Baudouin Foundation identified and described in more detail four success factors for research agenda prioritisation, based on experiences described in literature and a workshop held in Belgium:¹⁰⁶

- Thoughtful but flexible planning, defining the owner of priority setting exercise, establish a steering group or management team, map all the stakeholders and involve them, define the context and scale to set the boundaries of what is feasible and what is not (budgetary, geographically, etc), provide information (e.g. on scientific evidence already available) and collect data, plan and implement the priority setting dialogue.
- Broad consultation: start with homogeneous group consultation, followed by interim prioritization within the stakeholder group
- Integration and prioritization: facilitate mutual learning through dialogue
- Dissemination and implementation

A systematic literature review published in 2018 identified two additional highly structured patient and public engagement planning activities besides the James Lind Alliance Priority Setting Partnerships and the Dialogue Model: Global Evidence Mapping (application to Traumatic Brain Injury (TBI) and Spinal Cord Injury (SCI) in Australia), and the Deep Inclusion Method/CHOosing All Together (US).¹¹ This review identified the lack of evaluation data on the success and extent in which patients were involved as the major limitation of all four initiatives. Furthermore, issues relating to feasibility, coordination, communication and limited resources were identified.^{11, 107}

Many examples of research priority identification exercises have been published, in different patient populations, e.g. the elderly¹⁰⁸, forced migrants¹⁰⁹, in specific diseases, e.g. kidney disease¹¹⁰, mental illness¹¹¹, TIA and stroke¹¹², in a combination of both, e.g. young people with rheumatic



disease¹¹³, or in specific types of care, e.g. anaesthesia and perioperative care¹¹⁴, and organ transplantation¹¹⁵.

5.2 Defining the problem, scope, objectives and design of the study

Most evidence exists on the effectiveness of patient involvement in the stage of **defining the problem, scope, and objectives** of the study.¹¹⁶ Patients can report on experiences of living with the disease and on the barriers and facilitators to empower them in self-care (e.g. issues of adherence to treatment, deal with treatment costs, side-effects etc.). They can describe the impact of a disease and treatment on health outcomes, symptoms, physical and social functioning, costs, and quality of life, and explain the expectations and needs regarding a (new) treatment.⁶ Even though this contextualization cannot be used as scientific evidence as such, it might shed a different light on the issue to be studied.

An in-depth qualitative analysis of 25 study reports on public involvement during the development of applications for research funding in the UK found several reported benefits of patient and public involvement.¹¹⁷ Added value has been in terms of validating or adding to researchers' knowledge and perceptions of the intended research subject, developing the specifics of the intervention to be tested, ensuring that outcomes of interventions were of importance to patients, acceptability of data collection methods and tools, alerting to potential ethical or patient safety issues and advice regarding research processes (e.g. recruitment, drop out, follow-up).¹¹⁷ The same conclusions about the benefits of public involvement in the design phase were drawn from an earlier systematic literature review, although this review also highlighted challenges and barriers (see chapter 7).¹¹⁶

The importance of taking patient perspectives in terms of outcome measures to include in a study into account when designing a study, is highlighted by both the GRADE system and the Core Model for HTA of EUnetHTA.^{vv}

GRADE, which is a methodology used to develop evidence-based recommendations, requires a graduation of the relative importance of the outcome measures for which the input from patients is key.¹¹⁸ Patients can help to define the scope and identify the relevant research questions and outcome measures of specific interest.¹¹⁹⁻¹²¹ These might be different from those formulated by clinicians, agencies or governments.

Patient involvement in the scoping and design of a study might also be relevant for ethical review committees of study proposals. Staley et al. (2017) studied 2748 applications submitted to research ethics committees in the UK to assess to what extent the approaches to involvement support the review of the ethics committee.¹²² The percentage of researchers who reported involvement at one of the research phases varied from 10% (analysis phase) to 42% (dissemination phase). The assumption is that patient involvement can improve the ethical acceptability of a research project. However, the study found that researchers rarely describe any prior patient involvement in sufficient detail to allow the ethical review committees to confirm that the involvement made any difference for the research protocol or to assess whether it shaped the research design in any way to make it more ethically acceptable.¹²² Also, researchers' plans for future involvement are often not clear enough to enable research ethics committees to make a proper assessment of whether this involvement will be meaningful, or whether potential ethical concerns raised by involvement have been addressed.¹²²

Several examples of studies that involved patients in the scoping and design phase of a study have been described in literature. For example, Joss et al. described several examples from the literature on patient involvement in defining research questions in disability research⁴, McSharry et al. involved diabetes patients (type 1 and type 2) to prioritize target behaviors for research in diabetes.¹²³, Smith et al. (2008) established a service user reference group to refine the scope of a review on patient involvement in nursing, midwifery and health visiting research²⁵ and Davies et al. (2017)

^{vv} <https://www.eunetha.eu/wp-content/uploads/2018/01/HTACoreModel3.0.pdf>, accessed 26/07/2019



consulted rugby players for co-developing a player health study¹²⁴. The reported experiences were all in line with the conclusions of the reviews.^{116, 117} Benefits seemed to have outweighed the challenges.

5.3 Assessment of scientific literature and other sources of information, data collection and data analysis

Patients can help to contextualize and complete the information from the analysis of the conventional scientific evidence, since patients can identify the gaps or limitations of published **literature** in a given context.^{16, 125} Patients can explain why interventions that appear effective in clinical trials or in studies may not be so in real life. In that sense, patients could help in interpreting the literature.⁶

It is recommended, though, to triangulate the information obtained to reduce the risk of bias. Especially in those areas where there is no systematic review of the literature.¹²⁶ Triangulation implies the use of different data sources, researchers or experts and the use of different methodologies for the assessment, and confronting/comparing the results obtained via these different routes. To ensure transparency, it is necessary to clearly differentiate between the facts and the interpretations and to explain the perspectives from those who make such interpretations (patient, health professional, family, policy maker, researcher, etc.).²¹

Some aspects which are considered to benefit from patient involvement, should be studied by means of quantitative or qualitative research techniques, to obtain patient-based scientific evidence, i.e. evidence generated by means of scientifically validated and robust methods. This applies, for instance to aspects such as the acceptability of an intervention or the preferences for care options. Regarding qualitative patient-based evidence, the GRADE Group is developing a methodological tool to assess confidence in the results of Qualitative Evidence Syntheses (CERQual).¹²⁷

As for patient involvement in **data collection**, there are mixed opinions and experiences. Peer interviewing, for instance, might strengthen the collection of qualitative data and increase its relevance and reliability.⁶ However, also negative consequences of peer interviewing have been reported. Joss et al.

suggests that end-user control regarding the data collection tools is necessary to ensure that data collection tools are designed appropriately. If not, data quality will be impacted. For the data collection design, consultative or collaborative involvement of patients might be sufficient.⁴ Studies also reported challenges with recruiting patients from a diverse range (e.g. different ethnic groups) for data collection, and engagement in those patients who did agree to the involvement.⁷³

Davies et al. (2017) involved rugby players in developing data collection tools for a study on rugby players' health. The players readily engaged with the study and made many contributions to the development of the study questionnaire. They discussed whether topics were being collected satisfactorily, and whether the questionnaire encompassed the topics that were relevant for their playing experiences or that of others. They also reflected on the potential reliability of the answers, and ways to improve reliability. The choice of language, motivation for question inclusion and use of standardized versus novel measures were also discussed.¹²⁴

Shen et al. (2017) studied the literature about the involvement of parents as co-researchers in health research and found examples in literature of studies where parents were involved in different stages the research process, including also data collection, data entry and data analysis.¹²⁸

However, while it is important to involve patients in the design, content and use of the data collection tools, there is discussion about the appropriateness to involve patients in the actual data collection phase. Some authors provide evidence that supports the involvement of patients in data collection, e.g. as interviewer, because they are often able to elicit more sensitive information from participants⁴, others fear that this might reduce the scientific rigor.³³

Garfield et al. (2015) reported on the experiences of lay members with their involvement in collecting observational data in hospital data for research.¹²⁹ The lay members all reported that carrying out the observations had been an interesting and informative experience. However, some barriers and challenges were also mentioned, such as not having the infrastructure in place to support them in their lay research role, differing views on research governance held by the lay members and the researchers in relation to



consent procedures. The same group of researchers then described their experience with lay involvement in qualitative data analysis.¹³⁰ From their experience, there are fewer challenges in involving lay partners in the analysis of data than in involving them in the collection of data. As in other examples, it was found that lay members can bring in new perspectives and enhance the understanding of the qualitative data and thereby improve the data analysis.^{20, 130}

Patients can also be involved in the research process to give input on how to **recruit patients** for a study, by providing greater access to the research community and by identifying effective ways of accessing participants.⁷³ Jones et al. (2015) performed a literature search about the extent to which patients were involved in surgical research and found that patients' views on study acceptability and feasibility, and the comprehensibility of written information may help to improve the consenting and recruitment process.¹³¹ For clinical trials, randomization might be an important barrier. Patient involvement in the study design may shed light on the reasons for this fear for randomization in patients.¹³² Patients can also improve patient recruitment in clinical studies by, for instance, suggesting changes to the enrollment script, the use of an online option to enroll in an email link, requesting preferred follow-up times, and sending out reminders about the study.²⁹ Lee et al. described a study where these strategies increased enrollment from 65% to 95% of eligible patients, and completion of the study from 58% to 85%.²⁹

In community-based participatory research, involvement of patients or members of the target community is almost standard to improve the recruitment of study participants.^{86, 88, 133} Patients on the study team in that case serve as study subject recruiters and liaisons to the community. Also for research in hard-to-reach populations, patient involvement might help to define appropriate ways for recruitment.^{20, 109, 134}

5.4 Reporting the results of the study

Patients can take part in internal and external review of the study report.

Internal review is done by people who have been involved in the research project before. Involved patients can contribute for instance through assessing whether the context of the study has been well represented and highlighting possible information gaps.

External review of scientific reports is done by people who have not been part of the research group. Letting patients take part in the external review process can be particularly valuable when patients have not yet had the opportunity to contribute to the research project before.²¹ Patients external to the research can evaluate, for instance, the applicability of the results to a local context, if the study is mainly based on existing evidence from other contexts.

5.5 Formulation of recommendations

The involvement of patients in the translation of evidence into recommendations can contribute to the development of recommendations that are more in line with the experiences of patients and are formulated in an appropriate language and speak to patients' perspectives, needs and preferences.^{13, 16, 21, 135}

One example is that of the SHARED study, aiming at developing user-led recommendations around discharge from acute hospital care to community care for people living with undiagnosed memory problems or dementia and their carers. The patients and carers attended focus groups to shape and finalise the recommendations from the study.¹³⁶

Another example of how this can be achieved is provided by de Wit et al. (2011), who involved a core group of physicians and patients from different European countries in the elaboration of treat-to-target recommendations for rheumatoid arthritis in lay language.¹³⁷ During a one-day meeting, the group discussed, changed and reworded existing recommendations. Particularly interesting was that the group revealed a number of potential barriers for the implementation of the recommendations in clinical practice, such as



inequalities in arthritis healthcare provision across Europe. Such findings are important for policy purposes, and might not have been detected without the involvement of patients.

Berglas et al. (2016) examined the extent to which patient insights of were integrated in drug assessment reports and recommendations by the CADTH Drug Expert Committee between December 2012 and June 2014.¹³⁸ Patients can provide input, but do not co-produce the recommendations of the committee. The authors found that more than half of the insights provided by patients were highlighted in the recommendations documents. Of these, the majority was backed by scientific evidence. Insights provided by patients but for which no trial data were available, were sometimes highlighted as evidence gaps (e.g. data related to symptom relief, side-effects, avoiding further disease and mortality). A few recommendation documents (four out of 30), included patient insights, although that insight was without trial data and not included in the assessment protocol.

Blackburn et al. (2018) co-produced recommendations for public and patient involvement in primary care research with patients and members of the public.¹³⁹ They organized a recommendations workshop, which delivered 15 useful recommendations for public and patient involvement in primary care research.¹³⁹

A systematic review about the incorporation of patients' views in clinical guidelines found that 40 out of 56 institutions recommended the inclusion of patients in their guideline development processes. Of these, 35% recommended it for developing recommendations. However, very little guidance was provided on how to do this.¹⁴⁰

Based on other studies, we could conclude that, in the context of clinical guideline development, patient involvement in the recommendations is often limited to stakeholder meetings or a public meeting or online consultation where stakeholders can provide feedback and suggestions for alternative evidence that might be considered or alternative interpretation of the evidence.^{120, 140-142} The process of making recommendations, or dealing with the comments from patients is not always clear, which may leave patients who are involved with a bitter taste. This was found in a study on service user involvement in clinical guideline development for mental health services

in the UK, where mental health service users who had been involved in service guideline development groups were interviewed about their experiences.¹⁴² The study showed that the lack of clarity about how decisions are taken in the guideline development process and the formulation of the recommendations, made service users feel tokenistic ("...well what 's the point of me being here if that 's what you are going to do?").¹⁴²

5.6 Dissemination

Patients can play an important role in the dissemination of health policy research results.

Patients can be **consulted** about their preferred ways of receiving and learning about the recommendations formulated based on the research. This can be through existing research on dissemination preferences of patients or newly set-up Delphi panels or focus groups. Preferences relating to the format, styles and content need to be considered in the dissemination plan.²¹

If patients act as **collaborators** in the dissemination of recommendations of a policy report, they can help in formulating the recommendations in a patient- or public friendly language. Representatives of patient associations could help by publishing the report and/or its recommendations on their website and diffuse the messages by email to their members. Patients can also help in designing the surveys or Delphi panels to investigate dissemination preferences of patients.²¹

Patients could also take the role of **coordinators** of the dissemination strategy. In this approach, patients take a very active role in developing activities for dissemination, e.g. by producing articles about the study in newsletters, presenting the results in conferences, governmental commissions, etc. or even organizing workshops and training courses for patients.⁹⁶



A systematic literature review about patient and public involvement in health and social care research found that involvement of users may improve dissemination and implementation of research findings because of the dedication and influence of users in the community.⁷³ Patients who have been involved in studies can form a cohort of advocates for implementation and dissemination of results. Moreover, it was concluded that users were found to deliver more poignant messages at conferences and through newsletters by relating the findings to their own experiences and presenting them in a more lay user-friendly way.⁷³

In literature, there are several examples of patient involvement in dissemination activities.^{4, 29, 131, 143, 144}

Barnfield et al. (2017) created lay summaries of scientific articles about the links between dementia and stroke in collaboration with five members of the general public. The group selected the topics of most interest to the wider public and modified the language and layout of the lay summaries. For example, the lay members suggested to use a 'question and answer' style layout, add a glossary, and exclude scientific jargon.¹⁴⁴

Andrews et al. (2015) reported on a study where patients were –amongst others– involved to reflect on dissemination plans in the context of a grant proposal. They found that patients raised many points that the research team had not considered. The researchers adapted their dissemination and impact plan according to the suggestions.¹⁴⁵ Based on a review of 200 grant proposals and 181 projects, Blackburn et al. (2018) described the benefits and costs of patient involvement in dissemination activities. The benefits included the promotion of outputs when these take the form of training modules or toolkits and the guidance in terms of presenting the results in a format that is useful to non-researchers. At the cost side, they refer to the financial cost of patient-contributors attending conferences and external events.¹³⁹ Noteworthy is that while patient involvement was planned in 21% of the grant applications, less than 10 out of the 180 final reports reported actual patient or public involvement in dissemination.¹³⁹

Based on a literature review, a workshop with 32 university and 30 community partners and personal experiences, Allen et al. (2017) found that some models for translational research in primary care already incorporate patient involvement in the dissemination phase, suggesting that patient involvement in dissemination might increase the uptake of evidence-based recommendations.¹⁴⁶ In urologic oncology research, patient coinvestigators were also found to play a critical role in the presentation and dissemination of the study results.²⁹ Similar conclusions were drawn from another systematic review, which identified the impact of patient involvement on UK NHS healthcare services.¹⁴⁷ Information development and dissemination was regarded as an important area of service user activity. Patients were found to be involved in the production of public and patient information (newsletters, patient information leaflets, information directory), raising awareness of chronic conditions through community campaigns, and the development of training sessions for both patients and health professionals.¹⁴⁷ Evidence was found for different health areas, including mental health, cancer, colorectal cancer, stroke etc.

INVOLVE formulates a number of recommendations to encourage and support public involvement in dissemination, such as developing progress reports or newsletters to keep people informed throughout the project, reporting both positive and negative results, giving feedback on the results to all those who were involved, working with members of the public to develop dissemination plans, involving people in presenting at conferences, speaking to patients, and acknowledge the contribution of patients when writing the study report.⁶

Several examples of the possible role of patients or lay members in the dissemination of findings are also found in the field of community-based participatory research (CBPR)^{96, 148} and translational research^{149, 150}.

Becker et al. (2009) explored the viability of CBPR to disseminating empirically supported interventions to reduce symptoms and risk factors for psychopathology.¹⁵¹ With a case of dissemination of dissonance-based interventions for eating disorders, they demonstrated that CBPR and classic efficacy/effectiveness research are complementary for the success of the dissemination of this type of interventions.¹⁵¹ A systematic review of CBPR



studies to assess dissemination of research results beyond scientific publications found that dissemination to community participants and the general public is, however, variable.¹⁴⁸ Of the 101 articles included, only 48% reported dissemination beyond scientific publication. This does not necessarily mean that no dissemination had taken place, as 98% of the authors who responded to a survey reported having disseminated the results of the research to community participants.¹⁴⁸ Similar findings were made in other domains.¹⁵²

6 HOW TO SELECT PATIENTS TO BE INVOLVED?

The selection of patients to involve in the research process is one of the most important challenges in patient involvement.^{153, 154} Who can represent potential users of a technology? Do we need representativeness of the entire population? Who has appropriate experience? Should we include patients talking about their personal experience or patient advocates, representing a patient population? Depending on the phase in the research project, the profile of the patients to be involved may differ. Ideally, more than one person is involved.⁶

6.1 Identification

Patient involvement should ideally represent all types of patients with different experiences, values and preferences, both for democratic reasons and for scientific reasons.^{6, 36} Patients with diverse backgrounds (cultural, gender, ethnic, social class, etc) should be 'represented'.^{154, 155}

In this context, it should be noted that members of patient associations are usually a select minority of especially active patients compared to the total population of patients with a certain disease or condition. Therefore, they not necessarily represent the true nature of the experience of a certain disease or condition.¹⁵⁵ If the objective of the patient involvement is to collect information about the context and patient experiences, e.g. at the scoping phase of a study, it should be ensured that the patient representatives are providing input based on the experiences of the majority of the population they are representing, and not just their own experience. It might be required to ask the patient representative from a patient organization to consult its members to get a broader view of patients' experiences.

Moreover, some members of patient associations, especially the large ones or umbrella organizations are professionalized.^{156, 157} This facilitates their involvement, but may also create a bias and reduce the genuine experiential knowledge contributed.¹⁵⁷ In addition, there is large variability between



patient associations in terms of size, resources and capabilities, which has implications for their ability to contribute.

Eligibility criteria for involvement could also impact the identification of potential (organizational) patient partners in a research. For example, EMA defined the following eligibility criteria and rules for patient and consumer organisations within their permanent cooperative structure:

- Not-for-profit
- Legitimacy (legitimate claim to represent patients and citizens across the EU)
- Legal entity (legally established in the EU – with an EU focus and independent)
- Structure (governing bodies elected by their members – corporates excluded)
- Accountability (adequate means and procedures to consult and communicate with members)
- Transparency (registered status, disclosure of mission and objectives, list of members (governing bodies and geographical spread), sources of funding, annual financial statement, code of conduct for relations with funders)
- Sources of funding (if an organization has more than 20% annual budget coming from industry funding, then the funding must come from at least three different companies)

Other criteria that can be considered are geographical spread, disclosure of material and immaterial benefits.

Regarding the potential conflict of interest of patient organisations due industry funding, Van de Bovenkamp and Trappenburg (2011) concluded from a literature review that too much government funding might also not be ideal, as patients organisations might then become strategy followers rather than agenda setters. The empowerment of patient organizations might be undermined.¹⁵⁸

6.2 Recruitment

In a UK survey, five recruitment approaches were found: (1) via patients or service users known to the researchers or clinicians, (2) via local NHS comprehensive local research networks, (3) via voluntary organizations, (4) via established user groups, (5) via open invitation.¹⁵⁹

In a survey of the European League Against Rheumatism (EULAR), the proposed recommendation that “*The selection process of patient research partners should take into account **communication skills, motivation and constructive assertiveness** in a team setting*” gained large support from patients and professionals: 100% of the surveyed patients agreed with this statement, 96% of the professionals agreed.¹⁶⁰ What is meant by this requirement is that patient partners should have a critical, constructive and proactive attitude. ‘Critical’ means to be able to question the validity of statements irrespective of who makes them (professor, researcher, clinician...). A factor that might hamper this critical attitude is the possible clinical relationship between the patient partner and a clinical professional. Important in that case is to make mutual expectations explicit. The League also considers good communication skills from the part of the patient partner important to express personal experiences to professionals in a compelling and useful manner. Given its international perspective, EULAR also mentions that the capacity to read, write and speak English is essential for reviewing literature and to participate in international project meetings. For national projects this is not a requirement. With respect to patients’ professional or educational background, the organisation states that patient partners do not require academic training and do not need to become ‘professional researchers’. Interestingly, it adds that a medical background can even be a contraindication because professional knowledge may then become dominant and the basic objective of patient involvement is to add experiential knowledge, i.e. contributed by someone who can think like an outsider. Nevertheless, a basic familiarity with medical terminology is considered useful. Some background information or training can still be provided to the patient partners before the start of the study, if necessary.¹⁶⁰

Less support was found for the proposed recommendation to involve minimum two patient research partners in a project (93% versus 68%).



There is no solid evidence about the required number of patients to involve. Much will depend on the topic and the objective of the involvement. Arguments in favour of more than one patient are related to the health condition of the patients (implying their possible inability to contribute at some points in time), the relative weight of the patients' voice compared to that of the professionals, increased (self-)confidence of the partner, the opportunity to discuss issues with other partners, the introduction of more diversity in the contributed preferences and opinions, and improving the level of preparation.¹⁶⁰

Regarding the involvement of older patients in research, Puts et al.(2017) emphasise that researchers should make efforts to also involve hard-to-reach groups as they can provide different views and opinions.³¹ These groups could include, for instance, dementia patients, homeless people with addiction and mental health issues, patients from ethnic minority communities, frail older people who live in their own home, etc. They also include patients with communication impairments or not speaking the native language of the country.³¹ This contrasts in a sense with the recommendations of EULAR.

Vat et al. (2017) described, based on a qualitative study involving interviews with researchers and patient contributors, factors determining the recruitment of patients as partners in research projects, and developed recruitment strategies.¹⁶¹

Factors influencing the recruitment are listed in Table 6.

**Table 6 – Factors influencing recruitment of patient partners in research¹⁶¹**

Theme	Factors	Description (findings from the interviews)
Environment	<ul style="list-style-type: none">• Recruiters' characteristics and public image• Awareness of engagement opportunities and impact• Relationships, networks and infrastructure	The need for an environment in which the public has an awareness about engagement opportunities and the (potential) impact of patient engagement. Furthermore, relationships, networks and infrastructure facilities such as directories could increase the success of recruitment. The recruiters' characteristics and their public image are also noted as influential factors. Interviewees widely emphasized that the recruitment strategy should fit with the patient characteristics the team is hoping to recruit.
The opportunity	<ul style="list-style-type: none">• Match between interest, skills and experiences• Match with the lead/team• Clear role and responsibilities• Time commitment• Real impact	A clear description of the role, responsibilities, commitment and (potential) impact is helpful to recruit and select patients.
Patient characteristics	<ul style="list-style-type: none">• Desire to help• Time and resources• Health status• Education, skills and interest• Experiences and perspectives	Patients who have time and an interest in the research topic are more likely to become engaged. Patients bring skills, perspectives and experiences. Drop-out reasons were reported such as health issues or caregiving responsibilities, different priorities, frustration with the pace of the project and an overload of work or volunteer activities.
Climate	<ul style="list-style-type: none">• Recognition and compensation• Shared decision making• Communication and follow-up• Respect and trust• Social• Equality	Recognition and compensation are key factors for retention. Interviewees covered expenses such parking fees and travel costs. Multiple interviewees offered financial compensation such as an honorarium, hourly rate, per diem compensation or gift cards.
Education and support	<ul style="list-style-type: none">• Team support• Emotional support• Practical support• Education opportunities	Education opportunities, ongoing mentorship and support are influential factors for retention. A number of emotional and practical considerations have to be taken into account while working with patient partners such as supportable furniture, timely breaks, transport facilitation and accessible accommodation.

Source: Vat et al. (2017)¹⁶¹



Vat et al. (2017) describe three models for recruitment:¹⁶¹

- **Traditional model:** this model focuses on a case-by-case recruitment approach and is very much driven by the subject of research itself. Recruitment starts when a project team is looking for patient partners. Recruitment support might be available in various ways, but no formal structures (such as directories) are available.
- **Third party model:** this model is similar to a matching service. Generally, a third-party with access to patient directories helps researchers and patients find the right match. Recruitment could start before a specific engagement opportunity becomes available. A third-party provides assistance and can search through the directory to match patients and researchers.
- **Directory model:** this model is similar to a dating service. The model focuses on the creation of an (often) online directory of patients who are interested in partnering with researchers. A key difference between this model and the third-party model is that access is generally not controlled. Researchers can post opportunities for engagement, while patients can search for opportunities and can directly apply for research projects.

Possible recruitment strategies encompass social marketing recruitment (television, newspapers, social media), community outreach (booths, presentations), health system recruitment (via healthcare providers), partnering recruitment (via sickness funds, patient organisations, marketing company).¹⁶¹

Similar factors were highlighted in a review of current practices of patient involvement in research¹²⁸ and a review on involving specifically disabled children and young people as partners in research¹⁶². Both reviews found frequent use of existing relationships, advertisements or key community contacts (e.g. nurses, teachers) to recruit patient partners.^{128, 162} The importance of clearly explaining what the patient partners are being asked to get involved in, has been highlighted by many authors. The use of appropriate language and formats for communicating about the expectations is also of crucial importance.^{154, 162}

7 BENEFITS, RISKS AND CHALLENGES OF PATIENT INVOLVEMENT IN RESEARCH

Patients are increasingly involved at earlier stages in research projects, but the benefits and risks of partnership with patients in research is difficult to assess formally.¹⁶³ Therefore, there is relatively little hard evidence about the positive and negative impact of patient involvement in research.^{77, 164-166} One reason for this is that patient involvement approaches are very diverse, depending on the goal of the involvement, the topic under investigation, the research phase, the patient populations concerned etc.⁷⁴ The lack of standardisation in methods used renders comparisons or evaluation difficult. The lack of standardisation should not be regarded as a weakness, though, as it is actually due to the fact that methods are often personalised (e.g. instruments adapted to the target population, in CBPR based on the feedback from community leaders about cultural appropriateness) to more closely match the needs, resources and priorities of the community partnerships and target population.

Another reasons for the lack of hard evidence on the benefits and risks of patient involvement in research is that patient involvement is often poorly described in scientific research papers.^{74, 131, 166, 167}

In this chapter we describe (1) the existing evidence from systematic reviews on the benefits, risks and challenges of patient involvement in research and (2) the specific impact on patients, (3) on researchers and (4) on the research process and outcomes and (5) how the impact of patient involvement in research on the research processes and outcomes can be evaluated.



7.1 Benefits, risks and challenges of patient involvement in research: findings of published literature reviews

The evaluation of the impact of patient involvement in research is relatively limited. Nevertheless, we identified several literature reviews on patient involvement in research.^{30, 31, 34, 70, 73, 88, 89, 116, 119, 128, 139, 148, 162, 166, 168-179}

The review processes were often hampered by a lack of coherence in the terminology and definitions used regarding patient involvement across primary studies. Also the methods of patient involvement varied between studies, as well as the phase of the research process the patients were involved in. Most literature reviews about the impact of patient involvement in research identified only few studies that applied comparative methods to demonstrate the effects and added value of patient involvement: most of the reported effects seem to be opinion based and much of the evidence concerning impact remains relatively weak.

Nevertheless, it can be concluded from these reviews that, in general, patient involvement has effects on patients, on researchers and on the research process and research outcomes. The overall impression is that patient involvement has several benefits, but may also have negative effects and challenges associated with it. Several barriers and tensions have also been identified in the literature. A summary of the findings of the reviews identified through our literature search is provided in the summary of findings table in Appendix 1.4. The next paragraphs provide a brief summary of the results.

7.2 Impact on patients involved in research

Described **positive impacts** on patients involved in research are: feeling listened to and empowered³⁰, feeling valued, feeling part of a team^{73, 143, 168}, increased confidence, self-esteem and independence,^{89, 162, 174} having improved access to information, being able to engage with researchers^{31, 168}, learning how to share views^{143, 179}. Involved patients also value gaining knowledge of their condition,¹⁷⁴ and patient involvement in research also helps patients understand research better and develop a more positive attitude towards research.^{172, 174, 176}

Described **negative impacts** on the patients involved include frustration due to feeling not valued or listened to, feeling marginalized, feeling not being taken seriously, not receiving feedback from researchers and the fear of being engaged in something different or for wrong purposes.³¹ Some patients also reported increased emotional burden due to having to recall/talk about their own experiences and listen to those of other patients.¹⁶⁸ Lack of preparation and training led some patients to feel unable to contribute to the research.¹⁶⁸

Also operational issues may have a negative impact on patients. For example, patients may get frustrated with the lengthy processes that involve training, transportation, attendance to meetings, sense of powerlessness due to lack of awareness of certain research logistics etc.^{34, 128, 139, 168, 180}

7.3 Impact on researchers

The **positive impacts** of patient involvement on researchers include gaining new insights into the research issues and a greater understanding of the patients' needs, gaining respect and good connections with the (patient) community, enriched interpretation of research findings through integrating patient perspectives, the potential for wider dissemination and translation of their research results and learning new skills.^{30, 31, 89, 168} On the positive practical side, some researchers report that patient involvement might result in less workload for the researchers, depending on the role and decision power given to patients.³¹ For example, in a patient-led research project, researchers might take the role of advisors.



Negative impacts on researchers reported in literature are that researchers might sometimes feel uncomfortable when patients' ideas do not match their expert vision, particularly when it concerns different visions of what constitutes good research.³¹ Researchers also reported difficulties in incorporating patient and public involvement in meaningful ways due to lack of money and time.¹⁶⁸ Finally, patient involvement may induce additional costs and it may take longer to complete the research.³⁴

7.4 Impact on research processes and outcomes

Benefits of patient involvement in research on the research processes and outcomes are described for:

- the quality, appropriateness and relevance of the research;^{30, 31, 73, 171, 174, 176, 178}
- the credibility of the research;^{174, 176, 179}
- research ethics: consent process and developing ethically acceptable research;^{174, 176, 178}
- the study design;^{34, 139, 176, 178}
- the establishment or refinement of the research questions;^{30, 176}
- the writing of the participant information;^{139, 174, 176}
- recruitment and retention of study participants;^{30, 31, 34, 174, 176, 178}
- the choice of tools and methods;^{176, 178}
- defining the outcomes to be considered;^{34, 176}
- the data collection and analysis;^{174, 178}
- the reporting and dissemination of research results;^{148, 174, 176, 178}
- the uptake of research findings^{31, 170, 174} and contextual readiness for research implementation;^{30, 171, 175, 176, 178}
- the cultural alignment of survey and interview questions;³⁰
- patient empowerment;¹⁷⁰
- the creation of partnerships, with mutual learning by patients and researchers about each other;^{170-172, 175, 178}
- patients' trust in research and researchers;^{170, 171, 178}
- the transparency of research and increased understanding of the research process;^{171, 172}
- (specifically for CBPR) the health and well-being of the community members by means of community-level action;¹⁷³
- (specifically for CBPR) the participation of racial and ethnic minority subjects in research and the generalizability of effective interventions among these populations.⁸⁸

Less impact was reported on developing funding applications, managing or carrying out the research.¹³⁹

The **overall challenge** is to find the right balance between joint decision making of researchers and patients in research processes, safeguarding scientific rigour and validity of, e.g., the sampling procedures, measurements and analyses. **Barriers** to patient involvement identified in the literature relate to researchers' lack of motivation or inability to identify appropriate people to involve, or from scepticism or lack of interest amongst the people approached to be involved. The methods chosen may be inappropriate, or not reach standards, there may be insufficient financial, time and skilled human resources. Involved patients might be reluctant to express their views or be poor listeners. Research tasks themselves may not be open to influence by outsiders and the researchers and those they work with may be resistant to change. In addition, inequalities in involvement and unequal opportunities for involvement might in fact continue the exclusion of groups who are already alienated by organizational structures and procedures.³⁵ Additional barriers are lack of guidance, the use of jargon, and the difficulty of recruiting suitable patients.⁷⁴ Indeed, there is a lack of evidence about what works. Subjectively, it is felt by researchers and patients engaging in patient involvement activities that the level of benefit



derived for patient involvement is proportional to the level of resources made available and investment in obtaining and facilitating that involvement.⁷⁴

In the literature, we identified the following **challenges** of patient involvement in research:

- Ensuring sufficient time and resources and adequate planning of patient involvement activities;^{31, 33, 34, 153, 159, 162, 171, 174-176, 181}
- Predefining the roles and expectations of each research partner;^{31, 175}
- Working on patient relationships and trust;^{31, 171, 175, 181}
- Being flexible as researcher;¹⁷¹
- Sharing power;³¹
- Dealing with patients who have their own agenda,³¹ or request changes to research scope that are unfeasible;¹⁷⁶
- Communication among partners, including dealing with dominance of some people in meetings;^{175, 181}
- Uncertainty regarding resolving conflicts;¹⁷⁶
- Consistent and committed partner participation or membership;^{171, 175}
- Aligning visions, goals, and/or missions;¹⁷⁵
- Using a common language and shared terms;^{153, 175}
- Ethical concerns (in some institutional review boards);¹⁷¹
- Avoiding or reducing patient distress while participating, ensuring patients' confidence to actively participate;^{159, 171}
- Overcoming cultural differences between stakeholders and researchers;¹⁷¹
- Training of patients and researchers to acquire the knowledge, skills, and experience on how best to involve patients or be involved in research;¹⁷⁶

- Recruitment of patient representatives to be involved, taking risks of the involvement for patients into account possible fluctuating health state or energy of patients)^{31, 128, 159-162, 181}, see paragraph 6
- Representation: patients might lose their patient perspective through a process of socialisation into the research world;¹⁵⁹
- Communicating the research to lay people;¹⁵⁹
- Long-term commitment;¹⁵⁹
- Representation of hard-to-reach patient populations.²⁰

Specific **risks** of involving patients in research include:

- Scope creep: a theoretical concern that engaging patients in the research may include irrelevant community concerns and issues, which would make the research unfeasible;^{34, 35}
- Tokenism: a false appearance of inclusiveness, resulting in a devaluated patients' input;^{34, 153, 159}
- Disconnect between patient co-researchers and researcher foci of interest¹²⁸: researchers might be mainly driven by research gaps, whereas patients may be driven by their unmet needs
- Power imbalance;^{128, 153}
- Risk of bias.³³

To avoid tokenism and scope creep, adequate time should be spent to the building of reciprocal relationships between patients and researchers, fostering mutual respect and developing clear expectations that are explicitly described and documented in study protocols.⁷¹ Also, planning to assess the impact of patient involvement on the research process and outcomes could reduce tokenism.²¹ The Dutch Organisation for Health Research and Development (ZonMW), a funding agency for health research, has taken concrete initiatives to ensure genuine involvement of patients in the design



of the clinical trials they fund.^{ww} The organisation appointed an officer to support the development of patient involvement in clinical trial design and ensure that patient involvement does not become tokenistic.

The resource issue should not be underestimated. In times where the pressure to produce and demonstrate value for money is increasing, also in research, taking more time might not easily be accepted. Even modest patient involvement activities consume a great amount of time.²¹ It takes time to train patients, support them in the process and create documents in accessible language,^{77, 165} to allow patients to respond to specific request or to review draft documents and prepare their feedback.²¹ Therefore, adapting the deadlines to the possibilities of the patient involvement is essential for the involvement to be effective.¹⁸² For example, CADTH grants patients and patient organizations 20 days for the completion of forms asking about patients' experiences, values and preferences.

Besides time, also human resources to facilitate the participation of patients are necessary. Trained personnel should facilitate the involvement of patients.⁷⁷ patients should know to whom to turn to for all aspects related to the involvement activities, a coordinator should maintain ongoing contacts with patients and tutor them throughout of the process.^{21, 77} The coordinator of the involvement activities should meet a number of criteria: capable to select and invite participants, facilitate and moderate involvement, integrate the contributions of the patients in the research process, translate the process and the results to plain language, and monitor and evaluate the impact. Support for participants requires flexibility and creativity to adapt to unforeseen needs.²¹ In addition, patients should be given feedback on their contributions, highlighting the strengths and weaknesses of the contributions, as well as giving suggestions about how to improve their contribution in the future.^{125, 182}

Based on identified risks and challenges with involving the public in the research design phase, Pandya-Wood et al. (2017) developed 10

recommendations to perform ethical patient/public involvement in research in a UK context (Box 1).¹⁸³

Box 1 – Recommendations for ethical patient involvement in research in the UK¹⁸³

- Allocating sufficient time for public involvement;
- Avoiding tokenism;
- Registering patient and public involvement work in the research design stage with NHS Research & Development Trust Office at earliest opportunity;
- Communicating clearly from the outset around the objectives of the patient involvement to manage the expectations of all partners;
- Entitling public contributors to stop their involvement for any unstated reasons;
- Operating fairness of opportunity;
- Differentiating qualitative research methods and public involvement activities;
- Working sensitively;
- Being conscious of confidentiality and
- Valuing, acknowledging and rewarding public involvement.

Many of these recommendations refer to the quality of the preparation and organization of the patient involvement activities. This should be done in a professional manner. Poorly conducted patient involvement can lead to a

^{ww} <http://www.invo.org.uk/patient-and-health-consumer-involvement-at-zonmw/>



lack of trust among participants and a loss of reputation for an organization.¹⁸⁴

7.5 How to assess the impact of patient involvement in research?

The Spanish network of HTA agencies, RedETS, defines 'effective participation of patients' as a process whereby patients can effectively weigh on the decisions made within the assessment, from the selection and prioritization of topics up to the recommendations. It requires a commitment from assessors that patients' input will be taken into account.²¹

In 2014, Popay et al. published the Public Involvement Impact Assessment Framework, a practical guidance with examples to assess the impact of patient involvement activities in research.¹⁶⁷ The framework acknowledges the difficulty of assessing the impact of patient and public involvement in research, because of the diversity of public and patient involvement approaches in terms of the public involved and the aims and context of the involvement. The Framework therefore identifies the main elements that influence public involvement in research and the impact of this involvement. These include the **values** associated with public involvement by the members of the research team (normative, substantive of process values), the **approach** adopted for public involvement (e.g. consultation, collaboration or control, as members of a study advisory board or as data collectors), the **focus** of the research (e.g. HTA, health services research, public health), the study **design** (e.g. systematic literature review, survey, interviews) and a wide range of **practical issues** (e.g. available resources).

Impact can be classified as relating to the research (e.g. instruments used, outcome measures included, data collected) or to the people involved (researchers and patients). Impact may be seen on the short term (e.g. patient information leaflets) or the on the long term (e.g. on recruitment/retention of patients for trials). Impact may be positive or negative and intended or unintended.¹⁶⁷

When assessing the impact of patient involvement in research, it is important to consider also the economic impact, as patient involvement requires additional resources.

While quantitative research on the impact of patient involvement in research might be difficult, qualitative research might be performed. For example, when patients are involved in the dissemination of the research results through concrete messages, the impact could be measured by means of surveys that ask the target group to what extent they recall having seen the messages.

An example of impact of patient involvement on the researchers and on different aspects of a research process was provided by Heaven et al. (2016), see Box 2.¹⁸⁵

Box 2 – Example of clear description of the impact of patient involvement in a research project

It concerns a case where representatives of frail patients were involved in a multiple randomised controlled trial. The impact of involving frail patients in the study was described on several aspects:

- **Prioritising areas of research:** a researcher explained that the "Frailty Oversight Group" (FOG), a group of representatives of the community of frail elderly, had an important impact on his/her thinking, by showing little enthusiasm for his/her work. The group explained that the topic of the proposed study was not a priority for this community.
- **Study design:** the FOG facilitated the pilot testing of instruments, the data collection process. This allowed the researchers to demonstrate content/community validity and schedule their data collection in a realistic and sensitive timeframe for participants.



- **Management:** the involvement of the FOG taught the researchers that it is important to keep in touch with patients between the assessments at 6 and 12 months. A 'celebration event' was organised to this end. The group also highlighted the need to understand the drop-out: why do patients step out during the study?
- **Undertaking research:** the FOG has helped in safeguarding the interests of the study participants (e.g. through review of the informed consent forms), highlighted considerations for the analysis and interpretation of data.
- **Analysis, interpretation and reporting:** the FOG assessed the clarity of the language used in the lay summary and the relevance of sub-studies or criteria to classify people in subgroups. For instance, they highlighted that non-home owners were not necessarily people with lower incomes.
- **Dissemination:** FOG members distributed the results of the study through their newsletters, on websites of organisations they are affiliated with and co-presented the results at a national conference. Through their personal networks, they raised awareness of the study results

Source: Heaven et al. (2016)¹⁸⁵

8 STANDARDS FOR PATIENT INVOLVEMENT IN HEALTH POLICY RESEARCH

This chapter describes published standards for patient involvement in research. Some standards are specific for one domain of KCE research (e.g. for HTA or trials), others are more generic.

8.1 General requirements for meaningful patient involvement in health policy research

To meaningfully involve patients in health policy research, there must be a positive culture towards patient involvement, someone needs to take up the leadership for the patient involvement activities within an organisation and coordinate the activities, and sufficient resources must be available and training for researchers and patients on how to effectively involve/be involved needs to be provided.

8.1.1 Culture

The effectiveness of the contributions of patients is conditioned by the relationships that are established with the other actors involved. Empowering relationships and partnerships with patients, involving trust from patients and professionals, favor meaningful participation.

There is still significant resistance against patient involvement among doctors, investigators, and project managers.¹⁸⁶ An antiquated academic system, where merit is based on the journal in which research is published, not on how it affects society also contributes to this problem. If a researcher participate in extensive patient engagement and knowledge translation activities, this goes unrewarded.¹⁸⁷ Besides this "mental" barrier, other barriers need to be tackled as well, including communication, logistical, organisational, legal, financial, and administrative barriers.¹⁸⁶



It is important that all relevant stakeholders and institutions commit to the process, including the medical and research communities, the patient community, contract research organisations, hospitals, drug industry, government, etc. Institutional support for patient involvement is also essential for the success of patient involvement activities.¹⁵³ A climate of dialogue must be established, in which contributions of patients, citizens or their representatives are equally taken into account as those of professionals.

8.1.2 Leadership and coordination

Incorporating patient perspectives into a scientific evidence-based health policy research process is a delicate task and a challenge to researchers. They have to do justice to the voice of patients while remaining neutral and evidence-based. Transparency and clear communication with participants is key for successful and valuable engagement. A **coordinating team** for patient involvement may facilitate the organization and commitment to patient involvement in policy research. This team can be made responsible for:

1. Communication
 - Broad, ongoing communication and collaboration to integrate patient perspectives into evidence-driven processes
 - Ongoing discussion with each new project team
 - Clear expectations and role of patient preferences and values data
 - Help raising awareness among patients
2. Measurement, evaluation and impact
 - Evaluation and measurement of impact of patient involvement
 - Careful consideration of what questions to ask, how to ask them, how to measure the impact
 - Continuous refinement and evolution for both internal and external evaluations

8.1.3 Economic resources

Resources are required to organize and set-up patient involvement activities, both in terms of time and people.¹⁵³ In addition, budgets are needed for the reimbursement of expenses of patients involved in policy research projects. Some HTA agencies in Europe (NICE, G-BA, Haute Autorité de Santé (HAS)) remunerate the participation activities of the patients.²¹ A survey of patient organizations in Canada notes that a large majority (between 75% -100% of the organizations) consider the reimbursement of expenses necessary, while only a minority (between 20-30%) consider that it would also be necessary to be remunerated for participation.¹⁸⁸ Payments can also happen in the form of gift cards, meals, goody bags or childcare.¹⁸⁹

8.1.4 Information and training

Patients and researchers involved in HTA agree that providing information and training to patients and researchers about patient involvement is a requirement for meaningful patient involvement.^{21, 153} The training of patients and their representatives can include information about the evaluation process, the technical language used in the study and on the studied policy problem.^{13, 21, 190} Professionals may need to be trained to develop communication and language skills adapted to the level of understanding of all participants, as well as skills to promote positive attitudes among the people involved in the research.²¹ In addition to training, the moderation style of the groups can play an important role, through strategies such as the establishment of rules of the game with the participants and the development of the agenda.²¹

It should be noted, that for some roles training of patients may be necessary, while for others it might not.¹⁹¹ If patients are being asked to review study proposals, for instance, training in the principles of evidence-based medicine might be needed. If patients are involved to share their perspectives on a topic that concerns them, it might not be needed to give them formal training. For example, when patients are involved to help selecting the outcomes to be considered in a research project, or on patient information leaflets, 'untrained' patients may make particularly valuable contributions.¹⁹¹



However, for patients new to a patient involvement role, support to develop their abilities and confidence to express their views may be useful.^{182, 191} NICE and IQWiG (Germany) have participation support programmes for patients, including information activities, training and resolution of questions or problems.

Several training resources for patients already exist, e.g. EUPATI, PFMD, HTAi^{xx}. Resources for researchers include INVOLVE, PCORI, SPOR, HTAi. More information about some of these resources is provided in Chapter 11. The practical guidance for information and training will, however, be developed in the KCE process book.

8.2 Guidance from literature

The Canadian Institutes of Health Research (CIHR) have developed a framework for patient engagement that identified four guiding principles for patient involvement: inclusiveness, support, mutual respect and co-building.^{yy} Five desired outcomes of patient involvement in research are:

1. Creation of inclusive mechanisms and processes: patients should be involved on all levels;
2. Respectful collaboration amongst patients, researchers and healthcare providers
3. Valuing experiential knowledge of patients as evidence as part of the research process
4. Co-direction of research by patients
5. Common goal of timely implementation of quality research.

Further down the line of implementation of these principles, Nicholson et al. (2017) identified *6 requirements* for a positive outcome and impact of patient involvement in research from a systematic literature review of peer-reviewed

and grey literature, combined with interviews with researchers who involved patients in their work¹⁹²:

- Researchers and lay representatives should have a shared understanding of the moral and methodological purpose of patient involvement;
- A key individual serves as patient involvement coordinator;
- Representatives involved in the research should have a strong connection with the target study population;
- The whole research team should be positive about patient involvement input and fully engaged with it;
- Efforts to develop relationships should be established and maintained over time;
- Patient involvement is evaluated in a proactive and systematic approach.

Witteman et al.¹⁶³ translated these principles to concrete actions. Based on their experience with patient involvement in research, they make 12 concrete suggestions to researchers, to deal with three challenges of patient involvement:

- Challenge 1: Establishing and maintaining a **culture** and expectation of mutual respect
 - Have a face-to-face meeting with the full team as early as possible;
 - Introduce yourselves with stories, not titles;
 - State individual and project goals explicitly: ask all team members to state explicitly what they hope to bring to the project, what they

^{xx} <https://htai.org/interest-groups/pcig/resources/for-patients-and-patient-groups/>

^{yy} <http://www.cihr-irsc.gc.ca/e/48413.html#a4>



- hope to get out of it and what they hope the project contributes to healthcare;
- Offer orientation to everyone: orientation rather than training, to avoid the connotation of implicit power imbalance inherent in the term. Orientation intends to make all team members familiar with a specific terminology.
- Challenge 2: **Actively involving** all team members
 - Ensure funding for everyone's participation
 - Recognize different kinds of contributions and efforts
 - Invite people to contribute and take up roles
 - Privately check in with people who are quiet: some people may prefer to comment individually, by email or in a subsequent meeting after reviewing notes and summary documents.
- Challenge 3: Facilitating good **communication**
 - Think carefully about labels, as they convey implicit values:
 - Beware of jargon and acronyms
 - Occasionally regroup in smaller, more homogeneous groups
 - Create a visual map of the project

ZonMW also published a checklist for researchers and patients to improve the effectiveness of patient involvement in research, dealing with the different challenges identified in Chapter 7.^{zz}

- Define the concrete goal of the involvement;

- Describe the roles and tasks of each partner in the research process and make sure they match with the capabilities of the partners and the timing;
- Describe expectations regarding the collaboration
- Make a planning with clear decision points, define how the decision is informed and who will take the final decision
- Define timelines
- Discuss what you expect to learn from the collaboration
- Discuss confidentiality issues
- Decide on possible payments for patients
- Discuss the required support from researchers to allow patients to effectively engage in the research processes (e.g. kick-off meeting, workshop, contact person)
- Discuss any practical issues, e.g. related to the meeting facilities, or other practical requirements for patients

An important lesson from the projects of ZonMW on patient involvement in palliative care research^{aaa}, is that researchers should be careful about *a priori*'s. Thinking that patients are too ill to participate or will probably not be interested is a threat.

Similar requirements for an impactful involvement of patients in research were identified by several other authors^{7, 139, 193, 194}, such as:

- Patient involvement should be promoted as a core research function in all research by raising awareness of its value and impact
- Clear goals should be identified to clarify the purpose of the involvement

^{zz} https://www.elsi.health-ri.nl/sites/elsi/files/Een_10_voor_patienten_participatie%20ZonMw.pdf

^{aaa} <https://www.zonmw.nl/nl/actueel/nieuws/detail/item/de-stem-van-patient-en-naasten-verbetert-kwaliteit-onderzoek/>



- Sufficient preparation, training, support, supervision and financial remuneration is to be provided to enable the public to fully contribute and undertake the roles required.
- Reciprocal relationships are to be established where all involved can benefit
- Dedicated people should be assigned in research institutions to promote best practice
- Members of the public should be able to contribute in different ways
- Different approaches, perspectives, skills, styles and contributions are to be valued
- Academic and practice researchers must be open to relinquishing and sharing control to facilitate new ways of working.

Practical guidance will be further developed in a KCE process book.

8.3 INVOLVE's national standards for public involvement in research

INVOLVE, the national programme for patient and public involvement in research in the UK, developed a framework in 2015 with values and principles that should help to guide researchers interested in involving the public in their research project to define good practice for public involvement.¹⁹⁵ 'Public' is defined as including patients, service users, survivors, carers and family members. The values and principles are summarized in Table 7.

Table 7 – Framework of values and principles for public involvement in research (INVOLVE)

Values	Summary of principles
Respect	Researchers, research organisations and the public respect one another's roles and perspectives
Support	Researchers, research organisations and the public have access to practical and organisational support to involve and be involved
Transparency	Researchers, research organisations and the public are clear and open about the aims and scope of involvement in the research
Responsiveness	Researchers and research organisations actively respond to the input of public members involved in research
Fairness of opportunity	Researchers and research organisations ensure that public involvement in research is open to individuals and communities without discrimination
Accountability	Researchers, research organisations and the public are accountable for their involvement in research and to people affected by the research

Source: NIHR 2015¹⁹⁵

In 2018, INVOLVE published a set of standards and indicators for public involvement in research, which are generally applicable to different types of research, be it clinical research or health policy research.¹⁹⁶ The standards are not a recommendation for a particular approach or method for public involvement, but provide a comprehensive framework that allows researchers to set realistic expectations, encourage improvement and achieve excellence in public involvement in research. Table 8 summarizes the standards and indicators as published by the NIHR. If formulated slightly differently, the indicators can easily be seen as guidance for meeting the standards.

**Table 8 – NIHR's Standards and indicators for public involvement in research**

Standard	Indicators
Inclusive opportunities	<ul style="list-style-type: none">• People affected by and interested in the research are involved at the earliest stage• Barriers to taking up public involvement in research are identified and addressed• Information about opportunities for public involvement in research are made available, using different methods so that relevant and interested people are reached• Processes for public involvement in research are fair and transparent• Choice and flexibility in opportunities for public involvement in research are offered
Working together	<ul style="list-style-type: none">• The purpose of the public involvement activity is jointly defined and recorded• Public involvement plans and activities are developed together• There is shared understanding of roles, responsibilities and expectations, which may evolve over time• Individual ideas and contributions are recognized and decisions are upheld together
Support and learning	<ul style="list-style-type: none">• Resources to ensure and support effective public involvement are designated and monitored• A range of support to address identified needs is offered• There is a clearly identified point of contact for information and support• Learning opportunities are developed, delivered and monitored in partnership, for all involved in research• The team actively learns from others, builds on what was learned and shares learning
Communications	<ul style="list-style-type: none">• A communications plan for involvement activities is developed and delivered• Inclusive and flexible in communication methods are used to meet the needs of different people• Feedback is gathered, offered, shared and acted upon
Impact	<ul style="list-style-type: none">• The public is involved in the assessment of public involvement in research• Agreed purpose for public involvement and its intended outcomes are recorded• Information that will help assess the impact of public involvement in research is collected• The extent to which the intended purpose and predicted outcomes are met are reflected upon, learnt from and reported
Governance	<ul style="list-style-type: none">• Public voices are heard, valued and included in decision making• Public involvement strategies and/or plans are in place and regularly monitored, reviewed and reported upon• Responsibility for public involvement is visible and accountable throughout the management structure• Money and other resources are allocated for public involvement

Adapted from INVOLVE (2018)¹⁹⁶



There is clearly no one size fits all formula for patient involvement. The NIHR emphasizes that public involvement activities should meet the needs of the research and those who wish to be involved. Moreover, the emphasis should be on partnership.

8.4 HTAi's quality standards for patient involvement in Health Technology Assessment

The HTAi Patients and Citizens Involvement Interest Group defined quality standards for patient involvement in HTA.²⁴ (HTAi 2014) They are listed in Table 9.

Table 9 – HTAi quality standards for patient involvement in HTA²⁴

Process	Quality standard
General HTA process	<ul style="list-style-type: none">HTA organizations have a strategy that outlines the processes and responsibilities for those working in HTA and serving on HTA committees to effectively involve patients.HTA organizations designate appropriate resources to ensure and support effective patient involvement in HTA.HTA participants (including researchers, staff, HTA reviewers and committee members) receive training about appropriate involvement of patients and consideration of patients' perspectives throughout the HTA process.Patients and patient organizations are given the opportunity to participate in training to empower them so that they can best contribute to HTA.Patient involvement processes in HTA are regularly reflected on and reviewed, taking account of the experiences of all those involved, with the intent to continuously improve them.
Individual HTAs	<ul style="list-style-type: none">Proactive communication strategies are used to effectively reach, inform and enable a wide range of patients to participate fully in each HTA.Clear timelines are established for each HTA with advance notice of deadlines to ensure that appropriate input from a wide range of patients can be obtained.For each HTA, HTA organizations identify a staff member whose role is to support patients to contribute effectively to HTA.In each HTA, patients' perspectives and experiences are documented, and the influence of patient contributions on conclusions and decisions is reported.Feedback is given to patient organizations who have contributed to an HTA, to share what contributions were most helpful and provide suggestions to assist their future involvement.



8.5 Good Clinical Practice guidelines

Recommendations for patient involvement in clinical guideline development have been developed for specific diseases or conditions,^{141, 142, 160, 197} but also the Guidelines International Network has developed guidance for public and patient involvement in guideline development¹⁹⁸, the Institute of Medicine in the US¹⁹⁹ and independent researchers.¹²⁰ In this paragraph, we focus on the general standards rather than on the disease-specific recommendations.

The Institute of Medicine recommends the inclusion of patients in the guideline development groups, the involvement of patients at least at the time of clinical question formulation and draft clinical practice guideline review, the adoption of strategies to increase effective participation of patient and consumer representatives, including training in appraisal of evidence. Simple selection criteria could be developed to choose consumers who will consider evidence objectively, act in the interest of all patients, and not be unduly influenced by personal experience.¹⁹⁹ Patients should also be involved as external reviewers, and the final draft guidelines should be opened for public review.¹⁹⁹ Despite these recommendations, though, few North-American organizations require patient/public involvement on guideline development groups.²⁰⁰

The Guidelines International Network (G-I-N) developed an extensive practical toolkit for patient involvement in the development in clinical practice guidelines, describing possible approaches for each of the following aspects:¹⁹⁸

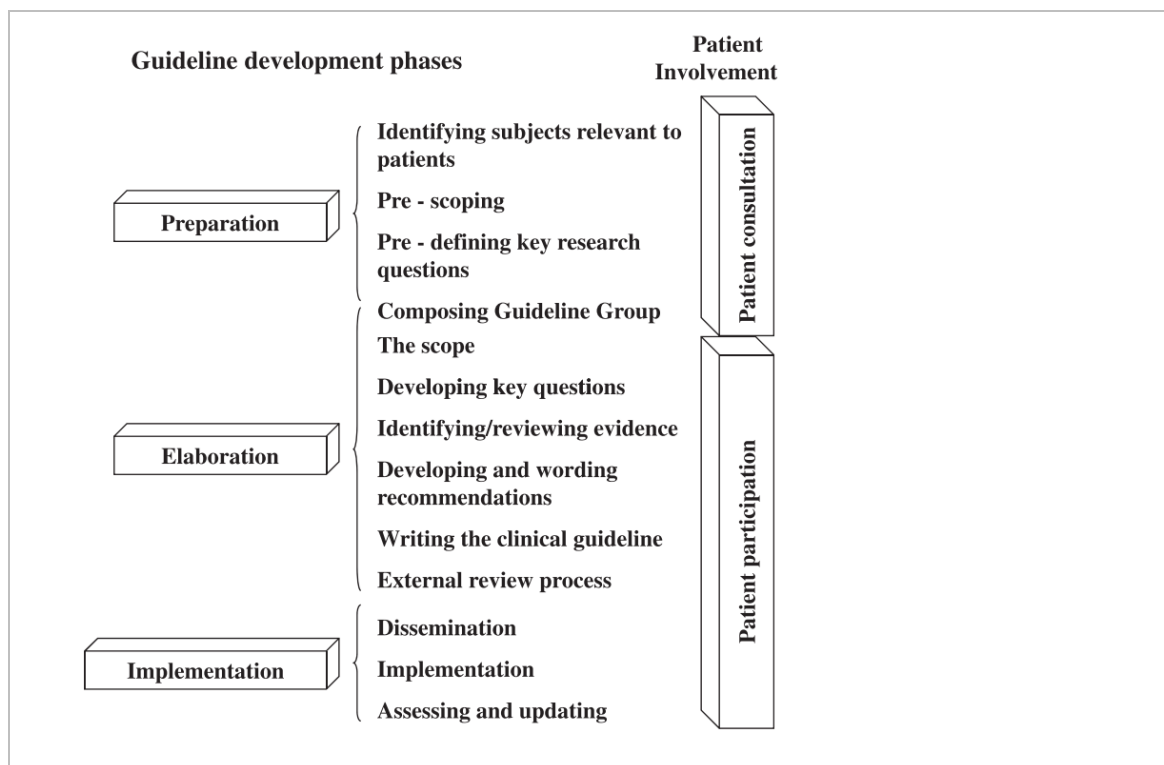
- Consultation of patients
- Inclusion of qualitative research on patient views in guidelines
- Recruitment and support of patients and the public in guideline development
- Involving people facing barriers to participation
- The role of the chair in patient and public involvement: training and support
- The use of web-based technologies to support patient and public involvement
- The development of patient versions of guidelines
- The involvement of patients in the dissemination and implementation of guidelines
- The use of guidelines to improve patient involvement in the clinic
- Tools to support patient involvement in HTA

The toolkit does not provide best practice guidelines for patient involvement in clinical guideline development.

Diaz Del Compo et al. (2011) developed a strategy for patient involvement in the different guidelines development phases, as shown in Figure 1.¹²⁰ Note that patient consultation refers, in this strategy, to primary research in patients, using qualitative or quantitative research methods (e.g. interviews, focus groups, Delphi panels, structured questionnaires) and review of existing evidence with respect to patient perspectives, whereas participation refers to the inclusion of patients in the clinical guideline development group.



Figure 1 – Strategy for patient involvement in clinical practice guideline development



Source: Diaz Del Campo et al. (2011)¹²⁰

The authors describe this strategy as a way to obtain “guidelines covering problems in which the patients’ views and their needs are as important as the effectiveness described for the interventions addressed”.¹²⁰ They recommend to perform primary and secondary research (i.e. primary data collection and literature review) to collect information regarding patient perspectives, experiences with illness, social circumstances, habits, values and preferences. Even if evidence is available in the literature, primary

research may improve the quality of the evidence, especially if the number of existing robust studies is limited. A requirement is, however, that the primary research is conducted with the necessary qualitative research knowledge.¹²⁰

With respect to the inclusion of patients in the guidelines development group, the authors highlight the potential barriers to effective patient



participation (time, resources, lack of knowledge, tokenism, and representation of all patients affected by the disease). They recommend a selection process to ensure patients' representativeness and appropriate support to facilitate effective patient engagement, providing clear guidance on their roles and responsibilities within the group and ensuring the opportunities to attend training events for all GDG members.¹²⁰

Work still needs to be done on the presentation. Despite all this, there is room for improving the approaches of involvement in clinical guidelines development, such as the presentation of the guidelines (e.g. highlight patient preference points).²⁰¹

8.6 Trials

Buck et al. (2014) investigated how patient and public involvement can be implemented in clinical trials and made several recommendations, taking both the researcher and the patient contributor's perspectives into account (Box 3). The recommendations relate to planning ahead, being flexible and asking patients how they want to be involved, establishing clear communication, paying attention to language, budgeting for patient involvement, and finding the right patients.²⁰²

Planning: Researchers and patient contributors emphasised how early and regular involvement allowed more effective involvement. Patient involvement prior to the trial (e.g. in contributions to grant writing, trial design, feasibility studies) was a key aspect of patient and public involvement.²⁰²

Flexibility: Regarding the context-specificity of public and patient involvement, researchers felt that involving contributors beyond an oversight role, i.e. not just as a member of the steering committee but in a managerial or responsive capacity, helped to foster meaningful patient involvement. Liaison with relevant patient panels or groups may be helpful when more diverse perspectives or wider consensus is needed; individuals might also consider whether surveys (e.g. of support group members) would be useful in answering 'burning questions', for example, regarding the acceptability of timing or format of interventions or data collection.²⁰²

Communication: researchers and patient contributors should communicate early on to clarify roles and expectations, and throughout the trial to optimise engagement and provide feedback about contributions. If particular contributors do not have the insights a trial needs, trialists may need to rethink their plans for patient involvement in the light of experience. Researchers should avoid seeming "dispassionate" during meetings when discussing a particular illness or condition that impacts on the lives of patient partners, and make a genuine effort to understand contributors' points of view.²⁰²

Language: patient contributors should be prepared to speak up if there is a problem and, with the help of researchers, be willing to acquaint themselves with specialist terms over time.²⁰²

Budget: Well thought-through plans will help inform how much to 'cost in' for public and patient involvement. It is important to talk to contributors to make sure they are able and willing to accept reimbursement beyond expenses.²⁰²

Fit for purpose: Plans for patient and public involvement should be centred round the aims and needs of the trial. Agreement about and understanding of *what* and *why* patient and public involvement is needed will help in planning it. Involving people with experience of the condition, intervention or service may be particularly useful for identifying research priorities and enhancing trial design. However, the inclusion of patients under the current care of a team member may lead to difficulties for researchers as well as contributors.²⁰²

**Box 3 – Recommendations for planning and implementing patient and public involvement (PPI) in clinical trials²⁰²****Early PPI**

- Begin planning PPI and consulting with contributors when starting to plan the trial.
- Consider including PPI contributors in managerial roles for example, as co-investigators.

Flexible PPI

PPI is context-specific so it is important to tailor PPI to the emergent needs of trials and be creative to encourage active engagement.

- Consider whether oversight PPI (e.g. on a trial steering committee) is sufficient to meet trial needs.
- Involve more than one or two PPI contributors, more than once or twice a year.
- 'Reach out' and make use of multiple modes of PPI, including responsive PPI.

Communication, clarification and interaction

- Negotiate with contributors at an early stage about what they can bring to the trial and what they want to bring.
- Determine whether this matches the trial's needs and clarify roles and expectations.
- Be sensitive to contributors' needs and preferences.

Language of research

- Minimise and explain jargon.
- Provide glossaries and 'translations' where applicable.

Budgeting for PPI

- Budget for PPI—think about contributors' time plus expenses.
- Explore opportunities for pre-trial support for PPI.

Fit for purpose PPI

- Agree what type of PPI would be appropriate and understand why.

- Consider benefits of involving those with experience of the condition.
- Recognise potential drawbacks of involving those under current care of the researcher.

Source: Buck et al. (2014)²⁰²

Bagley et al. are developing a toolkit for patient and public involvement in clinical trials, including a planning tool for the research team on things to consider before the start of the study, guidance for investigators on how to find patients for their teams, role description documents that can be customized for each study, and questions for the patient selection interview.¹ The first part of the development process for the toolkit consisted of the description of the pathway of patient and public involvement in clinical trials. The second part consisted of identifying existing resources and additional resources required for planning public and patient involvement in clinical trials. Tools were sought for (1) developing a plan for PPI in a trial, (2) identifying public contributors with appropriate experience and skills; (3) allocating appropriate costs; and (4) managing the expectations of public contributors.¹

Mader et al.(2018) developed Patient Led Research Hub to allow patients and the public to propose research questions, design, initiate and deliver their own research with all the necessary support from research professionals. This is an advanced way of patient and public involvement in clinical trials. The authors claim that patient-led research is feasible and patient organisations are able to initiate and conduct rigorous clinical research.²⁰³ It is based on a model where patients, via patient organisations, can submit topic proposals to the Patient-Led Research Hub. The collaborators of the Hub directly communicate with the submitting organisation to make sure the question is clearly understood and facilitate the collaboration between the proposers of a topic and researchers. Once the topic/issue is clear, researchers (clinical trialists and statisticians) assess the feasibility of the proposal in terms of technical and operational criteria (not the priority of the question). They address issues like required sample size, existing evidence, feasibility of recruiting sufficient participants etc. The conclusion about feasibility is reached together with the submitter. If the study is considered feasible, a full project outline is developed and external



field experts are invited to form a study management team and to submit a funding application. If the feasibility is uncertain, further work is done to allow an assessment.²⁰³ The major advantage of the patient-led research hub is that it helps to identify the needs of patients. The major difficulty of the Hub is the intensity of the feasibility assessment, requiring resources and input from experts, who are not always willing or available to commit to such assessments pro bono.

8.7 Community-based participatory research

Community-based participatory (action) research (CBPR and CBPAR) is a collaborative approach to conducting research where multiple stakeholders are equitably involved in all stages of the research process. CBPR tackles issues related to community health improvement and knowledge production.^{204, 205} The basic assumption of CBPR is that health interventions, and by extension health research, can be strengthened if they incorporate community values and insights. CBPR might be especially useful for studying health problems that affect mainly socially segregated population or difficult-to-reach populations, such as for instance intravenous drug users, homeless people. The objective of CBPAR is to enhance the effectiveness of health (promotion) programs resulting from the research, by actively involving the community actors in the implementation plan. Knowledge gained from CBPR is in CBPAR used to influence policy, and is integrated with interventions to improve the health of communities at large.²⁰⁴

Even though CBPR seems to imply a high level of involvement of patients or community members, different levels of involvement in CBPR have been described in literature,²⁰⁶ from targeted consultation, to community-led research. Interestingly, the terminology used in CBPR is different and based on who has control over the decisions in the study. McCabe-Selers et al.(2008) describe the following levels of involvement in CBPR:²⁰⁶

- Contractual involvement: this is the lowest level; researchers bring the proposal to the community and ask them to participate. The participants have no or little input or decision making, and the researcher is in full control.

- Consultation: researchers ask for the community's input and adopt some of the input. The researcher retains full control.
- Collaboration: the community and researchers work together to design and implement the study. The overall process is managed by researchers. This shared control model is currently the most frequently found model in CBPR.
- Collegiate model: all parties work together drawing upon different skills and there is mutual learning. The community has full control. The collegiate model requires training of community residents in research methods and previous experience in research studies.

Because CBPR is based on **cooperative relationships** between multiple parties (community members, service providers, program planners, policy makers, and academics), it requires a lot of specific (personal and scientific) skills to perform CBPR projects. Relationships can easily go wrong, when tensions and conflicts arise.²⁰⁷ Personal skills (how to interact) and relationship-building activities (what to do) are essential for successful CBPR. Personal skills include: being friendly and possessing strong social skills; being honest and authentic; demonstrating care, empathy, compassion, concern, and commitment; and being a clear and open communicator.²⁰⁷ Strategies to build collaborative relationships with individuals and communities include: getting in early, becoming involved in community life, practicing reciprocity, and creating an atmosphere of informality.²⁰⁷



9 ORGANISATION AND GOVERNANCE OF PATIENT INVOLVEMENT

There are at **four dimensions to patient involvement approaches**. The first dimension relates to the **intensity** or level of involvement, the second to the **structure**, the third to the **activities** and the fourth to the **representation** (i.e. who to involve as patient representative: individual patients, patient organizations, representatives of patient umbrella organizations or sickness funds).

The intensity of involvement refers to the level of decision power of patients in the research decision-making processes, as described in paragraph 2.1 (patients can be consulted throughout the research project or for specific aspects, make joint decisions with researchers or decide autonomously).

The structural dimension refers to how the patient involvement is organized; i.e. are patients structural members of a review committee, members of the research steering committee, or part of a separate patient advisory committee or are they consulted incidentally, depending on a specific question of for a specific purpose.

The 'activities' dimension refers to what patients are asked to do: review proposals, perform interviews in the context of data collection, write lay summaries, etc.

This chapter describes possible organizational and governance models, based on a few examples from existing agencies involving patients in their research programs and from literature. The appropriateness of an organizational model will at least partly depend on the role defined for patients and the expected activities. For example, a model where representatives of patient umbrella organizations are embedded members in an advisory committee is less apt for targeted consultation but fits better with an embedded consultation or joint decision making approach. Nevertheless, the organizational approaches described in this chapter are not mutually exclusive. The representation of patients in an advisory committee does not preclude the need for targeted meetings with patients for specific projects. By means of the examples, we describe how the goal

of the patient involvement might determine the role of the patient representatives and the actual persons to be involved.

9.1 Literature

A governance framework for patient involvement in health research systems has been recently been developed by Miller et al. (2018), based on a literature review.²⁰⁸ The framework includes recommendations for four dimensions: stewardship, financing, creating and sustaining resources, and producing and using research. **Stewardship** relates to the need to define and mobilise a vision for patient involvement in the health research system, define the role of patient involvement in identifying health research priorities, set ethical standards for health research, monitor and evaluate public involvement in the health research system. Securing funds for patient involvement in the health research system and defining the role of patient involvement for the accountable allocation of funds are the two relevant recommendations relating to **financing**. About the **creation and sustaining of resources**, the framework is rather vague, stating that human resource requirements to build, strengthen and sustain patient involvement capacity should be defined, as well as the organizational requirements to build, strengthen and sustain public involvement capacity. Finally, the role of public involvement in **producing and using research** should be defined.²⁰⁸

An interesting practical example of how a research institute could develop a patient involvement strategy was described. A framework for 'consumer and community engagement' was developed for the South Australian Health and Medical Research Institute (SAHMRI), including aspects related to governance, infrastructure, capacity and advocacy, which have been described in literature as organizational success factors for patient involvement in health research.²⁰⁹

Lessons learned from this example are that there are, on the one hand, conditions that foster successful patient involvement and, on the other hand, strategies and actions that enable patient involvement in a research process. Key conditions include, amongst others, an **organization-wide policy** that acknowledges patients as key stakeholders, with **mutual respect** to one another's different knowledge and experience, resources, formal and informal support networks. Strategies and actions include, for



example, developing **strategic partnerships** with patient organizations, make patient involvement mandatory in grant applications (e.g. for pragmatic trials), make sure patient contributions influence the research and communicate to patients how their contributions influenced the research.

Another example from the literature worth mentioning is the “Handreiking Patiëntenparticipatie in een (academisch) ziekenhuis”.²¹⁰ Even though this guidance is not focusing on patient involvement in health policy research, it describes nicely the requirements for a sustainable and meaningful involvement of patients in research. Anchoring of patient involvement in the **structure** of the organization, creating or fostering a **culture** that is open to and supports patient involvement, and **practice** where patients and professionals meet regularly to ensure the previous conditions are met, are key features of a sustainable and meaningful patient involvement strategy within a research organization. By creating procedures, assigning a responsible person for the coordination of patient involvement activities and providing education to researchers and patients, the anchoring within the research structure could be obtained. Similar recommendations were found in other papers.²¹¹

9.2 Patients as structural members of advisory committees, boards or councils

Including patients as members of a governing board, an advisory committee or a council is a structural approach to patient involvement. It helps to ensure organizational embeddedness of patient involvement. At KCE, patient umbrella organizations and sickness funds, as representatives of healthcare service users, are part of the board and they have voting rights. As such they co-decide on the governance of the agency.

Involvement of patients in existing governance or advisory structures usually comes along with normative provisions that characterize the role and procedures of patient involvement.

In an example of bio-banks, Boeckhout et al. state that the roles of the patient organizations in the governance of the biobanks should be fixed in terms of reference, especially when the organizations have particular interests and speak as advocates for their population.²¹²

Inclusion of patients in advisory committees is particularly useful if there are frequent, project-independent strategic or practical issues to discuss on an organizational level. An advisory board could act as a sounding board for the patient involvement coordinator, principal investigators or management of a research organization, but could also put own issues on the table for discussion.

9.2.1 German Federal Joint Committee (G-BA)

In Germany, the Council of the Directorate of the Federal Joint Committee for Health (Gemeinsame Bundesausschuss, G-BA), and the Institute of Quality and Efficiency in Health (Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen, IQWiG) ensure public and patient participation in HTA by informally incorporating 5 representatives of patient associations in their meetings of the General Council. Patient representatives contribute opinions and experiences, suggest technologies to be evaluated, evaluate the relevance of the outcome measures to patients, and provide comments to draft protocols and preliminary reports. Patient representatives also participate in G-BA events as speakers, facilitators and participants in discussions.

Patient involvement has been established by a **legal act** in 2003 (the Patient Involvement Act). The Patient Involvement Act sets forth the criteria an organization must fulfil to be recognized by the Federal Ministry of Health as a leading nationwide advocacy group. Independence of third parties is an important criterion for accreditation and nomination. The selection of participants requires nomination by patient associations accredited by the Ministry.

Several **statutory measures** have been taken to facilitate the involvement of patient representatives, such as reimbursement of travel expenses, compensation for loss of earnings up to a maximum sum and lump sum as representation allowance.

A **patient involvement specialist team** has been established within G-BA to organize the discussions with patient representatives, help them to submit requests. They provide methodological and legal advice, training, content support and help with discussion documents. The team also supports organizations with the nomination procedures for patient representatives. In



2016, the team established an online portal for communication, joint work and education. Internal appointment and collaboration rules for the relevant patient organizations and representatives are defined.

Moreover, patient participation and procedural rights of patient representatives have been included in the G-BA by-laws. The rights to request a decision is far-reaching: when the relevant patient organization requests a decision, the request must be discussed, a formal decision made about whether or not to perform an HTA on the requested topic. If the conditions for an HTA are met, the G-BA has to initiate the assessment and appraisal process and take a decision about reimbursement. An example is provided in Box 4.

Box 4 – Example of a decision request from a patient organisation to the G-BA

The Federal Association of Children with Heart Disease proposed the topic *Newborn Screening for Critical Congenital Heart Disease Using Pulse Oximetry*. The association is a member of the German Disability Council. They were supported by medical experts, such as the German Association of Paediatric Cardiology. The G-BA patient involvement specialist team reviewed the information and drafted the request according to G-BA conditions. After internal discussion and decision, the patient association submitted the request to the G-BA.

9.2.2 National Institute for Health and Care Excellence (NICE)

NICE's policy for public and patient involvement relies on two main principles:

1. Lay people, and organisations representing their interests, should have opportunities to contribute to developing NICE guidance, advice and quality standards, and support their implementation.
2. Because of this contribution, NICE's guidance and other products have a greater focus and relevance for the people most directly affected by our recommendations.

NICE commits, in policy-research related statements to ensuring "that all NICE advisory committees and working groups have at least two lay members (patients, service users, carers or members of the public)"(verbatim²¹³)

Patients can contribute to the guideline development process in many different ways,^{bbb} one of which is to join the committee working on a guideline. In the **guideline development committees**, people are included for their individual experience and do not represent their organizations. In all guideline committees, also at least 2 lay members are involved. Lay members are considered to be part of the public stakeholders. Public stakeholders encompass national patient, service user, carer and community organizations that represent the interests of people whose health or care is covered by the guideline. The guideline development committee considers for each comment received during the public consultation^{ccc} whether they require changes in the draft guideline. The committee formulates a response to each comment. If changes in the guideline have been made, this is made clear in the response, if not, it is explained why no changes have been made. All comments and responses are published on the NICE web-site. The committee may also request input from individual patients through questionnaires.

^{bbb} <https://www.nice.org.uk/process/pmg22/chapter/how-you-can-get-involved>

^{ccc} Once a draft guideline is ready, a public consultation takes place. Consultation is defined by NICE as giving a chance to stakeholders to comment on a draft scope or guideline.



Citizens are involved in a **Citizen Council**, consisting of 30 people who represent the social diversity of the British population.

Organization and coordination of patient involvement activities at NICE

A specific **patient involvement unit** (the Patient Involvement Programme, PIP) coordinates and supports patient involvement through information and training activities. The programme works across all NICE activities to make sure that patients and the public can participate meaningfully in NICE's activities (i.e. not limited to research). The unit is also in charge of the recruitment and accreditation, financial support, the development of versions for patients of the reports and the evaluation of the activities.

PIP supports between 200 and 250 individual lay committee members and experts. They identify experts to offer their expertise to the committees. In 2017-2018, they identified 82 patient experts for technology appraisal, highly specialized technologies and medical technologies committees and 23 patient experts for the Scientific Advice Programme.

Experience with patient involvement and evaluation

In 2017-2018, NICE evaluated the participation of expert patients in NICE's committees. The perceptions of patients varied depending on the treated subject and previous experience with the expert committees. However, 91% rated their experience as 'good' or 'excellent' in 2017-2018. Highlighted issues were the technical language used regarding clinical aspects, quantitative evidence being preferred over qualitative evidence. The important role of the moderator was also highlighted by the participants. The contributions of patients thus depend, to a large extent, on the moderator's abilities to actively involve them in the debate.

Based on the results of the evaluation, concrete actions were taken, such as the revision of the lay member information packs.

A few changes to the recruitment have been made as well:

- increasing the proportion of advisory committee positions of black, Asian and minority ethnic groups
- establishment of a PIP Expert Panel of patients and the public.

The aim of the **Patient Involvement Programme Panel** is to provide an expanding pool of patient and public expertise with knowledge and experience of NICE's work to contribute to NICE committees, which makes it easier to identify people with specialist input (as (patient) experts or reviewers) or members, without having to go through an open recruitment process on each occasion. Nevertheless, the idea is to refresh the panel on a regular basis.

9.2.3 Canadian Agency for Drugs and Technologies in Health

The Canadian Agency for Drugs and Technologies in Health (CADTH) applies different strategies to involve patients in its work: stakeholder feedback, use of patient input templates, synthesis of published literature, primary qualitative research and expert committee participation.²¹⁴

The number of patient- or public members involved in the expert committees varies depending on the programme. For the Common Drug Reviews, two public members are included, no patients; for the Pan-Canadian Oncology Drug Review includes three patients in the expert committee, the "Optimal Use" programme includes one or two public members, no patients in the expert committee and in the "Scientific Advice" Programme, no patients are included.²¹⁴ CADTH's Board of Directors also has two public members.

In some jurisdictions, the expert committees have a mandate to provide recommendations on the funding and use of health technologies. Patient input can be presented to the committee for deliberation either by patient representatives or public members of the public, depending on the committee. In both cases, patient and public members have the same rights and are held to the same terms of reference and conflict of interest guidelines as other expert committee members.²¹⁴ Patient or public members have voting rights.

Recruitment of patients or lay members varies depending on the topic, sometimes via patient organizations, sometimes via healthcare professionals, in hospitals or clinics. Identification and recruitment of patients or public members that can fulfill the role of expert committee member is often a challenge. Most programs have developed selection criteria for potential members. Very important is that the members can represent the broad perspective of patients who may use the technology



under review and have the confidence to meaningfully engage in the deliberations.²¹⁴ They need to be able to act with integrity, independent of specific interests and be able to respect a diverse range of values and beliefs.^{ddd}

Presence on a monthly full day in-person meeting is required. Representatives have to come prepared, extensive preparation might be necessary. Representatives receive a honorarium.

Experience with patient involvement and evaluation

Formal evaluation of patient involvement through membership of an expert committee for one specific HTA was performed in the *Centre Hospitalier Universitaire* de Québec.²¹⁵ Results showed that patient involvement through membership of the expert committee can effectively help identify critical implementation issues and strategies, clarify input from other stakeholders, inform the development of recommendations that reflect patient needs and ensure recommendations are accessible to the patients and families who will be impacted by them.²¹⁶

Recently, CADTH published a framework for patient engagement in HTA, following a critical self-reflection on its approaches using the values and quality standards for patient involvement in HTA of the HTAi Patient and Citizen Involvement Interest Group^{eee} as a basis.^{fff} For each of the values, they identified best practices from other Canadian organizations from which they could learn and on the basis of which they could adapt their own patient involvement initiatives.

ddd <https://www.cadth.ca/become-involved-cadth>

eee <https://htai.org/interest-groups/pcig/>

fff <https://www.cadth.ca/cadth-framework-patient-engagement-health-technology-assessment>

9.3 Patients as advisors

Inviting patients to ad hoc consultation meetings, to discuss particular aspects of research, or inviting patients to provide written input or feedback on draft documents are patient involvement approaches that are frequently applied in health policy research projects. Also KCE has involved patients in its research projects in this way (see chapter 13).

9.3.1 Examples from the Netherlands

In the Netherlands, several guidelines for patient involvement in research are based on the principle of patient consultation as a minimally required approach. This can be embedded consultation or targeted consultation.^{23, 212, 217-219}

“Participatiekompas.nl” is a platform for researchers, patients and policy makers, gathering knowledge and experiences with patient involvement in research, healthcare policy and healthcare.⁹⁹⁹ Several tools, methods and publications are provided to patients who want to be involved or researchers who want to involve patients in their research. One of the tools presented is the POWER-tool, a tool to help researchers involving patient representatives in choosing relevant outcome measures during rare disease clinical trials.²²⁰

The application of the tool foresees two face to face meetings with patients: a first meeting to discuss important aspects of the disease according to patients and the desired improvements from treatment, a second meeting to discuss the study protocol prepared by the researchers based on the outcomes of the first meeting.

Similar examples from the Netherlands are provided by Shölvinck et al. (2017)²¹⁰, Boeckhout et al. (2014)²¹², and de Wit et al. (2016)²¹⁸

999 <https://participatiekompas.nl>, accessed 6 August 2019. Participatiekompas.nl was established with support of ZonMW (www.zonmw.nl) and the VSB Fund (www.vsbfonds.nl/), and facilitated by PGOsupport (www.pgosupport.nl/).



9.3.2 National Institute for Health and Care Excellence (NICE)

Besides involvement in expert committees, NICE also gives the opportunity to patients to give evidence and testimony that can inform the development of NICE guidance and quality standards.²¹³ More specifically, patients can:

- attend a workshop, if one is held, to discuss the scope (which lists what the guideline will and will not cover);
- provide evidence if the guideline developer makes a 'call for evidence';
- comment on the draft scope and the draft guideline, including on equality issues;
- help NICE to promote the guidelines and put them into practice;
- tell NICE about reasons a guideline might need updating earlier than planned and contributing to consultation on whether to update a guideline.

All stakeholder organizations have to register their interest to be involved in the guideline development on a particular topic via an online form.

For participation in the **workshops**, NICE mainly works with expert patient representatives, i.e. people that represent an organization and are experts in guideline development. Organizations are asked to nominate one delegate to coordinate and consolidate the input from the members of the organization. Also for the guideline consultation, organizations are asked to combine their comments into one response.

The developer of a guideline might assess the relevance and acceptability of a guideline with people affected by the guideline. This can happen through a group discussion, interview or survey or through feedback on the draft guideline. Once a draft guideline is ready, a public consultation takes place. Consultation is defined by NICE as giving a chance to stakeholders to comment on a draft scope or guideline.

New since 2018 is the voluntary and community sector (VCS) evidence submissions for diagnostic and interventional procedures. The submissions enable VCS organisations to share patient data, perspectives, and issues that might complement or inform the published evidence and committee

discussions. Previously only individual patients were invited to contribute evidence.

Evaluation of patient consultation

Involvement of patients through questionnaires (i.e. committees requesting written information from patients with experience with the procedures considered) was assessed. Committee members' views on patients' input were asked and received for 7 out of 17 product assessments for which patient input was sought. According to the respondents, the input from patients had an impact on the committee's decision-making. Assessment of 'impact' varied across committee members but the majority agreed it reinforced the other evidence.

Two examples of patient input in health technology assessments are described in Box 5.

Box 5 – Examples of patient input in health technology assessments at NICE

Example 1: HTA of joint fusion surgery for low back pain

NICE received 15 questionnaires from patients who had had joint fusion surgery for low back pain. The published evidence demonstrated the procedure to be safe and effective. Information from patients identified that people commonly had to use crutches for a number of weeks following surgery. The committee added a comment to the guidance to reflect this.

Example 2: HTA of radiation therapy for Dupuytren's disease

NICE received 34 questionnaires from patients who had had radiation therapy for Dupuytren's disease. The committee noted that the patient feedback demonstrated a lack of understanding from the patients of the purpose of the procedure. The committee included a comment in the guidance suggesting clinicians should provide patients with clear, written information about the procedure and its purpose.



9.3.3 Healthcare Improvement Scotland^{hhh}

The Scottish Medicines Consortium (SMC) works in partnership with patient groups to gather information through **patient group submissions**.

Companies submitting a file to SMC must include a 'Summary Information for Submitting Patient Groups' in their submission, using a specific form. This summary information is used by the SMC Public Involvement Team to inform relevant patient groups about ongoing appraisals for which patient group submissions are requested. The SMC Public Involvement Team identifies patient groups for each appraisal, and encourages and provides support to them to provide input.

The 'Summary Information for Submitting Patient Groups' prepared by the companies should be a patient/public friendly version of their submission. Companies are advised to focus on the impact and implications for patients, such as:

- Severity of the condition
- Need for the medicine, including level of unmet need and how the medicine addresses it
- Added value of medicine for patient and patient's carer/family including secondary trial end-points including those related to Quality of Life
- Key side effects and the impact on Quality of Life

Representatives of patient groups identified by the SMC Public Involvement Team may wish to obtain additional information from the submitting company about the treatment(s) under consideration.

The Association of the British Pharmaceutical Industry (ABPI) has developed a code of practice for the development of "Summary Information for Submitting Patient Groups", and relationships with patient groups. The code of practice specifies, amongst others, that information about prescription only medicines made available to the public must be factual and

presented in a balanced way. Companies should be able to substantiate information with scientific evidence.

The submission should be sufficiently concise (5-10 pages), structured as questions and answers formulated in plain non-technical English.

The submission should contain the following parts:

- Front page, including the approved and proprietary name of the product, the submission date and name of the company, name and position of the main contact person for patient groups.
- Question 1: What condition is the medicine to be used for? Brief overview of the condition and the target population and selected subgroup of the licensed indication.
- Question 2: How is this condition currently managed in Scotland? Outline of the current patient pathway and current treatment(s) likely to be displaced by the medicine under review, which may include non-medicine treatment options. Consideration of the severity of the condition and the implications for patients.
- Question 3: How does the medicine work? How might the medicine be different and why might this be relevant to the way patients are managed?
- Question 4: How effective is this medicine and is it different from other medicines currently available to treat this condition? Detail of any unmet need and how this is addressed by the medicine. Brief and simple summary of the clinical trial results. Description of outcomes that are likely to be most important to the patient. Advantages and disadvantages from a patient perspective compared to current treatment(s)? Factual information and balanced presentation in a balanced way. Presentation of current body of evidence relating to the medicine and its benefit/risk profile.

hhh

<https://www.scottishmedicines.org.uk/making-a-submission/>



- Question 5: How is the medicine administered and how will this affect patients and carers? Form, frequency, handling and self-administration/or otherwise. Consideration of the impact on patient care, such as avoiding the need for hospital visit.
- Question 6: What are the side effects of this medicine and how are they managed? Main side effects that are likely to be experienced.
- Question 7: What is the quality of life impact of this medicine on patients and their carers? What is likely to be most important for the patient and patient's carer/family. Added value of the medicine for patients and carers compared to current treatment(s)?

Further online information about the medicine which patient groups may find useful might also be provided, such as published clinical trial data, publicly available regulatory documents regarding this medicine (e.g. Public Assessment Report), patient information materials and websites.

9.3.4 *Canadian Agency for Drugs and Technologies in Health*

The Canadian Agency for Drugs and Technologies in Health (CADTH) has developed different mechanisms and structures for patient involvement.

CADTH includes patient input resulting from a patient consultation process in its scientific advice development process. Patients are consulted in two ways:

- Via information provided by the companies that apply for scientific advice in the context of the CADTH Scientific Advice Program. Companies have to submit information about their patient engagement activities, i.e. any consultations or interactions with patients or patient groups related to their product development program regarding input on the design of the clinical trials.
- Via patient interviews. CADTH itself contacts at least one relevant patient group for each application for scientific advice, to gather information from patients directly about current therapies and remaining unmet needs. Interviews are performed with patient groups or, if none exists for the condition under consideration, individual patients with the

condition. Interviewees are financially compensated for their involvement.

Similar to the SMC, CADTH asks applicants (companies) to complete a "Patient Drug Information Form", which is a template for providing information about the drug and the planned phase 3 clinical trials. The completed form is used by CADTH during their conversation with patient(s) representative(s) about the application. Patients are required to sign a non-disclosure agreement and report possible conflicts of interest. Confidential information provided by the applicant is shared with patient representatives only if the applicant has given permission for this.

The participation is open to any agent interested in the assessed topic but can vary depending on the type of product and subject. The opening of an assessment report is actively communicated through "CADTH e-mail-Alerts" and its Twitter accounts. There are, however, guidelines on who can participate and electronic forms to submit contributions. Patients and consumers can participate individually through the website. The contributions are valued by an independent evaluation committee who considers whether they should have an influence on the recommendations.

Experience with patient involvement and evaluation

In 2011, CADTH commissioned an external evaluation on the involvement of patients in the HTA process. Evaluation was performed through forms, with feedback obtained from patient groups, evaluation experts and industry. The number of forms sent ranged between none and 9 depending of the technologies evaluated, with an average of 1,8. Most experts found that the information provided by the patients was relevant. However, the patients indicated that there was not enough time to complete the forms (15 business days). They also point out that the Patient Drug Information form was not extensive enough to cover a large number of aspects, such as the psychological impact.

CADTH recognizes that best practices for involving patients in the early scientific advice program are not yet established but the process will evolve as more experience is built up.



10 PATIENT INVOLVEMENT IN INTERNATIONAL NETWORKS AND ORGANISATIONS

Patient involvement in health policy research has become a topic of major interest at several international networks of public agencies, such as G-I-Nⁱⁱⁱ, EUnetHTA^{jii}, INAHTA^{kkk}, RedETS^{lll} and HTAi^{mmm}, and of international organisations such as the EMAⁿⁿⁿ and the FDA^{ooo}. Some networks are currently working on guidance document for patient involvement, either HTA (EUnetHTA, INAHTA) or regulatory assessments (EMA, FDA), or have already developed a guidance document (G-I-N, RedETS, HTAi).

10.1 EUnetHTA

In September 2017, EUnetHTA established a task group on the involvement of patients, consumers and healthcare providers in HTA. The aim of the task group is to develop procedures to involve patients and consumers in “early dialogues” (EDs) and HTAs.^{ppp} Early dialogues are a mechanism to inform manufacturers about the evidence requirements of HTA bodies to make their relative effectiveness assessments and draw their conclusions (e.g. what are appropriate comparators and comparisons). Joint assessments are assessments jointly produced by at least four EUnetHTA partners in different countries and are based on a submission from the industry. The joint assessment is coordinated centrally by EUnetHTA. This is the main difference with collaborative assessments, which are coordinated in a decentralized manner, e.g. by an individual HTA agency. Collaborative

assessments are basically done for topics that are on the national work programmes of at least two agencies, who wish to collaborate on the assessment.

Stated **goals** of patient involvement in joint and collaborative HTAs are to:

- elicit patients’ input on aspects regarding their condition, currently available treatments, and expectations with respect to new treatments
- identify subgroups and possible effect modifiers
- understand how their condition impacts upon quality of life
- gather information on outcomes that are important and relevant from a patient’s perspective
- gain insight into issues that are of an ethical or social nature (i.e. inform the ethical and social checklist of the relative effectiveness assessment)

10.1.1 Patient involvement in early dialogues

EUnetHTA describes three approaches for involving patients and consumers in early dialogues:

1. Individual patients living with the condition are interviewed in their own language to provide feedback. Because the aim of EUnetHTA is to stimulate collaboration amongst HTA agencies, the minutes must be translated to English and provided to the collaborating HTA bodies. The risk of issues arising due to conflicts of interest or confidentiality is considered to be low in this approach.

ⁱⁱⁱ G-I-N is the Guidelines International Network

^{jii} EUnetHTA is the European Network for Health technology Assessment

^{kkk} INAHTA is the International Network for Health Technology Assessment Agencies

^{lll} RedETS is the Spanish Network of Agencies for Assessing National Health System Technologies and Performance

^{mmm} HTAi stands for Health Technology Assessment international, a global, non-profit, scientific and professional society for all those who produce, use or encounter health technology assessment (HTA).

ⁿⁿⁿ EMA is the European Medicines Agency

^{ooo} FDA is the US Food and Drug Administration

^{ppp} <https://www.eunetha.eu/wp-content/uploads/2018/06/20180126-Patient-involvement-in-JA3-Minutes-FINAL.pdf>



2. A patient representative could provide general feedback and comment on issues identified by HTA bodies. Similar to the previous approach, an interview would take place in the patient representative's own language and the minutes would be translated into English. The risk of issues due to conflicts of interest or confidentiality is considered to be high in this approach.
3. A patient representative would provide general feedback, comment on issues identified by HTA Bodies, and also address specific questions or issues identified by HTA Bodies. In addition to an interview that would be conducted in English by the EUnetHTA ED scientific coordinator and rapporteur, the patient representative would attend the e-meeting between HTA Bodies where the list of issues is discussed as well as the face-to-face meeting with the company. The risk of issues arising due to conflicts of interest or confidentiality is considered to be high in this approach.

10.1.2 Patient involvement in joint and collaborative HTAs

EUnetHTA describes four approaches for involving patients in joint and collaborative assessments:

1. Open call for patient representatives on the EUnetHTA website and/or distributed to the patient and consumer organisations of the HTA Network stakeholder pool. Patient organisations are asked to complete a modified version of the HTAi questionnaire^{qqq}. The information informs the development of the PICO (population, intervention, comparators, outcomes).
2. Semi-structured interviews with individual patients recruited via EU patient organisations. The HTAi questionnaire is used as a starting point and complemented with some questions from the EUnetHTA HTA Core Model^{rrr}. Patients receive the questions in advance before participating

in a telephone call. The call is recorded, transcribed, and provided to the patient for validation.

3. Scoping e-meeting with the EUnetHTA assessors and coordinator, without the manufacturer: The draft PICO is made available to the patient representatives for commenting.
4. A focus group with a group of individual patients recruited via national patient organisations. A moderator guides the discussions in the patients' own language using a semi-structured interview. The questions can be based on the HTAi questionnaire or other tools and could be complemented with questions from the EUnetHTA HTA Core Model. The discussion is recorded, transcribed, and analysed. Focus groups may be appropriate in specific situations, but due to resource implications for both patients and EUnetHTA partners, this could only be done for a limited number of assessments.

In all these approaches, the involvement of patients would take place **early in the HTA process**, to gather a general patient perspective and to help define the **scope** of the assessment.

10.1.3 Eligibility rules for patients and consumers^{sss}

The *HTA Network Patients and Consumers Stakeholder Pool* developed draft rules for patients and consumers organisations within the Network:

- Not-for-profit
- Legitimacy (legitimate claim to represent patients and consumers across the EU)
- Legal entity (legally established in the EU – with an EU focus and independent)

^{qqq} <https://htai.org/interest-groups/pcig/resources/for-hta-agencies-and-policy-makers/>

^{rrr} <https://www.eunetha.eu/hta-core-model/>

^{sss} <https://www.eunetha.eu/wp-content/uploads/2018/06/20180126-Patient-involvement-in-JA3-Minutes-FINAL.pdf>



- Structure (governing bodies elected by their members – corporates are excluded)
- Accountability (adequate means and procedures to consult and communicate with members)
- Transparency (registered status, disclosure of mission and objectives, list of members (governing bodies and geographical spread), sources of funding, annual financial statement, code of conduct for relations with funders)
- Other criteria still under consideration are:
- Diversity of funding (financial conflicts of interest, thresholds, diversification)
- Geographical spread (25% of member states, i.e. 7 countries)
- Disclosure of material and immaterial benefit
- Other organisations

10.1.4 Experience with patient and consumer involvement so far

EUnetHTA has involved patients in three **Early Dialogues** (EDs). In one instance, the ED coordinator and a rapporteur conducted an interview with one patient that was recruited from an EU patient organisation. While the patient's feedback was used in the preparation of the common and individual HTA bodies' positions regarding the evidence requirements, it was considered that it would be better to have more than one patient giving his input, preferably from different countries. Another learning was that patients should be given sufficient time to review the Briefing Book, presuming that companies are willing to share the briefing book with them. If the latter is not the case, effective patient involvement in EDs becomes difficult. Finally, it was concluded that patient input should be visible in the final recommendations.

Experience with actual patient involvement in **joint and collaborative assessments** is yet limited within EUnetHTA, but there is ongoing or recently finished limited experience with each of the approaches proposed by the working group:

- The open call for patient representatives is currently being tested.
- The semi-structured interview with individual patients was used in two joint assessments of pharmaceuticals. It allowed interviewers to ask more in-depth questions to patients.
- A scoping e-meeting with the EUnetHTA assessors and coordinator (without manufacturer) has been done for one collaborative assessment and another is planned.
- A focus group with a group of individual patients recruited via national patient organisations was conducted for one collaborative assessment and two were planned (as of September 2018) for an on-going joint assessment.

10.2 RedETS

RedETS^{ttt} is the Spanish Network of Agencies for Assessing National Health System Technologies and Performance. RedETS started with the development of patient involvement activities in 2016. RedETS published a guide for patient involvement in HTA, including a comprehensive and flexible framework with proposed tools to involve patients effectively in the assessment process.²¹ The guidance is based on a systematic literature review, semi-structured interviews with HTA doers and policy makers, and a Delphi consultation with patient organisations.

RedETS procedures are developed for patient selection and recruitment, and patient involvement in RedETS activities. The proposed approach is now being pilot tested by member agencies. During the pilot studies, patients will at least participate in the protocol and preliminary version of the report.

^{ttt} <http://www.redets.mscbs.gob.es/en/home.htm>



10.2.1 Concepts

RedETS defines “patients” as people with the health problem, caregivers, representatives of patient organisations, or users of the health system. RedETS makes a further distinction between individual patients and expert patients (Box 6).²¹

Different roles of patients and healthcare users to be involved are defined, depending on the research phase. For example, for the identification and prioritization of technologies to evaluate, contributions of citizens or users of the health system are requested in order to avoid the inherent biases of people affected by specific diseases. When experiences about a disease are to be incorporated in an HTA, information from a group of patients affected by that health problem will be collected.

Box 6 – RedETS’ definitions of key concepts

Patients encompass people with the health problem, caregivers, and representatives of patient organisations.

Individual patients are people with experiential knowledge. They know what it means to live with a disease or health condition. They can contribute valuable perspectives on the impact (positive or negative) of the current or future health technologies from their own individual perspective.

Expert patients are patients who are familiar with HTA processes, objectives and have experiential knowledge about the health condition, either from personal experience or from the collection of other patients’ experiences (e.g. if they are representatives of a patient organization).

Caregivers or relatives can contribute by giving their ideas on the patient’s perspective if the patient is unable to contribute himself. Besides this, they have experiential knowledge as a caregiver.

Patient organizations are formal organizations that represent those affected by a certain disease or health condition and may undertake actions to claim the rights of their members, improve their care, treatments and quality of life.

Citizens, consumers or users of the health system are used in general decisions, such as the selection and prioritization of technologies to be evaluated.

10.2.2 Principles

RedETS defines goals for patient involvement for each stage in the HTA process: the identification and prioritisation of topics phase, the scoping phase, the assessment phase, the recommendations phase, the reviewing phase and the dissemination phase (Figure 2).

RedETS states that it is important to already consider in the preparation of the protocol what is expected from patients in terms of contributions, to be able to plan and guide the design of the patient involvement, the information that should be given to patients about their role, and the management of relationships between the patients and the rest of the participating actors (evaluators, clinicians, etc.).

Figure 2 – Objectives of patient involvement at different phases in the HTA process (RedETS)



Source: Adapted from RedETS²¹



10.2.3 Levels of involvement

RedETS identifies three levels of involvement: communication, consultation and engagement (Table 10). In consultations or qualitative primary investigations, RedETS recommends to pursue a maximum of variation and saturation of the results to achieve greater representativeness of the groups.

Table 10 – Levels of involvement applied by RedETS

Level of involvement	Clarification
Communication	<i>What?</i> Patients receive information about the technologies evaluated and disseminate it amongst other patients, caregivers or users. <i>How?</i> Communication can happen through newsletters, web-pages, social networks, media or face-to-face meetings.
Consultation	<i>What?</i> Patients respond to inquiries made by the HTA agencies about their needs, values and preferences. <i>How?</i> This can be through telephone, email, web-surveys, etc. Consultation can also involve the review of preliminary versions of an HTA report.
Engagement	<i>What?</i> Patients collaborate with assessors, exchange information and participate in the decision making at different stages of the HTA (e.g. identifying and prioritizing the technologies to be evaluated; helping to identify the objectives and patient-relevant outcomes; contributing values and preferences to the scientific evidence base; helping to adapt the reports to make them comprehensible to the majority of patients; collaborating in dissemination activities). <i>How?</i> Patients can contribute individually or through their integration in committees or working groups.

Source: Adapted from RedETS²¹

10.2.4 Design and procedures

RedETS describes in its guidance different methods and tools that have been used by HTA agencies in the past and complements them with other methods that could be used, based on the proposals made by EUnetHTA and GRADE. The network highlights that there is no evidence, nor agreement, on what is the most appropriate method for patient involvement in all stages of HTA.

Table 11 gives an overview of possible methodologies for patient involvement in different phases of the HTA process, identified by RedETS from the literature.

**Table 11 – Possible methodologies for patient involvement in different phases of the HTA process**

Phase	Who?	Level of involvement	Tools
Identification and prioritization of technologies to be evaluated	Patient / citizen organisations	Consultation	Web forms
	Citizens / patients	Consultation	Surveys
	Patient / citizen organisations	Engagement	Meetings, Delphi panels
	Patients or patient organisations	Engagement	Representation in an advisory committee of the agency
	Citizens	Engagement	Citizen's jury
Defining the problem, the objectives and scope of the assessment	Patients	Consultation	Review of qualitative literature
	Patients or patient organisations	Engagement	Protocol review
	Patients or patient organisations	Engagement	Expert panels
	Patients	Engagement	Templates to share experiences, values and preferences
	Patients	Consultation	Interviews and focus groups
	Patients or patient organisations	Consultation	Analysis of web pages of patient associations and other internet sources
Assessing the scientific literature and other sources of information (including experiential knowledge)	Patients or patient organisations	Engagement	Expert panel
	Patients	Indirect involvement	Systematic or narrative reviews
	Patients or patient organisations	Indirect involvement	Analysis of Web pages, blogs and social networks
	Citizens / patients	Consultation	Surveys
	Citizens / patients	Consultation	Interviews and focus groups
	Patients	Engagement	Templates of forms to share experiences, values and preferences
Formulating recommendations	Patients	Engagement	Expert panels
	Citizens / patients	Consultation	Discussion groups / citizen panel
	Patients or patient organisations	Engagement	Wiki
Reviewing	General public	Communication	Publication of the draft report on the web-site of the healthcare ministry
	Patients or patient organisations	Engagement	Expert panels



Dissemination	Patients or patient organisations	Consultation	Review forms
	Patients / citizens organisations	Consultation	Public presentation, press release
	Citizens / patients / citizens or patient organisations	Communication	Web publication, active and passive dissemination
	Patients	Consultation	Review of the version for patients

Source: RedETS²¹

10.2.5 Patient selection and recruitment

Who and how many patients to involve in the research process depends on the **objectives** to be achieved, the **expected contributions**, the required **representativeness** and the available **resources** (time and money). It is useful to define a number of selection criteria.

As for the question on how to select potential contributors, RedETS recommends to combine a democratic approach with a technocratic approach. The democratic approach implies an open procedure that facilitates free participation; the technocratic approach actively seeks the representation of those patients and / or organizations that can make specific contributions. This design avoids exclusive reliance on passive appeals (e.g. published online). According to the literature, there is evidence that this is not effective if there are no prior relationships between the requesting agency and the expected contributor.

The next relevant question is which type of participant to involve. RedETS recommends the use of the PROGRESS framework²²¹ to identify possible groups of interest. PROGRESS includes 8 dimensions that describe social differences in health: place of home; race, ethnicity, culture; occupation or work experiences; gender; religion; educational level (including health literacy); socio-economic status; and social capital and social exclusion.

It is meant to ensure that the research performed encompasses an equity lens.²²¹ RedETS recommends to exclude patients that are also health professionals, managers or researchers, in order to obtain unbiased

contributions. For the same reason, patients and professionals who have a healthcare relationship should not be included in the same working groups, according to RedETS.

Patient recruitment

According to RedETS, the following elements should figure in the invitations to patients:

- Description of the tasks to perform
- Characteristics of the people sought
- Whether you want the selected person to represent a certain group or whether you are looking for personal opinions or experiences
- Estimated time investment and effort
- Whether a compensation is foreseen



As for the criteria for the inclusion of organisations, no consensus was reached amongst the RedETS agencies. During a Delphi panel, only agreement (>70% of the agencies agreed) on two out of six criteria was found

- Organisations should express interest in HTA activities (93.8%)
- Organisations are representative in the field of interest (81.3%)

The four criteria for which no consensus was reached were:

- Selection of organisations depending on the expected benefit of the collaboration to the HTA (50.8%)
- Preference for more professional organisations or organisations with a higher level of scientific and social competence (53.2%)
- Preference for organisations that have a better geographical representation (50%)
- Preference for organisations that have social influence (49.2%)

Declaration of conflicts of interest and confidentiality agreements

According to the guidance of RedETS, patients must make a declaration of potential conflicts of interest, just like other experts or stakeholders involved in the HTA. They should make such declaration for both the individual level and for the organizations they represent. For instance, relationships between patient associations and the industry can generate conflicts of interest. It might be necessary to adapt the declaration of conflicts of interest to individual patients and representatives of organizations. RedETS mentions also briefly that it may also be necessary to sign an agreement confidentiality but not further details are provided on this aspect.

Resources needed for participation

Involving patients in HTA requires both structural and temporary resources, human resources and resources for providing information and giving training. Some resources will have a continuous nature (e.g. for the running of a patient involvement unit), others may be temporary (e.g. for the organisation of a training session for contributors or the participation in meetings at the agency).

10.2.6 Recommendations: short term, medium term, long term

The recommendations of RedETS regarding patient involvement in HTA are summarized in Table 12.

**Table 12 – Recommendations for involving patients in HTA (RedETS)**

Normative and conceptual framework	
Normative framework	Publish and disseminate a regulatory framework for patient participation in HTA in Spain.
Conceptual framework	Follow the conceptual and methodological developments of patient involvement in HTA at the international level.
Design and planning of the involvement of patients in HTA	
Design	Integrate patient involvement in all HTAs, except when the assessment concerns technologies that do not involve direct interaction with patients. Gradually incorporate patient involvement through a combination of methods.
Patient contributions	Establish transparent mechanisms to incorporate and translate the contributions from patients.
Selection and recruitment	Agree on and publish the procedure for selecting and recruiting patients.
Resources	Arrange structural financial and human resources as well as temporary resources to inform and educate patients. Consider incorporating patients into the structure of RedETS or create a unit that coordinates patient involvement. Incorporate patient involvement in the workflow, guaranteeing procedures and adequate times. Develop strategies for informing and educating patients.
Methods of patient involvement in the different phases of HTA	
Identification and prioritization of research topics	Create open spaces for participation and establish transparent involvement mechanisms. Adapt PriTec ^{uuu} to ensure patient involvement. Actively inform patients about existing tools and train them in their use. Consider the use of deliberative methods.
Defining the problem, objectives and scope of the assessment	Ensure open participation. Explore patient-relevant objectives and outcome measures that can complement pre-existing ones.
Assessment	Incorporate considerations about implementation: economic, ethical, organizational, social and patient-related aspects. Incorporate the experiences, values and preferences of patients through literature reviews (including white and gray literature), expert panels and/or consultations and primary studies.
Formulation of recommendations	Include patients in the expert panels. Follow the methods for patient involvement of GRADE.
Review	Include patients as internal reviewers.

^{uuu} PriTec is the prioritization tool used by RedETS for prioritizing technologies for assessment post-introduction.



	Actively promote the participation of patient organizations in the external review and feedback procedures.
Dissemination	Develop patient versions of HTA reports. Publish and disseminate the original HTA reports as well as the patient versions.
Evaluation	Evaluate the patient involvement and publish your results. Establish a calendar for the periodic evaluation of the patient involvement in HTA, allowing for continuous improvement.

Translated from RedETS²¹

Besides recommendations for patient involvement in HTA, RedETS also established **recommendations for the gradual implementation of patient involvement mechanisms**. These are particularly **useful in our Belgian context**, where patient involvement is now starting to be considered.

A progressive implementation strategy in 3 phases is proposed, with actions to be carried out in the short term, the medium term and the long-term. The gradual implementation strategy, slightly modified to fit our particular focus on patient involvement in health policy research, is summarized in Table 13.

Table 13 – Recommendations for the progressive implementation of patient involvement in health policy research

SHORT TERM ACTIONS

Normative and conceptual framework	
Normative framework	Make a public statement of the interest of the agency in the involvement of patients in health policy research. Agree on a normative framework for the involvement of patients in health policy research.
Design and planning of patient involvement in health policy research	
Design	Start with the implementation of patient involvement in some projects (excluding the rapid ones). Incorporate 2 or 3 patient representatives in external expert panels of the selected projects.
Resources	Develop materials to inform and educate patients about research processes, work of KCE and how to contribute to its different phases. Develop a tailored conflict of interest declaration form for patients.
Methods of patient involvement in the different research phases	
Build up experience through pilot studies	Incorporate patients in the external expert group of a health policy research project. Invite them to participate in the protocol review. Give them the opportunity to review the draft report. Prepare, with the help of patients, a summary of the report addressed to patients.

**MEDIUM TERM ACTIONS****Design and planning of the involvement of patients in health policy research**

Design	Expand patient involvement starting with a combination of methods.
Resources	Conduct a training session for researchers and create space for exchange of experiences. Continue to promote patient involvement through dissemination activities.

Methods of patient involvement in the different phases of health policy research

Research	Incorporate patient contributions through the review of the literature and websites of patients and their organizations.
Review	Actively disseminate the calls for review and feedback to the organizations affected by the evaluated technology.

Evaluation of patient involvement

Evaluation	Carry out a qualitative evaluation of the patient involvement. Create a checklist that can facilitate the transparency of the involvement actions and long-term evaluations.
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LONG TERM ACTIONS**Normative and conceptual framework**

Conceptual framework	Review the proposed framework and update the methodological guidance report.
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Design and planning of the involvement of patients in health policy research

Design	Integrate patient involvement mechanisms in all health policy research projects. Expand patient involvement with a combination of methods to cover all possible contributions from patients.
Contributions	Establish mechanisms to incorporate the contributions of patients and document them transparently.
Selection and recruitment	Guarantee representativeness and diversity in the composition and number of participants in panels and consultations.
Resources	Assess the incorporation of patients into the structure of the agency or have a mechanism that coordinates participation. Carry out annual information and training actions for patients and representatives.

Methods of patient involvement in the different phases of health policy research

Identification and prioritization of topics	Adapt existing tools for use by users / patients. Inform and actively train patient organizations in the use of existing tools.
Research	Incorporate the experiences, values and preferences of patients through some type of consultation or primary investigation when literature gives insufficient evidence.

Source: Translated and adapted from RedETS (2016)²¹



10.3 European Medicines Agency

The European Medicines Agency (EMA) is the central European regulatory agency for medicines that evaluates applications for marketing authorization of medicinal products. EMA also monitors the safety of medicines across their lifecycle and provides reliable information on human and veterinary medicines in lay language.

10.3.1 Objectives of patient involvement at EMA

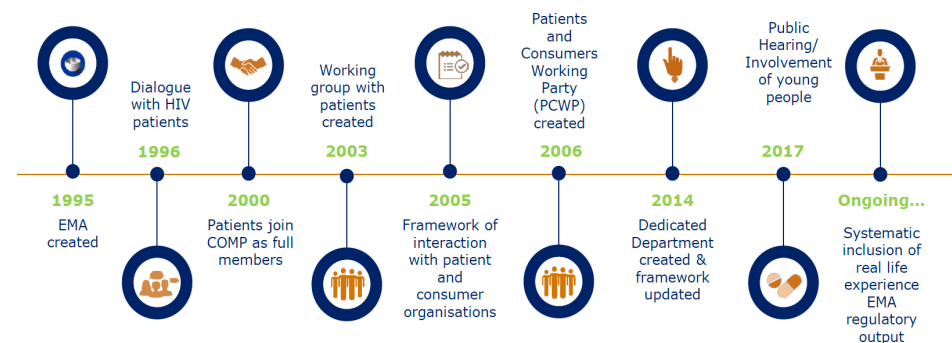
The stated objectives of patient involvement at EMA are:

- To bring the everyday aspects of living with a disease into the scientific discussions and to help bridging the gap between clinical trial data and real world data;
- To improve transparency and trust;
- To increase understanding and dissemination of EMA outcomes;
- To add to the quality of the regulatory outcome.

The underlying rationale is that *all* perspectives are crucial and result in more meaningful discussions for all people concerned.

Figure 3 gives an overview of the history of the experience so far at EMA with patient involvement.

Figure 3 – History of patient and consumer involvement at the EMA^{vv}



COMP: Committee for Orphan Medicinal Products

The Committee for Medicinal Products for Human use (CHMP) of the EMA has put the development of patient involvement procedures in assessment work on the work plan for 2018.^{www}

The objective is “to facilitate participation of patients and consumers in benefit/risk evaluation and related activities, to capture patient’s values and preferences and obtain information on the current use of medicines and their therapeutic environment, all along the lifecycle of the medicines, from early development throughout evaluation and post-marketing surveillance”.^m

The CHMP ultimately wants to incorporate patient involvement in its regular processes to capture and include patient views and preferences in its benefit-risk evaluations.

^{vv} https://www.ema.europa.eu/documents/presentation/presentation-overview-current-patient-involvement-m-mavris_en.pdf

^{www} http://www.ema.europa.eu/docs/en_GB/document_library/Work_programme/2011/01/WC500101505.pdf



10.3.2 Who to involve?

The EMA aims to involve patients and consumers for different activities (Box 7). It organizes an annual training for patients and consumers for an effective involvement.

Box 7 – Categories of patients involved at EMA for different roles

Patients as individual experts

- Scientific advice / protocol assistance procedures
- Scientific Advisory / ad hoc Expert Groups
- Scientific Committee Consultations
- Review of Documents

Patients representing their community

- Management Board
- EMA Scientific Committee(s)

Patients representing their organisations

- Patients and Consumers Working Party
- EMA Consultations
- Workshops

Individual patients are consulted as expert at several stages of the drug evaluation process: pre-submission, during the evaluation and post-authorization.

The EMA has defined explicit eligibility criteria for patient and consumer organisations who wish to be involved in its processes (Box 8). Patient organisations are defined as “*not-for profit organisations which are patient*

focused, and in which patients or carers (the latter when patients are unable to represent themselves) represent a majority of members in their governing bodies”. Consumer organisations are defined as “*not-for profit organisations which defend and promote the general interests of European consumers - citizens as purchasers or users of goods and services*”. Besides patient and consumer organisations, EMA also involves healthcare professional organisations in its processes, which are subject to the same eligibility criteria as the patients and consumer organisations.

The way in which the eligibility is assessed is described in detail and published on the website of EMA.^{xxx} Each year, organisations have to complete a self-declaration of eligibility document. Each year, a random selection of the up to 20% of the organisations is re-assessed in-depth.

EMA can also reach out itself to patient or consumer organisations. The EMA engages with over 35 patient and consumer organisations. Twenty out of these are represented in EMA’s Patients and Consumers Working Party. The EMA selects the organisations to be represented on the Working Party. The term of an organization is three years, and is renewable. The Patients and Consumers Working Party discusses matters that are not product-specific.

Also individual patients, carers and patient representatives can ask to be involved. Like the organisations, individual patients, carers or representatives need to register to express their interest to be involved. EMA has a database with over 200 patients. If individuals are contacted to be involved in a scientific activity, they are asked to provide information on possible conflicts of interest.

Eligibility criteria for organisations are listed in Box 8. One of the crucial ones is the criterion about industry funding. Briefly, if an organization has more

^{xxx} https://www.ema.europa.eu/documents/other/assessment-patient-consumer-healthcare-professional-organisations-compliance-ema-eligibility_en.pdf



than 20% annual budget coming from industry funding, then the funding must come from at least three different companies.^{yyy}

Box 8 – Eligibility criteria for patient and consumer organisations wanting to be involved in EMA processes.

Legitimacy: the organisation should, in principle, be formally established in one of the Member States of the EU/EEA. Organisations not formally established in an EU/EEA Member State may apply to become 'EMA eligible organisations' if they provide additional information that they have specific focus and carry out activities in the EU.

Mission/objectives: the organisation shall have its mission/objectives clearly defined and should agree to have it/them published on the EMA website.

Activities: the organisation shall have, as part of its activities, a specific interest in medicinal products which should be documented (e.g. through a report published on the organisation website).

Representation: the organisation shall be representative of patients or consumers or healthcare professionals throughout the EU/EEA. Organisations already registered at Community level, e.g. in the EU Health Forum, the Council of Europe, are considered to adequately represent patients or consumers or healthcare professionals for involvement in EMA activities.

In case of a lack of European associations for a specific disease or treatment area, the involvement of national organisations may be considered, although preference will be given to European wide-associations. These associations will need to fulfil the same criteria apart from representation, which will be at national level.

If several similar associations exist in different Member States, a choice will be considered on a case-by case basis.

Organisations can also be considered for eligibility as long as they have a European focus and representation, including EU/EEA based office(s) and/or membership covering at least 50% of all EU/EEA Member States.

Structure: the organisation should have governing bodies which are elected by their members, who shall be patients, or consumers, or their elected representatives or healthcare professionals.

Accountability and consultation modalities: statements and opinions of the organisation should reflect the views and opinions of its members and adequate consultation procedures with those members should be in place. In particular, the organisation should ensure that the appropriate flow of information is in place to allow dialogue both ways: from and towards its members.

Transparency: the organisation shall disclose to the EMA its sources of funding both public and private by providing the name of the bodies and their individual financial contribution, both in absolute terms and in terms of overall percentage of the organisation budget. Any relationship with corporate sponsorship should be clear and transparent. This information shall be communicated to the Agency on an annual basis.

For umbrella organisations the list of member associations should be made available to EMA.

The organisation shall follow a code of conduct/policy regulating its relationship with and independence from the sponsors.

The organisation shall publish on its website the registered statutes, sources of funding, and information on their activities.

^{yyy} <https://www.ema.europa.eu/en/partners-networks/patients-consumers/getting-involved>



In addition, patient, consumer and healthcare professional organisations shall commit to take an active part in the interactions with the EMA.

Source: https://www.ema.europa.eu/documents/regulatory-procedural-guideline/criteria-be-fulfilled-patient-consumer-healthcare-professional-organisations-involved-european_en.pdf

The application of the criteria may differ depending on which activity the patients are participating in. Exceptionally, a patient (individual or representative) or health care provider may be involved despite having an identified conflict of interest. This can be relevant, for example, during the evaluation of an orphan medicine.

10.3.3 *Experience with patient involvement*

In 2017, the EMA published the results of a pilot experience with patient involvement in benefit-risk discussions at the CHMP.²²²

First, patients were invited to participate during oral explanations about a specific product, where the EMA product leads considered that patients could bring added value to the benefit-risk discussions and the CHMP wanted to assess the impact of a possible recommendation on the relevant patient population. Patients were involved in 6 oral presentations. At least two patients (or carers) with personal experience and knowledge of the particular disease/condition under evaluation were invited to participate. The selection took relevance of their experience for the topic under discussion into account. Patients completed a potential conflict of interest form and signed a confidentiality agreement, like any other invited expert.

Patients were given a guidance document, explaining the work of the EMA and CHMP, the issues for discussion and their expected role. Patients could fall back on support from EMA staff to help them in case something was not clear and a 'mentor' from the Patients' and Consumers' Working Party. They also received specific questions that would be addressed during the oral explanations and for which their contribution was desired. Patients were

stimulated to share their views and participate actively in the discussions. They were not allowed to vote.

After the oral presentations, participants (patients, CHMP members and EMA staff) were asked via questionnaires to assess their experience with the patient involvement in the discussions.

Patients generally felt that they received sufficient information prior to the oral explanations, both regarding the issues and their role. They felt they were able to express their views and that their comments were taken into account. Their general feeling about their experiences was positive, including feeling part of the process and understanding the regulatory process regarding the medicine under evaluation.

The CHMP members and EMA staff were more differentiated in their response, although the majority (16 out of 22 respondents) found the contributions of patients useful, four respondents were neutral and 2 found the contributions not useful. Overall, they felt that the patients knew the disease under discussion, they actively participated and that this participation was useful. One participant highlighted in the written comments that it is important to warn patients that once they are selected to be part of the expert panel, they should not meet the company or respond to emails from the company. Patient representatives met with the company during the morning before the oral explanation, which made this respondent feel uncomfortable. Another comment made was that it is important to also include patients without experience with the treatment being discussed. In one of the oral presentations, the two patients who participated both had experience with the use of the medicine and were hence not considered neutral with regard to the treatment under consideration.

10.3.4 *Future plans*

EMA will continue to involve patients in oral explanations on a case-by-case basis, i.e. when it is considered that their input could be valuable to the assessment. Additional methods and consultation of patients on a more regular basis will also be tested, e.g. participation in CHMP discussions by teleconference, written consultations for very specific questions at any time



during an evaluation or focus groups. The objective is to create an opportunity for a larger number of patients to contribute.

EMA wishes to identify best practices and issue recommendations with regard to patient involvement.

Another area that is being investigated by the EMA is the elicitation of patient preferences. A collaboration with PREFER, a public-private collaborative research project under the Innovative Medicines Initiative (IMI), is ongoing. The objective of PREFER is to establish recommendations to support development of guidelines for industry, Regulatory Authorities and HTA bodies on how and when to include patient perspectives on benefits and risks of medicinal products.^{zzz} The experiences from the first pilots will be shared with other committees within the EMA (e.g. the Pharmacovigilance Risk Assessment Committee) that are not yet involving patients in their assessment processes.

10.4 Food and Drug Administration

The Food and Drug Administration (FDA) is an agency similar to the EMA but for the U.S. and with a broader remit. The FDA also evaluates marketing authorization requests of medicines, but also of medical devices. Besides that, the FDA is responsible for protecting and promoting public health through the control and supervision of food safety, tobacco products, dietary supplements, cosmetics and animal foods.^{aaaa}

10.4.1 Objectives of patient involvement at the FDA

The FDA recently developed four draft guidance documents for stakeholders (patients, researchers, industry) on how to collect and submit patient experience data and other relevant information from patients and caregivers for medical product and regulatory decision making.²²³, ^{bbbb} Guidance is developed for the following questions (one guidance document per question):

1. Whom do you get input from (sampling)? How do you collect information (operationalization and standardization of collection, analysis and dissemination of patient experience data)?
2. What do you ask, and why? How do you ask non-leading questions that are well understood by a wide range of patients and others?
3. How do you decide what to measure in a clinical trial and select or develop fit-for-purpose clinical outcome assessments?
4. What is an appropriate clinical trial endpoint?

Patient experience data is defined as data “intended to provide information about patients’ experiences with a disease or condition, including the impact (including physical and psychosocial impacts) of a disease or condition, or a related therapy or clinical investigation on patients’ lives; and patient preferences with respect to treatment of their disease or condition”.^{cccc}

A list of possible useful data to collect on patient experiences during the medical product life cycle is provided. The reason for collecting patient experience data is described as follows by the FDA:

^{zzz} <https://www.imi-prefer.eu/>

^{aaaa} www.fda.gov

^{bbbb} https://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM610442.pdf?utm_campaign=FDA%20Issues%20Draft%20Guidance%3A%20Patient-Focused%20Drug%20Development%3A%20Collecting&utm_medium=email&utm_source=Eloqua

^{cccc} Title III, section 3001 of the 21st Century Cures Act (Pub. L. 114-255), amended by section 605 of the FDA Authorization Act of 2017 (Pub. L. 115-52)



“Patients are experts in their own experience of their disease or condition and the ultimate consumers of medical products. The collection of patient experience data is important because it provides an opportunity to inform medical product development and enhance regulatory decision making to better address patients’ needs.”

Moreover, FDA states that

“Patients (including patients serving as advisors) should be meaningfully involved throughout the medical product development process – not only as study subjects but as partners. Engaging patients actively in the development process can potentially improve rates of trial enrolment and retention and increase applicability to patients”.

“Patient experience data is used to help inform clinical trial design, trial endpoint selection, and regulatory reviews including benefit-risk assessments as well as potential labelling (or other communications).”

10.4.2 Who to involve?

FDA uses patients and patient partners as relevant contributors to patient experience data. Definitions are provided in Box 9.

Box 9 – Definition used at FDA in the context of patient involvement to gather patient experience data

A **patient** is any individual with or at risk of a specific condition, whether or not they currently receive any therapy to prevent or treat that condition. Patients are the individuals who directly experiences the benefits and harms associated with medical products.

A **caregiver** is a person who helps a patient with daily activities, healthcare, or any other activities that the patient is unable to perform himself/herself due to illness or disability. The person may or may not have decision-making authority for the patient and is not the patient’s healthcare provider.

A **patient advocate** is an individual or group that advocates for patients’ health or healthcare. The advocate may or may not be part of the target population, and may work to influence healthcare policies or practices.

A **patient partner** is an individual patient, caregiver or patient advocate that engages other stakeholders to ensure the patients’ wants, needs and preferences are represented in activities related to medical product development and evaluation.

A **patient representative** is an individual, who may or may not be part of the target population, who has direct experience with a disease or condition (e.g. a patient or caregiver) and can provide information about a patient’s experience with the disease or condition.

Source: FDA, 2018²²³



10.4.3 Experience with patient involvement

The FDA has taken many initiatives to involve patients in their processes. A few examples are presented in Table 14.

Table 14 – Patient involvement initiatives at the FDA

Initiative	Patient Engagement Collaborative (PEC)	Patient Engagement Advisory Committee (PEAC)	Patient Representative Program (PRP)
Purpose	A forum to discuss and share experiences on patient engagement in medical product development and regulatory discussions	Provides advice to the Commissioner or designee, on complex issues relating to medical devices, the regulation of devices, and their use by patients in a public advisory committee meeting	FDA Patient Representative SM consultants provide direct input to inform the Agency's decision-making associated with medical products for drugs, biologics, and medical devices in a public advisory committee meeting or as part of agency-directed assignments
Meeting Frequency	Quarterly	Annually	Ongoing
Participant status	Patients, Caregivers and Advocates	Patients, Caregivers and Advocates that serve as Special Government Employees**	Patients, Caregivers and Advocates that serve as Special Government Employees**
Topics Covered	Patient engagement operations	Regulatory process and medical product review	Regulatory medical product review

**** Special Government Employees are appointed when conflict of interest restrictions need to be applied to participants for topics that cover specific products or regulatory issues.**



11 EMBEDDED PATIENT INVOLVEMENT PROGRAMMES AND INITIATIVES

With the increasing interest in patient involvement in research, new programmes and even institutions have been established to develop processes for embedded patient involvement in research, for example the James Lind Alliance (JLA)^{dddd} and INVOLVE^{eeee} in the UK, and the Patient-Centered Outcomes Research Institute (PCORI)^{ffff} in the US. In addition, collaborations have been established that are less embedded in an organizational structure but with similar objective, such as the Strategy for Patient Oriented Research (SPOR) in Canada, and Participatiekompas^{gggg} in the Netherlands. Their objective is to promote and support patient involvement activities with tools and guidelines and as such build capacity for patient involvement in research. Finally, multi-stakeholder partnerships have been set up to increase the number of patients having the capability to act as research partners in healthcare research projects, such as the European Patients' Academy (EUPATI)^{hhhh}, which has several national branches within European countries and "Patient Focused Medicines Development" (PFMD), which has a global perspective.

These initiatives may help provide the financial, structural, and educational support necessary for patient engagement in research.²²⁴

11.1 James Lind Alliance

The James Lind Alliance is a publicly funded non-profit initiative that brings together patients, clinicians and carers to identify and prioritize the uncertainties about the effects of a treatment, in order to set the agenda for future clinical research. The JLA is funded by the NIHR in the UK. The *raison d'être* of the JLA is the observed mismatch between the priorities of patients,

clinicians and carers in terms of research evidence and the research being carried out. This leads to inefficient use of research resources and hence high opportunity costs in terms of more valuable research not being performed. The JLA tries to solve this issue by bringing patients, clinicians and caregivers together to identify and prioritize uncertainties and research topics in a particular disease area. While the main and initial scope of the JLA is to identify treatment uncertainties (e.g. uncertainties about the added value of a therapy compared to another therapy, or about whether the treatment is doing more harm than good), also other healthcare interventions (prevention, diagnosis, rehabilitation), care, service organisation and delivery are sometimes addressed.

JLA establishes and facilitates "Priority Setting Partnerships" (PSPs), which are groups of people organized around a specific condition or healthcare area, including patients, carers, families of patients, their advocates and clinicians and other healthcare professionals. The PSPs identify priorities for research which are of direct relevance to patients and clinicians. Prioritization techniques include adapted Delphi techniques; expert panels or nominal group techniques; consensus development conference; electronic nominal group and online voting; interactive research agenda setting and focus groups. The method of using PSPs to identify research priorities is being applied since 2007 and is continuously evolving based on experiences and new evidence on good practices.

The initiative to establish a PSP can come from an individual or a representative of a group of people wanting to set up a PSP. The steering committee of the PSP has many responsibilities, from publicizing the initiative to potential partners, over collecting and collating uncertainties and check these against existing systematic reviews, to developing research questions from the agreed priorities and working with research funders

^{dddd} <http://www.jla.nihr.ac.uk/about-the-james-lind-alliance/>

^{eeee} <https://www.invo.org.uk/>

^{ffff} <https://www.pcori.org/>

^{gggg} <https://participatiekompas.nl>, accessed 6 August 2019. Participatiekompas.nl was established with support of ZonMW (www.zonmw.nl) and the VSB Fund (www.vsbfonds.nl/), and facilitated by PGOsupport (www.pgosupport.nl/).

^{hhhh} <https://www.eupati.eu/>



where necessary to provide any extra information they need. Its responsibilities are fully described in the JLA guidebook, with details on how to approach these.

The PSP is supported by an information specialist, who analyses the data collected, reviews the existing scientific evidence and formulates the research questions that highlight the evidence gaps. The information specialist plays a major role in the entire process. It has to be a skilled person, with experience in literature research, data extraction from the PSP discussions, and formulation of research questions.

Finally, the PSP has a project coordinator, responsible for the day-to-day running of the PSP: organisation of meetings and workshops, management of communications with stakeholders and the wider community etc.

11.2 INVOLVE

INVOLVE is a publicly funded programme within the National Institute for Health Research (NIHR), established in 1996 to support active public involvement in research in the National Health Service, public health and social care research in the UK. It was established as a national advisory group that brings together expertise, insight and experience in the field of public involvement in research. It focusses on public involvement in the identification, prioritisation, design, conduct and dissemination of research. Like the James Lind Alliance, INVOLVE is financed by the National Institute of Health Research (NIHR).

The INVOLVE advisory group has 15 members, encompassing health and social care services users, carers, representatives of voluntary

organisations, and health service and social care practitioners, managers and researchers. It has a Coordinating Centre with 10 staff members.²²⁵

Besides developing guidance for patient involvement in research (e.g. how to involve patients, how to get involved, how to co-produce research), INVOLVE continuously explores new territories. For example, it explored in-depth seven examples of research led by service users or disabled people in order to find out what the role and potential value of user-controlled research.²²⁶ The study was able to demonstrate what can be achieved by small organizations or groups of service users with sometimes small budgets, the motivation of the groups to invest in this type of research, the achievements, the challenges and the things that helped them to succeed.²²⁶ More recently, experts from INVOLVE have done a mapping exercise and survey, with advice and guidance from the Children and Young People's working group, to assess the extent to which children and young people are involved in research as advisers and to assess the barriers and benefits.²²⁷ Several other examples are available on the web-site.

INVOLVE has developed a rich source of information for researchers and the public on public involvement in research. On its web-site, one can find many practical tools and resources for researchers and public members, which are all published on their website www.invo.org.uk. There is a glossaryⁱⁱⁱⁱ, an evidence library^{jjj}, a guidance on how to involve people in research^{kkkk}, including information for research commissioners^{lll}, guidance for giving and receiving feedback to and from patients and the public^{mmmm}, an involvement cost calculatorⁿⁿⁿⁿ and much more. A lot of effort has been put into the user-friendliness of the web-site and accessibility of the

ⁱⁱⁱⁱ <https://www.invo.org.uk/resource-centre/jargon-buster/>

^{jjj} <https://www.invo.org.uk/resource-centre/libraries/evidence-library/>

^{kkkk} <https://www.invo.org.uk/find-out-more/how-to-involve-people/>

^{lll} <https://www.invo.org.uk/find-out-more/how-to-involve-people/information-about-research-commissioning/>

^{mmmm} https://www.clahrc-eoe.nihr.ac.uk/wp-content/uploads/2016/05/Guidance-for-Researchers-PPI-Feedback_2018.pdf

ⁿⁿⁿⁿ <https://www.invo.org.uk/resource-centre/payment-and-recognition-for-public-involvement/involvement-cost-calculator/>



resources. The work of INVOLVE is internationally used and referred to and continues to be developed.²²⁵

INVOLVE activities include publication of resources for researchers, establishment of a library of resources, and leading NIHR projects focused on public involvement in research.

11.3 PCORI

The Patient-Centered Outcomes Research Institute is a US-based funding institute for comparative clinical effectiveness research that requires patient engagement in all funded studies. It was established in 2010 as part of the US's Patient Protection and Affordable Care Act. PCORI has a 21-member governance board and a 17-member methodology committee. The methodology committee defines the methodological standards for research. Several other committees exist within PCORI⁰⁰⁰⁰: an engagement, dissemination and implementation committee, and research transformation committee, a science oversight committee, an executive committee, a finance and administration committee, a governance committee, and a selection committee.

PCORI funds research connected to one of its two research programs: (1) Clinical Effectiveness and Decision Science, and (2) Healthcare Delivery and Disparities Research. Domains in which PCORI funds research include, for example, conditions that place a heavy burden on patients, families, or healthcare systems, chronic conditions, multiple co-existing conditions, rare diseases, conditions with variable outcomes across sub-populations and populations at risk for health disparities.

Besides the two research programs, reflecting the research priorities of PCORI, an evaluation and analysis program performs strategic portfolio analysis for PCORI and evaluates patient and other stakeholder involvement in research funded by PCORI. An "Engagement Program" is established to

gives patients, caregivers, clinicians, and other healthcare stakeholders the opportunity to be meaningfully involved in all PCORI's activities.

Key features of research funded by PCORI are: comparison of at least two healthcare options, focus on outcomes that are meaningful to patients, engaging patients and other stakeholders at every stage, studying benefits and harms of care delivered in real-world settings, adhering to PCORI's methodological standards and likely to improve current clinical practice.

A research infrastructure program was established, to support the sustainability and enhancement of PCORnet, the national patient-centred clinical research network. PCORnet is a "network of networks", combining the strengths of large amounts of health information (e.g. from electronic health records) with strong partnerships between patients, clinicians and other stakeholders. It can support researchers for example in conducting pragmatic trials that include clinical and patient-reported outcomes. PCORI funds and manages projects that can enhance and optimize the network infrastructure and promote the sustainability of PCORnet.

A published overview of self-reported patient involvement activities in projects funded by PCORI found that 90% of the projects involved patients or healthcare users.²²⁸ Fifteen percent of the projects involved only one patient, while 56% of projects reported engaging six or more patients. Most projects reported engaging patients as consultants (35%) or collaborators (53%). Only 6% involved patients as co-leaders. Patients were most commonly involved at the stages of research question development, proposal development, study design, data collection, topic solicitation, and results review.²²⁸

PCORI has had an impact on the patient involvement culture in research in the US. For example, its work has been cited 30 times in evidence-based clinical recommendations, has been used as the basis for national coverage decisions by Medicare and Medicaid, and has influenced accreditation standards of rehabilitation services.

⁰⁰⁰⁰ <https://www.pcori.org/about-us/governance/committees>



11.4 SPOR

SPOR stands for Strategy for Patient-Oriented Research and is developed by the Canadian Institutes of Health Research (CIHR) to move patient involvement in research forward. The rationale for CIHR's work on the strategy was defined as "patients are active partners in health research that will lead to improved health outcomes and an enhanced health care system". SPOR aims to build capacity for various types of research that engage patients as partners, to focus research on patient identified priorities, and ultimately improve patient outcomes.

Because the web-site of the CIHR was no longer accessible (attempt to access 8 August 2019), the description of SPOR is based on the information retrieved from the former website of the Nova Scotia Health Research Foundation.²²⁹

SPOR is a collaboration of researchers, provinces and territories, healthcare providers, patients and families working in partnership to integrate research in patient care. It is publicly funded, with financing from the former Nova Scotia Health Research Foundation (changed on April 1st, 2019 to *Research Nova Scotia*). The public funds have been used to partially support the establishment provincial and territorial Support for People and Patient-Oriented Research and Trials (SUPPORT) Units.²³⁰ These units receive additional funds from ministries of health, provincial health research organizations, and in some cases, health authorities, academic institutions and industry. The SUPPORT units are multidisciplinary research service centres. They provide expertise to people engaged in different types of patient-oriented research. For example, a Patient Voices Network was established in Western Canada, originally designed to match patients with health care providers running quality improvement initiatives, but afterwards expanding its activities to the recruitment of patients for involvement in research.¹⁶¹

SPOR Networks are collaborations of patients, health service providers, policy/decision-makers, and health researchers across Canada. They are

organized around specific themes and conduct research that addresses the needs of patients. For instance, there is a network for "Transformational Research in Adolescent Mental Health", a network for "Primary and Integrated Health Care Innovations"

11.5 EUPATI

EUPATI is the European Patient's Academy, focused on training patients in order to give them the capabilities and tools to be meaningfully involved in research as 'patient experts'. It is a public-private partnership between pharmaceutical industry, academia, not-for-profit organisations and patient associations.^{pppp} EUPATI has several national branches, fostering and promoting patient education for involvement in research in the different European member states. On the web-site of EUPATI several training opportunities for patients, organised by EUPATI, are listed, as well as several guidance documents for patient involvement (in regulatory processes, HTA, industry-led medicines R&D, and ethical review of clinical trials). Besides giving training to patients and producing guidance for patient involvement in research, EUPATI also offers and maintains the toolbox on medicine development, a glossary and coordinates the network of national platforms for patient advocates.

11.6 Other initiatives: PFMD, Participatiekompas, PARADIGM

Patient involvement has attracted a lot of attention of the medical product industry as well. **Patient-Focused Medicines Development (PFMD^{qqqq})** is a forum where patients and industry work together, by involving also other stakeholders, to define good practice for patient involvement along the medicine product development lifecycle. PFMD aims at the "co-creation and implementation of a globally standardised meta-framework for patient engagement to make patient engagement more consistent, effective and meaningful". The process for the co-creation of this meta-framework

^{pppp} <https://www.eupati.eu>

^{qqqq} patientfocusedmedicine.org



consists of four steps: (1) mapping of the global patient involvement landscape and understanding stakeholder expectations, (2) convening multi-stakeholder working groups to explore current practices and define criteria for patient involvement, (3) integrate good practices into a meta-framework and (4) develop a toolkit for the practical use of the meta-framework. PFMD has published a book of good practices in June 2018, collecting a number of patient involvement initiatives that met the criteria of their 'patient engagement quality guidance'. The patient engagement quality guidance document is a practical guide to planning, developing and assessing the quality of patient engagement activities and projects throughout the development and lifecycle of medicines.

"Participatiekompas.nl" is a Dutch platform for researchers, patients and policy makers, gathering knowledge and experiences with patient involvement in research, healthcare policy and healthcare.^{rrrr} Several tools, methods and publications are provided to patients who want to be involved or researchers who want to involve patients in their research. All material is easily accessible to Dutch-speaking people. Participatiekompas was launched on January 1st, 2014 and is financed by ZonMW and the VSB Fund and is supported by PGOsupport.

PARADIGM^{ssss} is an ongoing large international project within the Innovative Medicines Initiative. It is a public-private partnership with 34 partners from industry, academia, public organisations, small and medium-sized enterprises and patient organisations. The objective of the project is to develop practical guidance and tools for involving patients in research along the medical product lifecycle. The project will also produce a set of metrics to measure the impact of patient involvement in research and development. The focus is on three research phases: priority setting in research, design of clinical trials and early dialogues. The results of PARADIGM will be available by the end of 2020.

12 PATIENT INVOLVEMENT EXPERIENCES IN BELGIUM

This part of the project has three main goals:

- To identify Belgian research projects in which patients are involved as partners
- To identify how patients are involved in these research projects
- To draw lessons for the development of research projects involving patients

The objective is to have an overview of the existing initiatives regarding patient involvement in research projects in Belgium, with attention paid to lessons learned and best practices that may be of interest for the development of the position paper and the process book of the KCE.

^{rrrr} <https://participatiekompas.nl>, accessed 6 August 2019. Participatiekompas.nl was established with support of ZonMW (www.zonmw.nl) and the VSB Fund (www.vsbfonds.nl/), and facilitated by PGOsupport (www.pgosupport.nl/).

^{ssss} <https://imi-paradigm.eu/>



12.1 Overview of the process and general timelines

The overall design of this research is a qualitative approach, based on semi-directive interviews with actors directly involved in the topic.

Table 15 – Overview of the process of the Belgian Initiatives survey

Major steps of the process	Timeline
<ul style="list-style-type: none">• Identification of informants• Development of interview guide	June 2018
<ul style="list-style-type: none">• E-mail contacts	July 2018 – October 2018
<ul style="list-style-type: none">• First round of interviews• Start of the analysis	November 2018 – March 2019
<ul style="list-style-type: none">• Contact with additional participants cited by the interviewees	January 2019-May 2019
<ul style="list-style-type: none">• Second round of interviews	April 2019-August 2019
<ul style="list-style-type: none">• Final reporting	August 2019

12.2 Selection and recruitment of the participants

Four groups were targeted: researchers, funders, (representatives of) patients and sickness funds. We opted for a purposive sampling in order to meet the participants who are the most likely to provide relevant information. By sampling 4 different groups, we also aimed at ensuring the diversity of perspectives regarding patient involvement, according to the 4 key stakeholder groups.

12.2.1 Researchers

We compiled a database with all research centres in Belgium likely to engage in patient involvement in research: the final database included 12 Belgian universities, 21 high schools with curricula related to the field of health and social sciences, 10 public research centres (community, regional and federal), and 5 non-academic research centres or initiatives.

All research centres were contacted by e-mail during the summer of 2018. Heads of public health / medicine / nursing / paramedical departments or research centres / study services of sickness funds were targeted as well as research administrations to increase the diffusion of the information.

This first e-mail contact aimed at identifying research centres with experiences and expertise in the field of patient involvement in research. To guide the recruitment, a common question list was sent in French or in Dutch to the potential participants (see Table 16). Participants were invited to share any published resource.

Among the research centres, 36 never replied, 5 declined participation as they are not currently doing such projects and 9 provided details about their current research projects.

Two research centres were excluded from the interviews: the first research centre only published a paper on patient involvement while the second only plans to involve patients. The 7 remaining research centres reporting involving patients in research were contacted for a semi-structured interview during a face-to-face or a Zoom meeting with one or two KCE researchers. Five projects were cited in the interviews.

The principal investigators of these projects were then contacted for an in-depth interview. Face-to-face interviews took place at the KCE or at the working place of the participants.

**Table 16 – Questions sent by e-mail to research centres****Questions for research centres**

- 1. Do you involve patients in your research projects?**
 - a. If yes, could you explain the project(s)?
 - b. If yes, how are they involved?
 - c. Is there any condition regarding their participation? (e.g. training, incentives, ...)
 - d. What are the lessons learned by involving patients in the research?
 - e. Do you have any specific institution / project to recommend as a best practice?
- 2. If you don't involve patients in the research projects, why?**
 - a. What would encourage you to do it?
 - b. What would prevent you from doing it?

12.2.2 Funding agencies

The database with the major public funders of research at regional and national level included 8 regional and community agencies, 3 federal agencies, and 2 private funds.

All funding agencies were contacted by e-mail during the summer of 2018. This first e-mail contact aimed at identifying incentives for the involvement of patients in the research. To this aim, a common question list was sent in French or in Dutch to the potential participants (see Table 17). Participants were invited to share any published resource or supportive document. Five funders replied.

Only one of the funders was contacted for an in-depth interview: two of the funders declared not having specific funding or incentives for patient involvement in research, two others have a specific funding but only related to a specific initiative (see section 12.8).

Table 17 – Questions sent by e-mail to funding centres**Questions for funding centres**

- 1. Do you require that patients are involved in the research projects as part of the conditions for funding?**
 - a. If yes, why?
 - b. Is there any condition regarding their participation? (e.g. training, incentives, ...)
 - c. What are the lessons learned by involving patients in the research?
 - d. Do you have any specific institution / project to recommend as a best practice?
 - e. Are there projects / themes for which you won't require patient participation?
- 2. If you don't require that patients are involved in the research projects, why?**
 - a. What would encourage you to do it?
 - b. What would prevent you from doing it?



12.2.3 Patient representatives

The three national umbrella organisations of patient associations were directly contacted for an interview with the research team: the *Ligue des Usagers des Services de Santé* (LUSS) for the French-speaking community, the *Vlaamse Patiëntenplatform* (VPP) for the Dutch-speaking community and the *Patienten Rat & Treff* for the German-speaking community.

Two additional patient representatives involved in research were recruited upon suggestion of interviewees from research centres and one patient representative was contacted through the snowballing approach.

12.2.4 Sickness funds

Sickness funds were considered as having an intermediary position between researchers, funders and patients: indeed, the 3 pre-cited positions could be found in each sickness funds. They represent the perspectives of patients as healthcare consumers. They also have their own study service, aiming – amongst others- at studying the needs and expectations of their members. They could also rely on external research teams and offer *ad hoc* funding for external research projects.

The directors of the 5 national health insurances were directly contacted by e-mail and all replied positively. The research team met the persons in charge of the study service. The CAAMI-HZIV was also contacted but declined participation.

12.3 Data collection

Data were collected through semi-directive interviews. Interviews were supported by two interview guides, developed by the KCE researchers based on the key points of patient involvement in research (see Appendix 3).

The two interview guides had a common question list but were adapted to the profile of the participants. Similarly, the question order was indicative and adapted to the participant discourse.

Interviews were held between November 2018 and July 2019, in French, Dutch or English. Interviews were held in face-to-face at the participant's location, at the KCE or through Zoom. All interviews were audio-recorded. No financial incentive was offered to the participants.

12.4 Data analysis

Each initiative was first briefly described regarding the context, the objectives, the profiles and roles of patients as well as its funding.

Data were then grouped according to the following pre-defined categories: 1) lessons learnt: consensus; 2) diverging issues; 3) enablers to patient involvement; 4) barriers to patient involvement and 5) lessons for the future.

12.5 Experiences of the Belgian research centres

12.5.1 Description of the sample

Nine interviews were performed with research centres, two with hospital teams, one with a private research initiative and one with a support organisation in health care quality and patient safety (the *Plateforme pour l'Amélioration de la Qualité et de la Sécurité des Soins*. (PAQS)).

After the interview, the private research initiative was excluded as not fulfilling the inclusion criteria.

Although not a research centre, the *Plateforme pour l'Amélioration de la Qualité et de la Sécurité des Soins* was included because it has developed over the years a specific expertise regarding patient involvement in healthcare and provides support to a wide range of healthcare services. It was cited as a resource by three interviewees.

Five interviews were performed with the sickness funds.

The three umbrella organisations of patient associations accepted the interviews. Three patient representatives were also interviewed: one from EUPATI Belgium, one in charge of the patient-partner model at the ULB and one belonging to the association who contributed to the development of the survey on the living conditions of persons with HIV.



Three funding agencies replied by e-mail and shared their terms of reference for their calls, 1 was encountered during a face-to-face meeting and 1 was interviewed by phone as it was more convenient for the participant.

12.5.2 *Institute of Tropical Medicine: Community-based participatory research project*

12.5.2.1 *Context & setting*

The TOGETHER project was developed by the research group HIV and Sexual Health (department of Public Health) in response to the lack of data regarding the prevalence of HIV, the amplitude of undiagnosed HIV infections and influencing factors among sub-Saharan African migrants (SAM) living in Flandres. The study was conducted in Antwerp in 2013-2014 within the community of sub-Saharan Africans.^{231, 232}

12.5.2.2 *Objectives*

Using a **mixed method** and a **community-based participatory approach**, TOGETHER aimed at increasing “*the communities’, researchers’, and policymakers’ in-depth understanding of the dynamics of the HIV epidemic among SAM, to improve primary prevention interventions*”.^{231, 232}

12.5.2.3 *Profile of patients involved*

All lay community-researchers as well as the study participants self-identified as sub-Saharan migrants. Also patients from this group were involved, i.e. “persons living with HIV”. Efforts were made to reflect the diversity of the community while recruiting: various profiles in terms gender, age, origin, duration of residence, education level and employment status were included.

12.5.2.4 *Role of patients*

After a first exploratory phase with stakeholders of 48 sub-Saharan African countries, a Community Advisory Board, constituted of members of community-based organizations active in HIV prevention and sexual health promotion, was established.

Nine **lay community researchers** were hired: they were specifically trained to conduct diverse tasks related to the entire research process, i.e. deciding on the study design, collecting data, interpreting the results and ethics. The lay researchers played a major role in preparing the data collection, engaging in contact with the participants and developing intervention messages for the disseminating the final results. Based on the results of the study, the lay researchers suggested actions for the daily prevention practice likely to be effective based on the study’s insights. . The researchers invested substantially in ongoing supervision to ensure data quality.

The lay researchers had a volunteer contract with ITM: they were employed for a limited amount of hours (as the payment ceiling is around 1300 EUR/year). As an additional consequence, only SAM with documented status could be hired, since proof of their official migration status was need for the administrative procedures.

12.5.2.5 *Funding*

The project TOGETHER was funded through a research grant of the Fund for Scientific Research on AIDS (King Baudouin Foundation).

12.5.3 *ULiège: patients involved in different roles*

12.5.3.1 *Context*

Since several years, the department of Public Health of the *Université de Liège* has a global philosophy of patient involvement. This philosophy could be found in various projects on patient involvement / integrated healthcare, currently ongoing.



12.5.3.2 Objectives

The overarching objectives are to better involve patients in healthcare and research and to assess how patient involvement is defined and experienced in practice. For example, one may question the impact of patient involvement on social health inequalities or patient involvement may have side-effects on some patients or on the system. By investigating patient involvement, the research teams aim at producing evidence on patient involvement. This is illustrated by the following projects:

- Project Interreg “Patient Partenaire de soins”: to understand the role of the patient in healthcare (governance, education and research), with two sub-objectives: 1) to understand what participation means for patients; 2) to identify, collect information, assess and support various initiatives aiming at better involving patient in the Walloon health care system.
- Be Hive - French research chair on first line care: development of a specific axis on patient involvement (under discussion at the time of the interview)^{tttt}
- Collaboration with PAQS: development of community of practices with respect to patient committees
- Principal agreement with LUSS: establishment of a community of exchanges on a regular basis to examine the place and the position of patients in research

12.5.3.3 Profiles of patient involved

Profiles of patients vary according to projects but they mostly have a high degree of health literacy, a high socioeconomic level and good communication skills. Researchers however are thinking about how better involve patients with diverse profiles and experiences. Depending on the

projects, patients involved could be members of patient associations. Some studies recruit patients to give input based on their expertise, others recruit patients as research partners.

12.5.3.4 Role of patients involved

Patients are mostly **key informants**, although future projects could involve patients as co-researchers (under development).

12.5.3.5 Funding

The project Interreg is funded by the European Funds for regional development. *Be Hive* is funded by the King Baudouin Foundation thanks to the donation of D. De Coninck.

12.5.4 UCLouvain & Haute Ecole Léonard de Vinci: Participate Brussels

12.5.4.1 Context

‘Participate Brussels’^{uuuu} is a collaborative research project led by the Institute of Health and Society (UCLouvain) and the Institute Parnasse-ISEI (*Haute Ecole Léonard de Vinci*). The researchers are supported by a steering committee gathering health care institutions, patient associations and other services, active in the Brussels region. Chronic diseases require complex health care in the community, with a preponderant place devoted to self-care. For self-care activities including health promotion activities, to be successfully initiated and maintained by patients, personal preferences and health goals in relation to medical and non-medical determinants of health, need to be discussed. These are, however, often poorly considered in the development of health care plans for chronic patients.

^{tttt} See more here: <https://www.lespecialiste.be/fr/actualites/socio-professionnel/be-hive-la-nouvelle-chaire-interuniversitaire-en-soins-de-1ere-ligne.html>

^{uuuu} Project website: <http://www.participate.brussels>



12.5.4.2 Objectives

Overall, this project aims at improving co-creating ways to improve the dialogue between patients and professionals, as part of the process of implementing personalised health care plans for patients living with a chronic disease in the Brussels region.

12.5.4.3 Profiles of patient involved

Patients involved as **co-researchers** are members of patient associations (such as the *LUSS*). In a later phase of the project (forthcoming), additional profiles of patients are likely to be involved. Patients (and family caregivers) that are consulted about their lived experiences are any patients with a somatic chronic condition living in the Brussels region.

12.5.4.4 Role of patients involved

Patients are represented in the project's **steering committee**, which also includes other partners such as representatives of the first and second line health professionals, sickness funds, and non-profit associations such as *PAQS* or *Cultures & Santé*. According to their availability and interest, the members of the steering committee:

- have been involved in the **drafting** of the research project prior to submission,
- have participated in the formal presentation of the project in front of the selection panel, and other formal presentations following the initial presentation
- participate in the meetings of the research team, and may advise on the orientation of the project and discuss the results
- were involved in the definition of the **recruitment strategy**, and participate in the recruitment process by identifying potential participants or communicating about the project

In a second step of the project, the research team plans to involve patients, health care professionals, and other stakeholders in a **dialogue to co-**

decide on a feasible and relevant dimension of a method to facilitate the development and implementation of personalised health care plans in the Brussels region.

12.5.4.5 Funding

'Participate Brussels' is funded for 3 years through the program BRIDGE 2017 Health & Well-being of the Brussels Institute for Research and Innovation (INNOVIRIS) (see also section 12.8.1.).

12.5.5 *Observatoire du sida et des sexualités : patients as initiators, co-researchers and research leaders*

12.5.5.1 Context

The *Observatoire du SIDA et des sexualités* is a research centre in human and social sciences, with a lens of sexual health promotion. Two research projects performed by the centre directly involved patients: 1) description of living conditions of people with HIV/AIDS in Brussels and Wallonia; 2) identification of the needs of people with HIV/AIDS aged 50 years and more (ongoing).

12.5.5.2 Objectives

The overall objective of the Observatory is to improve the coherence between the needs of the target groups, the daily practices of the health and social care professionals and the health policies. The first project (2007-2012) aimed at describing the living conditions of persons living with the HIV/AIDS, with a particular focus on patients with few or no contacts with support groups. The second project, still ongoing, aims at understanding the specific needs of elderly patients living with HIV/AIDS.



12.5.5.3 Profiles of patient involved

Patients were all members of a patient group and all had a diagnosis of HIV/AIDS when they solicited the *Observatoire* to conduct the research. In the first project, patient profiles were highly heterogeneous and reflected the variety of situations experienced by the rest of the patients.

12.5.5.4 Role of patients

Patients were the **initiators** of the research in both projects. In the first project, patients knew each other via the support group Action+/Grécos of the *Plateforme Prévention SIDA*: they directly solicited the Observatory based on their individual experiences. Patients were then involved at all stages of the research, with the exception of the data collection process. They identified the objectives, chose the methodology, participated to the pre-test of the research instruments, discussed the results with the researchers (the researchers pre-digested the data) and edited their own recommendations at the end of the project (in addition to the scientific report, which fell under the full responsibility of the researchers).²³³

In the second project, the partnership mostly concerns the methodological aspect of the project: Utopia, the patient association, will conduct the research on its own (i.e. patient-led research). Researchers help to design the instrument for data collection. The project is still ongoing.

12.5.5.5 Funding

The Observatory has a mandate to support these kinds of projects and benefits from an institutional funding to achieve this mission.

12.5.6 Institut Jules Bordet: patients involved in the design of clinical trials

12.5.6.1 Context

The Jules Bordet Institute is specialised in caring for patients suffering from cancer. Patient involvement is part of the accreditation process of the hospital: internally, the project is materialised through the patient partnership approach.^{www} The Institute is also member of the Organisation of the European Cancer Institutes (OEI) which strongly promotes patient involvement. In clinical practice, patients are involved in two projects: 1) improving communication (like making leaflets more patient-friendly); 2) supporting the redesign of services from a patient perspective.

Regarding research activities, patients were involved in the development of a tool to support the informed consent process (videos) and a group of patient's advisors (PISARO) is currently place to review research protocols and patient documents.

12.5.6.2 Objectives

By including patients in the research protocol review process, the Institute aims at adding relevance to the research protocols, especially regarding the research questions.

12.5.6.3 Profiles of patients included

All patients included are members of patient organisations and are already volunteers at the Institute. They need to be able to act as a bridge between organisations and professionals. Patients mostly have a high socioeconomic profile and a good health literacy.

^{www} See more here: <https://bx1.be/news/linstitut-bordet-veut-ameliorer-la-recherche-en-oncologie-avec-laide-des-patients/>



12.5.6.4 *Role of patients*

Two levels of patient involvement are distinguished:

- Consultation (i.e. patient as advisor): patients are invited to give feedback on a specific aspect or to join a project temporarily
- Collaboration (i.e. patient as partner): patients have a liaison function between patient organisations and health care/research professionals

In the PISARO project, the opinion of patients is consultative.

12.5.6.5 *Funding*

The project is supported by the institutional budget.

12.5.7 *Groupe Jolimont: patient partner*

12.5.7.1 *Context*

The group Jolimont gathers the current hospitals of Jolimont, Nivelles, Tubize, Mons, Warquignies and Lobbes. In the future, they aim at concentrating their activities on 4 distinct sites: Mons Borinage, Bassin du Centre-La Louvière, Thudinie-Charleroi Sud & Brabant Wallon Ouest-Nivelles.

Since 2015, the group Jolimont has developed an institutional culture of patient involvement at micro (clinical) and meso (organisational) level. Currently, 8 patient partners, among which 4 with a high degree of involvement, are active in the group Jolimont, in various projects: patient partner committee, committee for patient identification (in a patient safety context), accreditation and therapeutic education.

12.5.7.2 *Objectives*

The patient partner committee aims at supporting the implementation of participatory management by including the perspectives of all actors on generic transversal themes, relevant for the entire institution.

The committee for the identification of the patients is a technical and thematic group: it aims at developing a better identification system of the patients during their stay at the hospital to prevent medical errors or patient safety issues.

Involvement of patients in the accreditation process was reported as an emerging activity: when initiating the accreditation process, the institution did not foresee the involvement of patients. When examining the quality criteria, the institution became aware of the need for a better involvement of patients and decided to better involve patients and at the earliest stages possible. Patients are, for example, associated from the start in the development of information support tools for new patients coming to the hospital. For example, patients help professionals to develop appropriate guidelines for informed consent.

Two patient partners are also involved in the co-construction of a project on therapeutic education for chronic obstructive pulmonary diseases (COPD).

12.5.7.3 *Profiles of patients involved*

Patients involved have diverse profiles but are all able to take distance from their personal situation. Patients involved are not “independents” but rather perceived as “actors” in their care.

12.5.7.4 *Roles of patients involved*

Patients are mobilised as partners: they are invited to provide input at various stages of the projects and in their deliverables.

12.5.7.5 *Funding*

All expenses are covered by the hospital budget.



12.5.8 *UGent: longstanding expertise*

12.5.8.1 *Context*

The '*Universitair Centrum voor Verpleegkunde en Vroedkunde*' of the Ghent University focuses on patient involvement at micro and meso level (care processes, organization in the hospital, hospital departments). Patient involvement activities emerged from a focus on qualitative research approaches. The examination of possible 'best practices' for patient involvement in research is part of the (academic) interests of this group.

12.5.8.2 *Objectives*

All research projects involving patients aim at complementing academic expertise with the specific strengths and expertise of patients.

12.5.8.3 *Profiles of patient involved*

Patients are involved on a personal basis and need to volunteer to be involved in a project. There is no restriction regarding membership of a patient association.

12.5.8.4 *Role of the patients involved*

Involvement of patients takes different forms:

- Action research: brings together patients and professionals to work on a concrete project. Researchers examine the processes (of change) and dynamics between patients and professionals.
- Co-creation of research with patients: involvement from the very beginning of ideation up to dissemination. Co-creation involves fundamental discussions at each step of the research process. Patients are not asked to reflect from a research perspective but from their own personal perspective. Researchers make, based on these extensive reflections, proposals for research.

Patients taking responsibility for certain aspects of the research project: an experiment was performed where patients collected data in other patients by means of questionnaires or interviews, to examine the added value or effect of patients' taking care of data collection. Patients did not receive training beforehand but were nevertheless screened for their motivation. The conclusion of the experiment was that there are some issues related to giving patients the responsibility for data collection, both in terms of the scientific validity of the data and in terms of impact on the patient collecting the data. In another project, patients were involved in data analysis, both quantitative and qualitative analyses. This was considered to be a huge enrichment to the project. Patients gave different insights, ideas, and determined or influenced the direction of the analyses.

12.5.8.5 *Funding*

The initiatives of the '*Universitair Centrum voor Verpleegkunde en Vroedkunde*' are funded from various academic sources.

12.5.9 *Plateforme pour l'Amélioration de la Qualité et de Sécurité des Soins (PAQS)*

12.5.9.1 *Context*

The Platform for Continuous Improvement of Quality of Care and Patient Safety (Plateforme pour l'Amélioration continue de la Qualité des soins et de la Sécurité des patients – PAQS ASBL) aims to promote, support and organise the development and implementation of initiatives of continuous quality of care and patient safety improvement in Brussels and Walloon healthcare institutions. Providing tools and methodological support for implementing such projects, PAQS is currently involved in several projects in which patients are involved: 1) implementation of PROMs & PREMs; 2) recruiting and training patients; 3) patient involvement in improving quality of care and patient safety, and 4) involvement of nursing home residents in the admission process of newcomers. Although not a research centre *per se*, PAQS has developed a specific expertise regarding patient involvement.



12.5.9.2 Objectives

The objective of all these projects is the improvement of quality of care and patient safety across the health care system in Brussels and Walloon healthcare institutions.

12.5.9.3 Profiles of patients involved

The profiles depend on the project but there is a preference for patients with a chronic disease as they have an in-depth experience of the health care system. Involving acute patients, young adults, patients with mental health problems or living in precarious conditions is more complex but still achievable with the right support.

12.5.9.4 Role of patients

The roles are project dependent but patients should be considered as **equal partners** and be involved **in all stages** of the improvement project. While some projects are initiated based on patients' feedback, it's the institution that thinks of an improvement, based on context and available resources but patients should be associated at a very early stage of the process.

12.5.9.5 Funding

PAQS is funded by the sickness funds, the Brussels-Capital Region, the Walloon Region and the Federation Wallonia-Brussels and by the associations of health care institutions: santhea, UNESSA, GIBBIS and Wallcura.

12.5.10 KU Leuven: first steps of patient involvement

12.5.10.1 Context and setting

The Leuven-Basel Adherence Research Group (LBARG) of the Academic Centre for Nursing and Midwifery at KU Leuven increasingly involves patients in its academic projects, based on the principle that patient involvement is key for improving patient health and well-being. The

researchers aim to focus on research that addresses the needs and priorities of patients. Therefore, it considers patient involvement in research as crucial.

12.5.10.2 Objectives

Three projects were reported:

1. Collaboration with UK regarding the use of Facebook groups to identify needs of patients after allogeneic stem cell transplantation.
2. Picasso Tx project "Is there a preference for interactive health technology as self-management support for solid organ Tx patients?", as part of a completed PhD project on the development of e-health applications to support patients after solid organ transplantation.
3. IPF project, currently ongoing, also part of a PhD project that aims at improving the quality of life of patients with idiopathic pulmonary fibrosis (IPF), a rare pulmonary diseases. More specifically, it aims to "develop, implement and test a new care model for patients with IPF, by using implementation science methodology, by actively involving patients and other relevant stakeholders in all phases of our planned research, and by adopting a patient-centred approach in which improving patient-reported outcomes is of paramount importance".

12.5.10.3 Profiles of patient involved

Various profiles of patients are involved, depending of the objectives of the patient involvement. Individual patients are preferred if the objective is to share experiences or identify needs while patient organisations or representatives are preferred for brainstorming sessions with health care professionals.



12.5.10.4 Role of patients

In the Picasso project, the first phase investigated patients' needs and preferences regarding e-health support. In a second phase, researchers went to patients' homes to see how patients cope with their condition in daily life: patients shared their tips and tricks, and needs. Patients helped to develop and test various prototypes (design, content, etc.).

In the IPF project, patients will be involved during the whole process, with inputs varying according to the research steps. In the early stage, patients and their caregivers contribute to the identification of gaps in their current care: this will help to determine the focus of the novel care model and to guide the further development of the research programme. In a later stage, patients and professionals will gather in brainstorming sessions to shape the content, process and structure of the new care model.

12.5.10.5 Funding

The Picasso-Tx project was funded by the special research funds of the KU Leuven^{www}.

12.5.11 LiCalab, Care Living Lab for innovation in health and care

12.5.11.1 Context and setting

Launched 6 years ago, LiCalab has developed a specific expertise in technological innovations, exercise and revalidation, mental health and informal care^{xxxx}. LiCalab is involved in several European projects (eg Interreg, collaboration with other living labs) and regional projects. Also private companies - SMEs and corporates - work together with LiCalab for innovation projects that strongly benefit from end user involvement. LiCalab conducts co-creation sessions and prototype studies; e.g. where patients, elderly or care professionals are invited to help define the design of medical

devices, intelligent package for medications (human factor study), new processes...

12.5.11.2 Objectives

LiCaLab aims at supporting "businesses and organisations in the health and care sector by testing and validating innovations with end users, in their own living environment". They aim at making sure that the innovation meets the needs of patients. End users help to identify desired and useful functionalities in medical devices, tools to support revalidation or applications that allow elderly to live longer in their own home. Numerous projects are ongoing, but all aim at gathering the human experience in the development of resources.

12.5.11.3 Profiles of patient involved

LiCaLab relies on a network of 1000 patients – including elderly - and around 200 health care professionals (pharmacists, nurses, general practitioners...) that have expressed their interest to participate in various projects. This extensive database offers easy access to respond to the specific needs of each project. LiCalab works with individual patients, but also works with patient associations that act as intermediary between researchers and patients.

12.5.11.4 Role of patients

Patients are involved in different ways. In the research design phase, patients are invited to share their insights through quantitative and qualitative questionnaires. They also participate in co-creation workshops to design projects from the start, and are involved in real life tests to experience the new tools in their own living or working environment. Most of the topics are defined by external parties. Besides this, LiCalab also organises a yearly

^{www} See here for the detailed project: <https://soc.kuleuven.be/mintlab/blog/project/picasso-tx/>

^{xxxx} See the website of the LiCaLab for the current and past projects: <https://www.licalab.be/en/projects>



'panel day' during which suggestions can be put forward (brainstorming on whether the proposal could be an added value).

12.5.11.5 Funding

Between 2013 and 2016, LiCaLab received funding from the Flemish government for 3 years and was supported by Stad Turnhout, Welzijnzorg Kempen and Thomas More University College. Since 2016, LiCalab works in regional and European programmes and delivers services to private companies. LiCalab is part of Thomas More University College.

12.5.12 ULB: patient as partner

12.5.12.1 Context and setting

Since 2.5 years, the Ecole de santé publique de l'ULB has developed the model of the patient-partner in various aspects of research and clinics. Inspired by the principles of the Montreal model, developed by the Université de Montréal, the patient-partner model of the ULB is fundamentally based on the importance of the experiential knowledge of patients and equity of all actors involved in health care, whatever the dimensions of health care.²³⁴ Contrary to the Montreal Model, the model developed by ULB is focused on the micro and meso context, arguing that the experiential knowledge is always context-specific. The model is rooted in qualitative methods, inspired by the socio-constructivism trend.²³⁵

12.5.12.2 Objectives

The patient-partner model is proposed as a transversal dimension of existing projects, aiming at maintaining a high level of patient involvement at all stages of the process. This model offers a complementary approach to capture patient experiences and knowledge.

12.5.12.3 Profiles of patient involved

Patients involved should be patients in the service / institution initiating the research questions (and should be connected to the health care system). They also have to be "partners of their own health care", meaning that they "*share their life experiences with the disease, their goals and priorities with the professionals to orient the treatment in function of their vision of their future life ('life project'). In addition they have to take themselves decisions that concern their care*" (translated from Lecoq & Néron 2018)²³⁶.

Additionally, patients should be resilient and be able to take distance from the situation, with a critical lens, while being able to share their experience with health care, the disease and living with disease. Finally, they have to be ready to be involved in a dynamic of health care system improvement.²³⁶

12.5.12.4 Role of patients

Patient partners are involved at the early stage of the projects and give inputs during the whole process, similarly to the other actors involved. This is currently tested in the accreditation process of the CHU Erasme, the educational curricula of future health care professionals and in research.

12.5.12.5 Funding

The current projects are funded by the budget of the institutions and by the cell Quality and Safety of the Federal Public Service Public Health.



12.6 Patient representatives and patients involved in research

12.6.1 *Ligue des Usagers des Services de Santé (LUSS)*

12.6.1.1 *Context and setting*

The Ligue des Usagers des Services de Santé represents the patients of the Fédération Wallonie-Bruxelles. The LUSS represents the patients in more than 30 different institutions, and 80 patients associations are members of the LUSS.

Currently, the LUSS is involved in various research projects, among which the representation of patients in 3 ethical committees and the presence in the Board of the KCE. In addition, they are involved in committees at the Belgian Drug Agency (FAGG/AFMPS).

12.6.1.2 *Objectives*

The LUSS is legally acknowledged as one of the three official umbrella organisations of patient associations in Belgium. By being involved in research projects, the LUSS aims at ensuring that the patient voice is heard and that his/her rights are respected. Adding the patient's perspective helps to take better into account the (unmet) needs and priorities of the patients.

12.6.1.3 *Profiles of patient involved*

Patients should be members of a patient association before they can be involved in a project, managed or supported by the LUSS. Patients should be able to share negative experiences and not only “success” stories and be able to move from an individual experience to a collective perspective. Patients should be regular and reliable in their participation, and need a minimal level of health literacy.²³⁷

12.6.1.4 *Role of patients*

Patients are always involved via a mandate and are supported by a LUSS professional. The role of the patients depends on the project but patients are usually required to act as patient advocates.

For example, in the pilot projects for the ethical committees, patients share a peer vision of the topic and help the committee members to stay connected with the priorities, the needs and the daily experience of patients. They are also the “guardians” of patient rights and ensure that information is provided in a clear and comprehensible language. They analyse and review the documents submitted to the ethical committee, similarly to the other members.

12.6.1.5 *Funding*

The LUSS is an independent non-profit association. The LUSS benefits from subsidies from the Federal government, the Walloon region, the Fédération Wallonie-Bruxelles, and the King Baudouin Foundation.

12.6.2 *Vlaams Patiëntenplatform (VPP)*

12.6.2.1 *Context and setting*

Flemish Patients Platform – Vlaams Patiëntenplatform vzw (VPP) is an independent platform of 120 patient organisations from Flanders, which strives for an accessible care system, tailored for the patient and his environment.

Patient organisations provide information to their members and to the larger constituency. A better understanding of the causes and the course and treatment options of a disease reduces uncertainty for patients. Patient organisations work in a preventive manner. They contribute to a fast(er) detection of disease. Members of patient organisations recognize the symptoms associated with a particular disease at an early stage, this makes them visit a physician sooner.



The Flemish Patients Platform seeks active participation of patients in health policy and health care. The VPP projects are born by experience experts in patient organisations. Patient rights, independent complaint right for patients, quality and accessibility of care, equal opportunities in employment and insurance, e-health,... are important themes in the operation.

The VPP is involved in numerous projects, among which the Patient Expert Centre, the development of guidelines at the CEBAM, the *Vlaams Instituut voor de Eerste Lijn* (VIVEL), the Project *patiëntenparticipatie* in de *Limburgse eerstelijnszones* and the pilot project of the national Drug Agency (FAGG/AFMPS), together with the LUSS.²³⁸

12.6.2.2 Objectives

As a Flemish independent platform, the VPP aims to act as an interlocutor in health policy as providers, policy makers and researchers. The Flemish Patients Platform aims to improve the quality of life of the patient and their environment through the realization of participation and representation in the organization and development of the policy on health in all its aspects and at all levels.

Similar to the LUSS, the VPP is an official spokesperson of patients in Belgium. The VPP plays a role in supporting the networking between patient associations and, by being involved in research projects, similar to the LUSS, it ensures that the patient perspectives are considered and included in the research. In that perspective, involving patients empower them and open a room for dialogue.

12.6.2.3 Profiles of patient involved

The VPP emphasizes the collective experience and involves preferably patients able to share their experiences beyond their individual situation.

When being asked to join a (research) project, if it is specific to a pathology, the preference will go towards the patient association concerned by the issue. If the request is more general, the VPP will take the lead, with the support of its members.

12.6.2.4 Role of patients

Ideally, patients should be involved in the various steps of the research projects but they are usually contacted when the research protocol is already written. Different roles could be endorsed by patients, depending on the projects.

12.6.2.5 Funding

The VPP is a non-profit association, receiving public subsidies from the Flemish and the Federal governments.

12.6.3 Patienten Rat und Treff (PRT)

12.6.3.1 Context and setting

The Patienten Rat und Treff (PRT) represents the rights and interests of the German-speaking patient community in Belgium. The focus is on health promotion and disease prevention by informing the citizens.

The PRT has official representation mandates in 5 public institutions. Due to the small size of the population (they represent less than 80 000 people) and the PRT (2 FTE), there is no membership of patient associations. The PRT organises, among other things, peer-support groups. The PRT is not yet involved in research projects but is involved in other projects such as training programs with high schools, collaboration with the Banques Alimentaires or the Interreg project of the Universiteit Maastricht.

12.6.3.2 Objectives

The PRT is the third official spokesperson of patients in Belgium. Alongside the patient information and support, the PRT also aims at ensuring the particularities of the German-speaking Belgian patients regarding access to health care.



12.6.3.3 Profiles of patient involved

As the PRT has no experience yet with patient involvement in research, there is no information about the profiles of patient likely to be involved. It seems that those willing to join this kind of process would need a certain degree of education and be available, such as retired patients.

12.6.3.4 Role of patients

In research projects, the PRT could act as an intermediary between patients and researchers, by helping in the recruitment process and by supporting patients involved in the project (i.e. debriefing and training).

12.6.3.5 Funding

The PRT is a non-profit association, benefiting from public subsidies of the Health Ministry of the German Community and of the Federal government.

12.6.4 EUPATI Belgium: patients as experts

12.6.4.1 Context and setting

EUPATI^{yyyy} was developed to fill the growing need for including the patient perspective in drug development. EUPATI stands for the European Patient Academy on Therapeutic Innovation and is currently implanted in 14 countries as national associations. EUPATI Belgium vzw/asbl is active in Belgium as one of the EUPATI National Platforms (ENP). Since 2018 EUPATI Belgium is further developing its services, focussing on providing training for patients to become patient experts in the field of drug development using the EUPATI Toolboxes (see [eupati.eu](https://www.eupati.eu)). The National EUPATI Platforms (ENPs) reflect the European multi-stakeholder initiative on a national level, always consisting out of patients and patient representatives, academic representatives and pharmaceutical industry representatives. The basic principles of EUPATI are based on three pillars:

transparency, communication and feedback. EUPATI believes that, in order to see patients as partners in healthcare, one cannot be a real partner in healthcare if he or she does not understand the processes and the complexity of the healthcare ecosystem about drug development and drug access. In other words, it is EUPATI's belief that patients should be equipped with adequate training to dialogue on an equal basis with other actors.

EUPATI is complementary to Patient Expert Centre: EUPATI provides education on drug development and patient participation in drug development, PEC helps patient organisations to set up disease specific courses together with generic content (or which EUPATI is part) and offers a system to contract patient experts to provide input to stakeholders who have a need for patients.

12.6.4.2 Objectives

By supporting a dialogue between patients, academics and pharmaceutical industries, EUPATI aims at promoting patient involvement in research and development of new drugs. It supports a better integration of the patient needs and perspectives throughout the drug development lifecycle. To achieve this aim, EUPATI provides specific training to patients on various modules, such as clinical trials, health technology assessment and ethical aspects to make them "patient experts". To support these activities, EUPATI has developed a common toolbox at European level.

12.6.4.3 Profile of patients involved

Patients involved in EUPATI are mostly, but not exclusively, members of patient associations, EUPATI is open to all patients, as long they have a strong interest to become a patient expert. Patients representatives involved in EUPATI mostly have a higher education, and their existing professional expertise is often leveraged to bring value to the EUPATI network. Some

^{yyyy} See more information here: <https://www.eupati.eu/>



patient representatives are on temporary or permanent work incapacity, others are full time employees or self-employed.

12.6.4.4 Role of patients

The Board of EUPATI is composed of patient representatives and academics, with equal voting rights, and representatives of the pharmaceutical industry, with partial voting right (denied in case of a conflict of interest). The chair should always be a patient representative.

In Belgium, several projects are currently ongoing:

- National survey on the status of patient participation in research in Belgium
- Continues educational sessions for patients using EUPATI Toolboxes, such as Clinical Trials, PROMS, Patient Registries, HTA,...
- Collaboration with other (patient) organisations to educate patient experts.

Depending on the project, patients could be involved at all stages of the research process.

12.6.4.5 Funding

EUPATI at international level is funded by the Innovative Medicines Initiative (IMI) and co-led by the European Patient Forum. In Belgium, funding of the yearly events come principally from the pharmaceutical industry in the form of grants.

12.6.5 ULB: patient-partner, the perspective of the patient

12.6.5.1 Context

On the request of the dean of the faculty of medicine, A. Néron, director of the first European Office of Patient Partnership, was invited to develop a patient partnership unit in Belgium.

12.6.5.2 Objectives

The patient partnership unit has two objectives: 1) to implement a methodology to recruit and involve patients as partners at a high level into research 2) to create a training certificate for health care professionals regarding patient partnership. The ultimate aim of the unit is to add “relevance to the debates”, and to better implement patient involvement in the initial and continuing education of health care professionals, as well as in projects aiming at improving health care quality and safety.

12.6.5.3 Profiles of patients involved

Patients are involved on an individual basis, should not be members of patient association or in an acute phase of their disease. Patients in the acute phase of their disease might have been diagnosed recently and not have accepted their condition yet; also alcoholics or illicit drug users who are not yet weaned would not be involved. Profiles of patients vary depending on the project. The inclusion criteria are determined and decided by the health care professionals. Health care professionals establish a list of potential participants, who are, in a second step, directly contacted by A. Néron. If the phone contact is positive, the potential participant is invited to a face-to-face interview with A. Néron. Patients included should not be easily influenced or be influencers to ensure a real partnership.

12.6.5.4 Role of patients

Patients bring in the expertise of “living with the disease” and not a scientific expertise. Patients discuss with the researchers at all stages of the process.

12.6.5.5 Funding

Institutions fund the projects and patient involvement from their own budgets.



12.6.6 Plateforme Prévention SIDA: patients as initiators of research

12.6.6.1 Context

The *Plateforme Prévention SIDA* offer different activities to persons living with the HIV, including patients group. Action+/ GRECOS is one of these groups: its members, all persons living with the HIV, decided to work on the identification of the needs of people with HIV/AIDS (see paragraph 12.5.5). Action+/ GRECOS decided to ask the *Observatoire du sida et des sexualités* to develop a survey to explore the needs of persons living with the HIV/AIDS^{zzzz}.

12.6.6.2 Objectives

The project aimed at gathering the needs, experiences and perspectives of patients living with the HIV/AIDS in the Brussels and Walloon regions. Being involved as patient made sense for them and was a way to confirm their subjective and individual perceptions.

12.6.6.3 Profiles of patients involved

Patients were involved at personal title and were all members of the Action+.

12.6.6.4 Role of the patients involved

Patients were involved at all stages of the process as “experts in HIV/AIDS”. They did not participate to the data collection as they were likely to have personal connections with the respondents or may be emotionally affected by the discourse of the patients.

12.6.6.5 Funding

The project was financially supported by the *Plateforme Prévention SIDA* and the *Observatoire du sida et des sexualités*.

12.7 Sickness funds

As representatives of the healthcare users, the 5 sickness funds are particularly concerned by the public and patient involvement in research. All agree on the need to (better) involve patients and wish having more methodological support to do so.

Each study service develops its research agenda, based on the priorities decided by the sickness funds itself. Topics cover a wide range of subjects but are all related to the domains of expertise of the sickness funds. The final results could help the sickness funds to improve its own activities or to inform the public authorities about issues of relevance for the healthcare users. It could also lead to the reimbursement of new treatments or additional services to fulfil a need expressed by the patients.

Usually, the studies produced by the sickness funds rely on their own database and the experiences of their members. Some sickness funds have an annual patient survey that may involve patient satisfaction, patient (unmet) needs and patient priorities. In some cases, members are invited to join focus groups to investigate specific issues or to discuss broader results. One sickness funds relies on their partner volunteer organisations: these volunteer organisations help to bring researchers and patients together. Patients were also recruited to help collecting data, e.g. during face-to-face interviews.

No formal impact assessment of patient involvement on the research has been conducted so far. (In)formal feedback reveals that patients appreciate being involved, that involvement gives them a sense of pride and

^{zzzz} See more here: https://preventionsida.org/vivre_avec_vih/action-plus/enquete/plus-sur-le-projetde-lenquete/



usefulness. Patients involved as researchers also report having discovered social realities they were not aware of.

12.8 Funding agencies

12.8.1 Innoviris –Brussels region

12.8.1.1 Missions

The *Brussels Instituut voor Onderzoek en Innovatie /Institut Bruxellois de Recherche et d'Innovation* (INNOVIRIS) is the regional institute for research and innovation. It aims at connecting, stimulating and providing financial support to citizens, research centres, companies and non-profit associations.

12.8.1.2 Rationale & forms of patient involvement

To date, public involvement was required in several funding programs: led by or involving Innoviris: BRIDGE (now named Strategic Platforms), Co-Create (now named Co-creation), Experimental Platforms and the EU program AAL (Active Assisted Living). The call for research BRIDGE 2017 Health and Well-being explicitly required the participation of patients. In the AAL program, the involvement of end users is mandatory from the moment of the project preparation to the exploitation of results. The Co-Create and the Experimental Platforms programs explicitly targets the participation of all actors concerned by the research topic in the research/experimental process. These programs are not specific to health and health care but a submission concerning health will therefore require patient involvement as a prerequisite for the funding. In these calls, INNOVIRIS requires that the patients are really involved and not just consulted as “advisors”. The highest degree of involvement is required in the Co-Create program where the concerned people (i.e. the patients in health related projects) should have

control of the research process and involved as co-researcher. As projects involving patients are still ongoing, no feedback is available yet.

12.8.2 FWO - Research Foundation-Flanders

12.8.2.1 Missions

The Research Foundation – Flanders (FWO) is a public utility foundation aiming at financing fundamental and strategic research at the universities of the Flemish Community. It also stimulates the scientific cooperation between Flemish university research centres and other research centres.^{aaaaa}

12.8.2.2 Rationale for involving patients

Most FWO research resources are allocated to fundamental research, for which no thematic priorities are set. As the primary focus of these programs is on generating *de novo* knowledge, patient involvement is not a specific point of attention.

Patient associations are mentioned as potential stakeholders in the manual for applicants for some specific funding programmes. For example, according to FWO, the 2019 call for applied biomedical research with a primary societal finality (TBM) places more emphasis on involving patients or patient organizations in the advisory committee, although this inclusion remains the choice of the applicant. Patient associations or individual patient involvement could be considered as a selection advantage as the scoring grid of applications considers the relevance and the adequacy of the advisory committee.^{bbbbb}

^{aaaaa} See more here: <https://www.fwo.be/en/>

^{bbbbb} See here the score grid: <http://www.fwo.be/media/756828/TBM-Score-grid-2018.pdf>.



Another example of patient involvement in the FWO programmes: the role of the patient committee for *Kom op tegen Kanker* is described in the call text.^{cccc}

12.8.2.3 Forms of patient involvement

For the *Applied biomedical research with a primary societal finality (TBM)*-program, the FWO recommends the set-up of an advisory committee to support the valorisation of the research results. This advisory committee should, depending on the type of research, be composed optimally and will often also contain patients. It is also expected that the advisory committee is actively involved before, during and after the project, in order to guarantee an optimal transfer of the research results. According to FWO, experience with funded research projects showed that if the right stakeholders were not involved in this advisory committee, the projects had lower chances of success with respect to utilisation of the results.

An advisory committee is mandatory for the *Strategic Basic Research*-program. As non-clinical research is also supported within this program (the program is open to all science disciplines), patient involvement in the committee is not a requirement. The composition of the advisory committee is evaluated per project. If the evaluators believe that involving patients is crucial for the success of the project, this is also taken into account in the evaluation.

For projects supported by the FWO within the *Kom op tegen Kanker* programme, patient involvement was not a requirement until recently. For the current call, a limited and structured form of involvement of patients in the form of a patient committee was implemented, mainly to determine the topics of the project call and the assessment of project applications. For the assessment of applications, the FWO will require from the applicants a brief explanation of the relevance and short-term impact of their research project in a language accessible to non-experts. On the basis of this, the patient

committee will assess the proposal's relevance and the project's possible short-term impact for patients. The patient committee will formulate a non-binding advice that will be submitted to the scientific committee.

With regard to fundamental clinical research, there are currently no expectations in terms of patient involvement as researchers supported by these programmes still focus very much on de novo knowledge creation.

12.8.3 F.R.S.-FNRS- Research Foundation Fédération Wallonie-Bruxelles

12.8.3.1 Missions

Similarly to the FWO, the *Fonds de la Recherche Scientifique (F.R.S.-FNRS)* is a public benefit foundation aiming at promoting and funding research in the Fédération Wallonie-Bruxelles. The F.R.S.-FNRS acts as an umbrella organisation, managing various associated research funds with their own rules and practical criteria. Most of the subsidies of the F.R.S.-FNRS come from the Fédération Wallonie-Bruxelles, and are allocated to fundamental research. By law, the F.R.S.-FNRS could not establish a research agenda or research priorities, the research being financed by a bottom-up approach. The assessment of the research proposals is based on the excellence of the proposals, whatever the topic.

12.8.3.2 Rationale for involving patients

Currently, due to its legal framework, it is difficult to integrate patient involvement in the F.R.S.-FNRS process and there is no funding available to support this mechanism. The F.R.S.-FNRS could therefore be involved in networks promoting patient involvement regarding the definition of the research priorities. As there is no distinction between regional and

^{cccc} See the complete call text [here : http://www.fwo.be/nl/actueel/opoeppen/onderzoeksproject-kom-op-tegen-kanker/](http://www.fwo.be/nl/actueel/opoeppen/onderzoeksproject-kom-op-tegen-kanker/)



community funds in Flanders, the FWO has a greater flexibility than the F.R.S.-FNRS.

Under some circumstances, the patient perspectives should nevertheless be heard to help to prioritize the research agendas. If a major impact is aimed at, it is not viable to work with researchers only: other actors should be involved, including patients. However, patient involvement should not jeopardize a form of independence regarding priorities. A research agenda not constrained by a defined topic gives more important results on the long term. Developing shared decision-making could lead to some resistance: involving patients could “contaminate” other disciplines and stakeholders may wish to be involved too. There is a risk of breaking with the “research freedom”.

« Il n'y pas de grande découverte si la recherche n'est pas basée sur l'excellence. »

(Quote from an interview)

If there is a wish to give patients a voice in the selection process, there is a need for validity and credibility of the patient perspectives. This raises the importance of the representativeness of patients and their legitimacy. In that sense, patient involvement via patient associations is preferred as it supports a broader perspective than the individual experience and may allow for a “professional” exchange, when facing scientific experts.

12.8.3.3 Forms of patient involvement

In the research funds allocated by the Walloon region, the aspects of innovation and societal impact are dominant: e.g. the submitted projects should take into account the applicability and transferability of the patent. The constraints and limits are therefore more focused on the processes than on the profile of the persons to be involved (so far, no patient has been involved in such process). Again, the allocation of research funds is based on the excellence.

The FWO and the F.R.S.-FNRS also manage the EOS funds, directed by a management committee: this could allow for the integration of the patient perspective.^{dddd} The EOS funds are cyclic funds, with specific legal decrees at each opening, allowing for flexibility in its functioning. This could be a venue to integrate patients' voices, at the condition it makes sense for the research projects.

Patient involvement is, however, made possible when considering the participation of the FNRS into (inter)national networks, promoting or supporting patient involvement such as the European Joint Programming for Rare Diseases (EJPRD). In the case of the EJRD, the objective is to coordinate the different European agencies active in the field of rare diseases: as the number of rare diseases is high (more than 6000) but concerns a very limited number of patients, federating researchers is of the utter most importance. The EJRD has two different calls for research proposals: 1) open calls, with a bottom-up approach; 2) thematic calls, for the EJRD has launched a shared decision making process with experts and patients.

The FNRS also manages private funds, which is not constrained to specific legal terms (at the exception of the testamentary dispositions in case of donations. This funds was recently used to open a specific call on Climate, with the requirement of having a research program based on solutions.

« On pourrait avoir une plus grande souplesse par rapport à ça [l'implication des patients] si on est séduit par une étude ou si on obtient la preuve qu'impliquer les patients est efficace dans les priorités, alors on peut ouvrir le fonds privé à l'implication des patients comme celui-ci échappe aux contraintes politiques ».

(Quote from an interview)

^{dddd} See more here: www.eosprogramme.be/



12.8.4 King Baudouin Foundation (KBF)

12.8.4.1 Missions

Created in 1976, The King Baudouin Foundation (KBF) is a public benefit foundation, funded by donations and public subventions. *“The Foundation is an actor for change and innovation, serving the public interest and increasing social cohesion in Belgium and Europe. We seek to maximize our impact by strengthening the capacity of organizations and individuals. We also stimulate effective philanthropy by individuals and corporations. The Foundation’s key values are integrity, transparency, pluralism, independence, respect for diversity, and promoting solidarity. The Foundation’s current areas of activity are poverty and social justice, philanthropy, health, civic engagement, developing talents, democracy, European integration, heritage, sustainable development and Africa and developing countries”.*²³⁹

The KBF has developed a specific expertise in methods supporting multipartite and stakeholder involvement.^{eeee}In 2007, the KBF already published a major report on the Patient participation in health care and health policy, aiming at reviewing the current international and Belgian initiatives regarding patient participation.

12.8.4.2 Rationale for involving patients

The KBF has a longstanding tradition of citizen involvement, with a spectrum of activities beyond the healthcare sector. Patient and citizen involvement is rooted in the democratic participation and the empowerment of the

individuals. Patient involvement is also perceived as a form of civic engagement. In addition, involving patients add consideration for the quality of life. It helps the researchers (and other actors) to connect with the needs and expectations of the patients and/or the citizens.

In all the projects involving patients or citizens, the KBF is the guarantor of the process and of the methodology. It provides methodological and logistic supports but, above all, the KBF ensures that all voices are equally heard and respected.

More and more researchers appear to be willing to involve patients but often lack of competences and skills to achieve it. Once experience has been built up, researchers are usually willing to reiterate the experience.

12.8.4.3 Forms of patient involvement

There are currently 80 funds supporting health research with varying degrees of patient involvement. Partnerships with patient organisations, involving patients and/or patient organisations in the (pre)-selection process are developed for some calls. Recently, three pilot projects aimed at setting up a dialogue on research priorities between the patients and the researchers: 1) working together to set research priorities for Non Alcoholic Steato Hepatitis (NASH) with an optimal contribution of biobanks^{ffff}; 2) working together to set research priorities for Tuberous Sclerosis Complex⁹⁹⁹⁹ and 3) returning to work after long-term work incapacity in partnership with the National Institute for Health and Disability Insurance.^{hhhhh;iiii}

^{eeee} <https://www.kbs-frb.be/en/Virtual%20Library/Result?serie=0465bb55-977c-422f-81ff-478cfe44dd5d> // <https://www.kbs-frb.be/en/Virtual-Library/2007/294967>

^{ffff} NASH project: <https://www.kbs-frb.be/en/Activities/Publications/2018/20180822PP>

⁹⁹⁹⁹ Tuberous Sclerosis Complex: <https://www.kbs-frb.be/en/Activities/Publications/2018/20180418PP1>

^{hhhhh} Long-term work incapacity: <https://www.kbs-frb.be/en/Activities/Publications/2018/20180418PP>

ⁱⁱⁱⁱ Results of the three projects: <https://www.kbs-frb.be/en/Activities/Publications/2019/20190118avc1>



Patients are usually involved through stakeholder consultations and meetings: they could be invited to reflect on the different stages of the process.

In a project on palliative care in geriatrics, one of the permanent citizen panels of the KBF was solicited by the research team to discuss about the communication with patients and families. The permanent citizen panels of the KBF involve a group of citizens that are solicited to discuss issues and topics submitted by researchers or other key actors. A recent project involved a panel of 32 citizens on the use of genome information in healthⁱⁱⁱⁱ.

12.9 Lessons learnt: consensus

12.9.1 Patient involvement needs to make sense

Patient involvement in research is considered as positive by all interviewees, as long as this **involvement makes sense** for the research project, the patients and the researchers. Involving patient is likely to increase of the value of the research and to reduce the “waste”. However, some interviewees stated that involving patients in fundamental research is not relevant. Patient involvement should not be compulsory because this could lead to a “tokenistic involvement”. Some interviewees highlighted the risk of instrumentalisation of the patients by the researchers or other actors.

« Au moment où ça nous arrange dans les conditions qui sont définies par les chercheurs et non pas par les patients »

(Quote from an interview)

The decision of joining a research project (or not) should remain in the hands of the patients, as well as the decision of involving patients should remain the responsibility of the researchers. One interviewee talked about the

“predatory” side of research, where patients are forced to join research projects.

“Do not strive to the dictatorship of patient participation. Patient involvement must make sense, should NEVER be a checklist exercise”.

“Impliquer un patient lambda pour dire qu’on a impliqué un patient »

(Quotes from an interview)

Patients should not replace researchers but should be considered as adding a **complementary expertise**. Patient involvement help to make sense of the reality, add transparency to the process, support knowledge and practice transfers, identify the needs ... For some interviewees, involving patients is first of all a question of **democracy**. Another warned that researchers and professionals can become over-enthusiastic about what they can expect from patients in a research project, leading to a level of ‘freedom’ perceived by patients to take decisions without prior discussion with the research team. This is related to the reserve expressed by some interviewees that experiential knowledge of patients should not replace scientific knowledge but rather be considered as a different kind of knowledge that can serve different objectives.

The involvement of patients may question the **legitimacy and the hierarchy of the knowledges** but it is of particular importance to preserve the expertise of the patients and the researchers. According to some interviewees, the expertise of patients should not be overrated while the expertise of the researchers should not be de-legitimatised. There should be a **win-win relationship**, in which different types of expertise and knowledge are respected and heard. Another pitfall to avoid is the “over-responsabilisation” of patients.

ⁱⁱⁱⁱ See more here: <https://www.kbs-frb.be/fr/Activities/Publications/2018/20180712PP>



To help making sense, involving patients should have a **clear and defined objective**, explicitly communicated to patients.

So far, none of the projects investigated made a formal evaluation of the impact of their patient involvement activities on the patients who were involved. Interviewees report the **empowerment** of patients as positive outcomes. Feedback from patients are usually positive: it gave them a **sense of pride and usefulness**. For example, in the research on the living conditions of HIV patients, alongside the scientific report, patients edited a memorandum that is still considered as a reference document by public authorities. For some patients, being involved in research become a “real” job. One interviewee reports “**feeling a difference in the outcomes**” when patients are involved from the start of the project. Involving patients may help to (re) focus on the objective, as it was reported in a jury on multiple sclerosis: the experts were debating on the scientific aspects and the patient asked them to focus on the quality of life of the patients, as it was the objective of the call.

Researchers with a clinical background – i.e. medical doctors or nurses – perceive that **patients feel more involved in their healthcare** and are more compliant. Researchers also learn to work differently and perceived their research as more concrete, better suited for the “real” world. The presence of the patients in ethical committees helps to make language more accessible.

As patient involvement may help to identify unmet needs, there could be a secondary benefit for patient associations: unmet needs –not addressed in the research projects– should be communicated to the patient associations that may include it in an advocacy paper or a memorandum. Involving patients may also **help the associations to gain legitimacy**.

From the perspectives of the funders, patient involvement **should not be compulsory** in all research projects as it may inhibit the development of original and innovative research findings. Patient involvement should not be a “box to tick” but need to make a real difference in the process. It is thus recommended that the calls for research proposals include the “why” and “how” of involving patients. In other words, the applicants should justify why they need to involve (or not) patients and which methods they will use to

make this involvement effective. To prevent the risk of the “box to tick”, the research call should be formulated in a language allowing for the most inclusive and appropriate approach. This is likely to raise the awareness of the researchers and to inform the final decision of (not) involving patients. Some interviewees suggested to add these questions also to the annual call for proposals of the KCE.

12.9.2 Patient involvement needs to be prepared

All interviewees agreed that patient involvement should be prepared. One interviewee even states that there cannot be involvement without **structural support**. The opinions about the magnitude and the content of the preparation are diverse. Some interviewees recommend a formal training about the research topics or the research methods while other emphasise the need for acquiring communication skills or health literacy. One interviewee insisted on the need to valorise the existing background of the patients and to consider not only the experiential knowledge of patients but also the professional knowledge of patients. In other projects, preparation consists in a pre-digestion of the results or the information by the researchers or the health care professionals (i.e. when patients are involved in the interpretation of the results).

Preparation and support is also needed for the researchers: they need to know why and how they will collaborate with the patients. They may need extra training regarding communication and vulgarisation of their scientific discourses. Researchers should be ready to have their knowledge and proposals questioned by “naïve” participants and accept that the research process is likely to become iterative.

This preparatory step should preferentially be done before the formalisation of the project to ensure that the patients have all the elements necessary to understand the research process.

Several interviewees also insist on the need for a **confidentiality agreement**, before the start of the project, especially when sensitive issues are targeted by the project. Some interviewees insist on the need for a clear framework to guide the patient involvement. Some suggest having a “contract” between the patients and the research team: it may allow for



reflexion, formalizes the process and may frame the participation. As pointed out by an interviewee, as the participation of patients is mainly voluntary, it is difficult to bind them: a form of “contract” could be then helpful. This could be negotiated between patients and researchers as a first step of the process.

Interviewees also insist on the **practical preparation**. Attention should be paid to all day-to-day details and need to take into account the **specific needs of the patients**: avoiding long meetings, considering dietary needs, transportation issues, hours of the meeting, day-care for children, patients needing constant supervision from relatives, or language used (i.e. German-speaking Belgian patients are often wrongly considered as fluent in French). Hosting meetings in a hospital meeting room could lead to a different atmosphere than hosting a meeting in a community centre or in the cafeteria of a nursing home. Moreover, researchers need to consider possible vulnerabilities.

Researchers could be helped in such **preparatory work**, by the patients themselves, by representatives of patients or by experts in multiple stakeholder discussions. An asset to projects involving patients is having a neutral and experienced facilitator.

12.9.3 Patient involvement takes time

The **time component** is particularly critical before the formal meetings between the patients and the researchers. Involving patients in the research needs a well-planned preparation: there is no room for improvisation and all participants, researchers, patients or professionals, should be carefully prepared to join the process. It could also be necessary to devote extra time for a specific preparation of the patients / the researchers but interviewees did not converge about the content and the amplitude of this preparation.

If the process is well-prepared, it is possible, according to one interviewee, to obtain results after a single work session of 2 hours but this will depend on the objectives of the project.

Moreover, researchers often neglect the importance of maintaining a **regular communication** with the participants: this also could take time but

is considered, by some interviewees, as a major issue. Communication should be adapted to the needs of the patients and this may require extra time.

12.10 Diverging issues

« Chacun fait à sa sauce, il n'y a pas de recette magique »

(Quote from an interview)

Interviewees diverged about the following issues:

1. profile of the patients to be involved;
2. topics;
3. financial aspects related to patient involvement and
4. steps of the research process where involving patients makes sense

Above all, the vocabulary and the concepts related to patient involvement, patient participation or patient implication diverged from one interviewee to another. As an illustration, one interviewee recommended the use of “involvement” as “*it goes beyond participation*”. Others relied on participation as this is more frequently used, although one interviewee stated that it could lead to confusion and erroneous use as it is “*à la mode*”. Moreover, interviewees noted that researchers, patients and health care professionals have a different idea about what constitutes ‘participation’.

12.10.1 Who should be included as patients?

The profiles of patients to be included leads, for some interviewees, to concerns about the representativeness of those involved: **involving patients, but who? Who is speaking for who?** Some also question the motivation of the patients to participate. In fact, it appears that the profiles and the number of patients depend on the methodology and the final objective of the involvement.

Some interviewees only rely on patients who are **members of patient associations** while others strongly discourage the involvement of this kind



of patients. Supporters of involvement through patient associations argue that these patients are able to share perspectives beyond their own individuality and may represent various perspectives. Patient associations could also offer backup support and training. It also eases the recruitment process and the adequacy of the profiles regarding the project. Patient associations have also a legitimacy, especially when there are spokespersons with a clear mandate. Those advocating the involvement of **patients on their own behalf** stated that members of patient associations share an ideological and political message that may prevent an open dialogue between researchers and patients. They also question the representativeness of these associations, even the “monopolist” position of some of them. Some interviewees perceive a “formatted discourse”, “a homogeneous discourse” of patient associations. Moreover, some interviewees state that not all patient associations are of equal force, as some have developed to a “professional” organisation (i.e. with salaried workers, official spokespersons) while others remain lay initiatives, supported by one or two persons. For some interviewees, if there is a good patient association, it is appropriate to work with them but, if not, they advise to work with an organisation that has a database of patients (i.e. a sickness funds or a hospital). Some interviewees question the funding of some associations, although efforts have been made to preserve the independence of patient associations. An interviewee also noticed that patient associations are more and more solicited and lack time to participate effectively in research projects.

One interviewee discouraged the involvement of patients who have a professional background in healthcare or as researchers, while another interviewee clearly insisted that these kinds of profiles are likely to add an interesting perspective that might benefit the research project.

For some topics, some interviewees recommend involving **citizens rather than patients**. This may be of interest when the research concerns organisation of health care, financing or aspects related to prevention and health promotion. As an example, in the pilot project about NASH, the participants involved patients and citizens, as there is no NASH patient organisation.²⁴⁰ Similarly, the sickness funds represent not only patients but

also health care consumers and citizens. Involving citizens may also raise awareness about an issue. Involvement of citizens should be done professionally to ensure a good representation of the public.

One interviewee recommends avoiding as much as possible the political aspects:

“If you succeed in leaving out the political part devoted to the public institutions, this helps but it should be considered later in the process. You may need to let people play the game: i.e. access to confidential information: we know it’s existing but we cannot use it right now.”

For this interviewee, when the topic has a lot of financial and political implications, it was important to have individuals representing their own individual and personal experiences and agendas and not the political agenda of a patient association or a lobby. Similarly, in some issues, not only patients should be involved but also other actors: researchers need to ensure that **all stakeholders** are represented. Some interviewees recommend avoiding “positive discrimination” while acknowledging that supplementary efforts should be made to involve patients as they are perceived as more vulnerable.

Interviewees also diverge regarding the **stage of the disease or the health condition** the patients are in when involved. Some interviewees advise having patients in an acceptance phase, that is, patients who have accepted their diagnosis, while others consider that patients in the early stage of the disease could bring another expertise and experience, as long as they are able to take distance from their situation. Some pointed out that confronting patients with different stages of the diseases need to be carefully prepared: for some patients, it could be harmful to be confronted with others at a later stage of their disease or having experienced complications (i.e. amputation of a leg due to an unbalanced diabetes or severe malformation because of non-response to a treatment).

For some interviewees, having non-compliant patients or patients using illicit drugs or alcohol should be avoided while others believe that these patients



could be involved, as long as these persons know how to cope with their condition. Similarly some interviewees discourage working with patients with psychiatric and mental health problems while others are in favour of including them, but again with a cautious preparation. Finally, for some interviewees, it makes more sense to involve patients with a chronic disease as they have a more extensive experience with the healthcare system than those with acute healthcare problems.

Some interviewees highlighted that, beyond the stage of the disease, patients likely to be involved should **first have their healthcare needs ensured**: it makes little sense for a patient to be involved in a research project if he or she cannot access the health care system. For example, in the German-speaking part of Belgium, patients face barriers to access specialty care as there is a lack of specialists who are fluent in German.

Interviewees also diverge about the **number of patients** to be involved simultaneously. Some projects are developed with a group of patients, sharing various types of expertise and representing the diversity of their “community”, while, in the patient-partner model, it is advised to have only one patient per project to avoid that patients “pollute themselves”. Depending on the project, it could sometimes be easier to involve “patient-experts” than “patient-partners” and vice-versa.

Some interviewees also raise concerns about the “professionalization” of the patients: some patients are so involved in projects that they lose their “profane” perspective. Another interviewee points to the risk of having “patients who are ‘in the pockets’ of the researchers”, that is patients always willing to participate, ready to say what researchers want to hear and not questioning the proposals. This could be influenced by the nature of the project itself: some projects require having patients familiar with scientific approaches, able to read the scientific literature in English. Some interviewees advise to have a limited mandate for patient representatives to ensure a diversity in the representativeness. One interviewee also reports that some patients do not recognise themselves in what the patient with a longer experience with a (chronic) disease is saying. For this interviewee,

such “more experienced” patients could have a frightening effect on other patients that are still in the earlier phases of their disease.

It should be noted that none of the interviewees mention the participation of children. Besides, most of the interviewees stress the need for a better involvement of marginalised groups, as those joining the research projects are often well-educated and have a high degree of health literacy.

12.10.2 Which topics should be investigated?

For some interviewees, there is no restriction on the type of topics that can be discussed with patients. It may require extra training of patients to allow for real involvement of patients for some topics. This could include the development of a shared language: one interviewee was referring to “translation” of the research. Some interviewees consider patient involvement particularly important when the topic is highly controversial. Other interviewees recommend involving patients only in topics with a direct impact on patient experience and avoid “purely medical” topics / fundamental medical research. It has also been mentioned that patients should have a benefit of being involved. One interviewee stated that patients should primarily be interested in the topic and that they need to have a real motivation so that their involvement is relevant. Some also discouraged the involvement of patients in studies concerning children or expensive health care costs, fearing that “emotions” would impede the discussions. One interviewee feared that sometimes research questions are too large for the patients to grasp the real implications, and that “big questions” should be split up in smaller and more specific research questions.

An overall conclusion could be that a good balance between patients’ and researchers’ expertise should be sought when designing a study and considering patient involvement in different research phases.

“A priori, on peut impliquer les patients partout mais il faut voir avec les chercheurs ce qu’ils en pensent »

(Quote from an interview)



12.10.3 Should the patient be paid?

The remuneration of the patients is a crucial point of divergence: for some interviewees, participation should be a form of volunteering and no financial incentive should be offered to the patients. For others, a financial contribution should be offered. One interviewee suggested that the question of the (financial) compensation should primarily first be discussed with the participants themselves.

The remuneration could take the form of vouchers, but at least some kind of compensation for patients' time and effort should be granted (e.g. a goody bag, book voucher, travel voucher or training). Hosting a "party" or "thank-you" event is also recommended by some interviewees. Offering a financial incentive also allows for including "young" patients or those with a professional activity to ensure a larger diversity of the participants.

Some organisations hire part-time "experts by experience". For some patients, however, it may not be desirable or feasible to receive fees or have a "contract" as it may negatively impact their sickness or retirement allowances. It was mentioned that, in France, citizens have the right to a "journée d'absence rémunérée" (paid day off) to allow them to participate to research projects. Some report that payment could also be transferred to the patient association to which the patient belongs, as it is considered as a collective benefit.

Some interviewees state that being involved does not always require a financial incentive. Patient participation is motivated by a sense of commitment towards others. One interviewee mentioned that none of the participants to their projects is paid, whatever its status. However, other interviewees fear that, without financial incentives, it is complicated to motivate participants.

« Il y a des patients qui s'engagent pour leur pathologie, pour leur maladie, pour les autres : ce n'est pas l'argent qui va les motiver »

« Whenever I can help to bring the message »

(Quotes from an interview)

All interviewees agree that catering and transportation costs should be reimbursed by the research centres.

12.10.4 In which stages of the research should the patient be involved?

Interviewees diverge about the stages of the research in which patients should be involved. There is a general consensus that involving patients makes sense in the scoping of the project as it may contribute to a better targeting of the research questions or to the identification of the research priorities. Helping to define the problem is consensually reported as the added value of the patient involvement at this stage. For some interviewees, patients could also be involved in research by submitting a topic of interest for research, although they may need support to translate an unmet need into a research proposal. While some are reluctant to involve patients at this stage, others recommend to have faith and trust in the patient experiences: patients could also be aware of the needs for fundamental research. One interviewee explained that, in their case, patients are involved after the development of the research protocol. Another interviewee stated that, in her experience, patients do not want to be bothered too much with the in-between phases: this increases their confrontation with their disease and may increase their fatigue.

Some interviewees recommended to rely on patients for the recruitment strategy of participants for the actual data collection. Patients could act as intermediary, especially the patient associations, but should not be the ones deciding the final inclusion of the participants.

Patient involvement in the choice of the design and all aspects related to the methodology of the study is less supported by the interviewees, as this is considered by some interviewees as the specific expertise of the researchers. For some interviewees, patients could however be involved in choosing between different instruments or testing the data collection instruments. They may help to develop the instruments to make them more appropriate but interviewees advise not leaving the patients on their own in this step.



Interviewees usually recommend to keep the data collection under the responsibility of the researchers: only two projects relied on patients to collect data from the field. Particularly in Community Based Participatory research projects or in action research projects, involving the patient as co-researcher is one of the basic components of the methodology. If the patients are involved in the data collection, attention should be paid to their “comfort” and their support. As stated by one interviewee, in one project, patients interviewed other patients and were confronted to social issues that they were not aware of.

Similarly interviewees prefer that researchers manage the analyses, although patients could be involved at this stage too. Some interviewees suggest patient involvement after the first data cleaning and analysis: for them, patient perspectives make more sense on defined topics or on pre-analyzed data (i.e. patients could receive descriptive results before being invited to a focus group to discuss it more in-depth). In that sense, for interviewees, patient involvement concerns more the interpretation or the contextualization of the results rather than real analyses.

For some interviewees, in projects with practical deliverables, patients could be involved in the development and testing of the interventions, as done by the LiCaLab.

Reporting is never mentioned as being managed with patient involvement, while the formulation of recommendations could be done, for some interviewees, with the support of patients. In this case, patients help to make the recommendations more readable for other patients and citizens. It is important, though, to distinguish the research conclusions and the final recommendations, especially when the latter are written up for policymakers. As an illustration, it may emerge from the results that there is a perceived lack of information support, despite the fact that there is a lot of information support available: the recommendation could then not be that there must be more information support, but rather that patients should be better informed about where they can find the information.

Depending on the nature of the outcomes, dissemination and implementation could involve patients but there are few experiences with this so far.

The involvement process, methodology and its consequences (e.g. consensus about the interpretation of results in a participatory approach) should be respected up to the end and even beyond the research project. It is not acceptable that one of the partners would try to oppose against the agreed methodology and conclusions after having accepted its terms.

Finally, some interviewees also recommend to think about “when” to involve participants (and not only patients).

12.11 Enablers of participation

12.11.1 Supporting organisational and legal context

The context is of particular importance: if the management or the institution has a strong commitment towards patient involvement, obtaining resources and support for such kind of initiatives is easier. For some interviewees, patient involvement has to be inscribed in the institutional strategy. For some patient groups, there are international recommendations supporting their involvement. For example, since 1994, the “Greater Involvement of People Living with HIV and AIDS (GIPA)” recommends the involvement of persons living with the HIV/AIDS in research projects to better consider their perspectives and needs. This is an important enabler as it gives legitimacy to the process of patient involvement. Similarly, a legal incentive is also perceived as an enabler of participation, as it was the case for the Observatoire des Maladies Chroniques or the ethical committees. Clear structures help to fix the opportunities as well as the boundaries of patient involvement: they may prevent attempts to re-discuss and influence the conclusions after the end of the project and avoids that researchers have to justify their choices and methodologies when presenting their final results. If patients are involved in the structures, they have been fully informed about the reasons for the choices.

Some interviewees advise having a steering committee involving patients to guide the research process and to ensure its adequacy at each step of the project.



12.11.2 Relational aspects

Attention should be paid to the relational aspects: patients and researchers should feel respected and legitimated in their respective expertise. A climate of trust and exchange should be ensured and efforts should be made to create a **“win-win” situation**. Both researchers and patients should have the willingness to work together. Patients should feel safe and be adequately prepared: if needed, a training should be organised. The minimal preparation should be a clear explanation about the objectives of the research. Interviewees recommend having a “coach” for the patients, so that they could share their experience of participation with a “trust person”. Other interviewees rely on a professional facilitator, with a neutral position, able to “feel the tension of the room” and to ensure the balance of power between participants.

Having a **confidentiality charter** is of particular importance to ensure the safety and the respect of each participant, health care professionals and researchers included. Safety and comfort could be reinforced by having a dedicated place for the research activities: patients may then become familiar with the place and feel members of the research (this is particularly important when the research requires more than one meeting). One interviewee stated the importance of feedback to patients. During the research activities, some interviewees recommend having sufficient informal contacts between participants. Moreover, some interviewees have a positive experience of setting-up an informal committee alongside the formal committee, as this may help to build consensus.

Some interviewees insist on the image sent by the researchers to the patients: in a “perfect” world, patients should already know the researchers beforehand. Researchers should be on the field to “take the temperature”, e.g. attending events of patient associations, reading position papers or holding informal meetings. Having a **permanent concertation platform** with patient associations or patient representatives could be good practice.

12.11.3 Valorisation of the patients ‘contribution

The patient voice should be legitimised and acknowledged as such: the patient experience should not be considered as “anecdotic”. Patients should

be **formally acknowledged** for their contribution: they could participate to conferences, be associated to the redaction of documents or be mentioned in the institutional organigram. In some research centres, the volunteer status of “scientific collaborator” is proposed to the patients.

12.11.4 Preparation of researchers

Researchers should **be prepared to work with patients** and **determine the framework** in which this involvement will take place, which boundaries are necessary/desirable and which adaptations they are ready to make to ensure involvement (i.e. working outside regular hours). Some interviewees recall that the researchers should endorse their own research questions that may diverge from the perspective of the patients. On the contrary, one interviewee reports that researchers need to be humble and that, by involving patients, the project is no longer their sole property. It also appears that some research methods or designs are more suitable to involve patients than others.

As pointed out, researchers are often willing to involve patients but often lack the skills to do so in an appropriate manner. Exchanges of experiences between researchers help to decrease possible resistances. In that sense, the King Baudouin Foundation was cited as an example of good practice in Belgium. Interviewees also reported numerous international initiatives that support patient involvement in research and that may serve as an inspiration for Belgium: the consultation panels of NIVEL, the Montreal model of the patient-partner, the James Lind Alliance or the Nederlandse Patiëntenfederatie.

12.11.5 Definition of the role of researchers

Researchers should be **clear about their duties and responsibilities as researchers**. The processes and the methods should be rigorous, patients could be involved at all stages of the research process, as long as it makes sense and is well prepared. One interviewee stated that the final scientific report should stay “in the hands of the researchers”: discussion may take place with the patients about how to present the results but the final responsibility of the scientific aspects remains with the researchers.



Some interviewees advise to listen to emerging issues but to **stay focused** to avoid dispersion and frustration. While setting-up the scene, the researchers should make clear that if something outside the focus comes up, it will be kept but that the group has stay to the focus of the research. The role of the researchers should then involve the transfer of such information to the relevant actors. One of the pilot projects of the KBF lead, for example, to the redaction of a book gathering all the emerging results.

“What comes up is not necessary on a research question but might inform about the needs of patients”

(Quote from an interview)

12.11.6 Definition of the roles of patients

To avoid frustrations and to ensure meaningful involvement of patients, some interviewees recommended considering three groups of patients: patients as citizens, patients as experts and patients as representatives. Depending on the group, a specific role and related activities could be defined. One interviewee suggested to define clear criteria to assess whether patient involvement is relevant and, when relevant, whether this involvement is effective and meaningful.

12.12 Barriers to participation

For some interviewees, the context can either support or jeopardize patient involvement in research, e.g. if the legal framework or the organisational context does not support patient involvement. A frequently reported barrier to patient involvement is the lack of financial resources and the time needed to prepare the process of patient involvement. For one interviewee, politics are against priorities in fundamental research as there is a need for a return on investment. Even if a consensus emerges about the need for more patient involvement, resources do not support it. For some interviewees, organisations should consider it a genuine task, to which resources should be devoted, and not a side-activity.

Within a project, the **time component** is also important: a patient is not able to read 20 scientific papers in one week. Patients are also not always readily available for researchers, as they might have other activities.

The **financial aspect** could also be a problem for patients with a professional activity, for whom meetings during office hours might not be easy to attend. As a consequence, those being able to participate during office hours are either retired, on long-term leave or have the professional possibility to take a day off to join a research project.

Transportation issues are additional barriers. Not all research centres are made accessible to patients. For some patients, arranging transport to access health care is already problematic. They may not be willing to spend their energy for a research project. Some interviewees advise to develop outreach strategies, to meet the patients in their community.

Involving informal caregivers of patients might even be more difficult: not all of them benefit from a special status and they have often numerous priorities to cope with before being able to join a research project. If no specific arrangement is made, they are unlikely to participate. For example, a relative of a dement person could not let him or her alone at home to join a focus-group.

The limitations related to the **health conditions** or the **social situation** could impose another barrier: it requires flexibility from the research team and the development of extra strategies to ensure the regularity of the



presence and the retention of the patients. When involved in the data collection and data analysis, patients need to be ready to discuss about their experiences and to hear harsh things: this is not always desirable. Some patient profiles are more difficult to involve: patients from ethnic minorities, those with low socioeconomic status or poor health literacy. The symptoms of the patients such as pain or fatigue could prevent them from attending research activities. Moreover, the unpredictable character of some diseases might impede a regular involvement of the patients.

Several interviewees warned for the **risk of exhaustion** of patients as those participating in such kind of projects are often already solicited for other projects or volunteer activities. This could in particular occur when addressing “niche” topics”, where the patient community is by definition limited. For other interviewees, the requests to involve patients exceed the availability and resources of the patient associations. Some interviewees prefer then investing in a limited number of projects to ensure the quality of the involvement.

Researchers may “overestimate” the possible contribution of the patient and may be disappointed if the contribution does not match the expectations. It occurs that the researchers have **disproportionate expectations about the contributions of the patients** and vice-versa: some patients may expect finding a solution to their individual problems by joining the research. If the request addressed to the patients is not clear, the motivation to get involved in the research project could be jeopardised. The perception of researchers and health care professionals of what involvement actually implies could constitute an additional barrier. Finally, health care professionals and researchers may also be concerned about confidentiality. Involving patients requires a change in the research paradigm.

Conducting a research project in partnership with a health care service or health care professionals might raise questions about the **therapeutic relationship**: patients could be involved in projects in which their own treating health care professional is involved too.

For some interviewees, it should be not neglected that, for some patients, the researchers, the medical doctors and other professional profiles occupy “powerful” positions. The **prestige** and the **power** related to the professional

roles could constitute a barrier to involvement for the patients as they may be afraid of challenging or questioning the ideas and opinions of such persons. Patients may feel unprepared to participate.

A final barrier could be the **lack of formal assessment of the impact** of patient involvement on the research results. None of the interviewed project leaders had conducted a formal assessment so far. This could constitute a barrier to the more general implementation of patient involvement in research, as there is no evidence of the positive or negative impact of patient involvement. Informal assessments support evidence that patient involvement has a positive effect on the research process: higher participation rate into surveys, better implementation of interventions, positive individual effect on patients...

12.13 Summary of lessons learnt from Belgian patient involvement experiences

In summary, we learnt that, to establish a successful researcher-patient partnership, it is important to:

- clarify the perspectives of what involvement implies before initiating the project;
- clarify the expectations of each party to prevent frustrations and misunderstandings;
- involve patients - and other relevant actors - as soon as possible in the research process;
- ensure that patient involvement makes sense to the research and to the patients;
- define a clear objective, with a work framework agreed upon by all actors;
- develop a strong and rigorous methodology – including the evaluation of the impact of patient involvement;



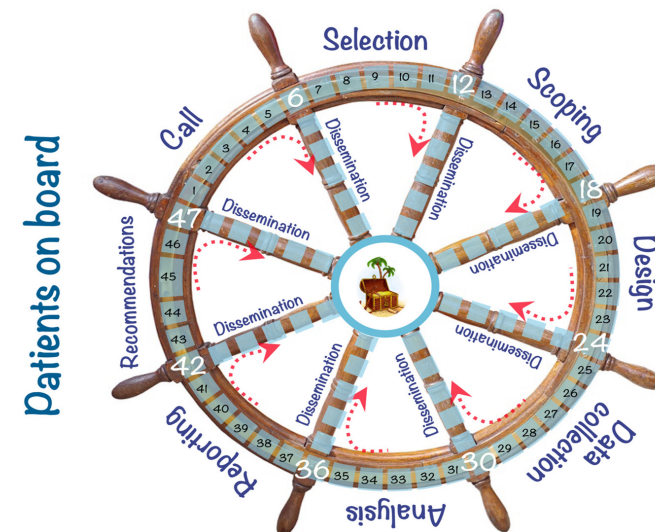
- plan and organise (a) feedback and (a) debriefing moment(s) with the patients;
- pay attention to the very practical aspects to ensure that everybody feels comfortable (physically and mentally).

13 PATIENT INVOLVEMENT CULTURE AT KCE

13.1 Method: KCE's "Patients-on-board"-game

We measured the existing patient involvement culture at KCE amongst all employees, using a nominal group technique. For this, we created a board game (KCE's "patients on board" game, Figure 4) as an instrument to collect KCE employees' perspectives on patient involvement in different phases of policy research processes.

Figure 4 – KCE's "Patients on Board"-game



We included the entire KCE staff in the game: researchers, supporting staff and management. Eight groups of 6 to 8 people were created to keep the group work manageable. Groups focussed on GCP, HTA, HSR or KCE



Trials respectively. Supporting staff, which are not directly involved in research projects, were asked to take the position of a patient if that would make them feel more comfortable. The members of the management, who all have a scientific background and experience with executing KCE research, were allocated to the different domain groups (HTA, HSR or Trials). Data analysts, who are involved in all domains, were put together in a separate group. All other researchers, even though most of them work in different domains, were allocated to the group of the domain with which they have most experience.

13.1.1 Objectives of the game

The objective of the game was to collect arguments for or against patient involvement or conditions for patient involvement in the different phases of policy research: call for proposals, selection/prioritization of topic proposals, scoping of the research project, design of the study, data collection, data analysis, reporting of the results, formulating the recommendations and dissemination of the results.

13.1.2 Organization

Each group was guided by a game master, who supervised the flow of the game, made sure the rules were followed, wrote down the arguments on post-its, and monitored the time. For each argument pro or contra patient involvement given by the person who has rolled the dice, another player could give a counter-argument. For every eligible argument and counter-argument the group received a Lego brick. With the collected bricks, the groups built a construction. An argument could only be given once per research phase.

After the game, every group selected its main argument pro, contra and its main condition for patient involvement, across all phases. This was brought to the plenary session, where all groups presented their two main arguments and one condition and put it in a global matrix. This was followed by a plenary discussion with all participants about the arguments and about patient involvement in general.

13.1.3 Data analysis

For each group, as well as for the entire group's main arguments matrix, a table was created listing all arguments pro, contra and conditions for patient involvement in all research phases. Two KCE researchers (WC and IC) analysed the data in several sequential steps: one coding round and two additional clustering rounds.

1. First, all arguments pro and contra and conditions for patient involvement in research were coded by one researcher. Initially, the wording of the nodes was deliberately kept close to the wording of the arguments and conditions. By consequence in this first coding step most nodes had only one reference, meaning that they were only mentioned once (and hence not by several groups). This resulted in 219 nodes.
2. Next, a second researcher clustered nodes with similar content. The number of nodes was thus reduced to 140.
3. Finally, 25 encompassing themes were identified and nodes clustered into these themes at a more conceptual level.

Patterns in arguments for or against patient involvement and conditions for patient involvement were sought, using NVivo 11 Software and Excel. A quantitative as well as a qualitative analysis of the nodes by group, research phase and theme was made.



13.2 Results

13.2.1 Quantitative exploration

13.2.1.1 Most frequently mentioned arguments and conditions across groups

After the initial coding, where we stayed very close to the original formulation of the arguments and conditions, we ranked the nodes based on the number of references per node (see **Error! Reference source not found.**). The fact that patients are well-placed to define the unmet needs in a specific disease area and should hence be involved in the call for proposals was mentioned five times, by three different groups.

In the context of the selection of topics for research, it was mentioned four times that patient involvement would require a new selection criterion for studies, such as 'relevance for patients'.

For data collection, the argument 'patients are a source of information', was mentioned four times as argument pro patient involvement. It is unclear, however, whether respondents sufficiently kept in mind that the objective was to assess the value of patient involvement in research as co-researchers, rather than as participants to research.

In the scoping phase, patients are thought to provide information that might be interesting for the researchers to identify what is necessary or interesting to include in the research questions.

In terms of arguments against patient involvement, patients' lack of knowledge and potential conflicts of interest were each mentioned three times, in respectively the design and analysis phase.

Table 18 – Top 7 of nodes ranked by the number of references after initial coding round

Node	Number of references	Number of groups
Call_Pro_Patients are well placed to define unmet needs	5	3
Selection_Condition_If new selection criterion is introduced	4	2
Data_collection_Pro_Patients are a source of information	4	4
Scoping_Pro_Listen to patients to know what is necessary or interesting	4	3
Design_Contra_patients lack knowledge	3	3
Analysis_Contra_Patients' conflict of interest	3	2
Recommondations_Pro_Who merits care must be determined bottom-up	3	1

13.2.1.2 Comparison between categories of personnel based on initial coding

If we look in more detail at the arguments provided by the different categories of KCE staff, we see that there is only limited overlap in the arguments provided by the different groups (Table 19).

The HTA and supporting staff group share 3 arguments:

- Call_pro_P are well placed to define unmet needs
- Scoping_pro_Listen to P to know what is necessary or interesting
- Scoping_contra_P lack scientific knowledge

The data analysts and secretariat groups share 5 arguments:

- Selection_contra_Patients cannot correctly score topic proposals
- Scoping_pro_Patients provide unique information



- Design_Contra_Patients lack knowledge
- Data_collection_Pro_Patients are a source of information
- Recommendations_Pro_Recommendations always impact patients

The table also shows the total number of arguments provided per category of staff. The HSR group is not surprisingly the group which produced most arguments, since it actually consists of two separate groups of players. They came up with 54 arguments. The group which produced the least arguments is the secretariat support group, with 22 arguments.

Table 19 – Number of arguments shared between groups

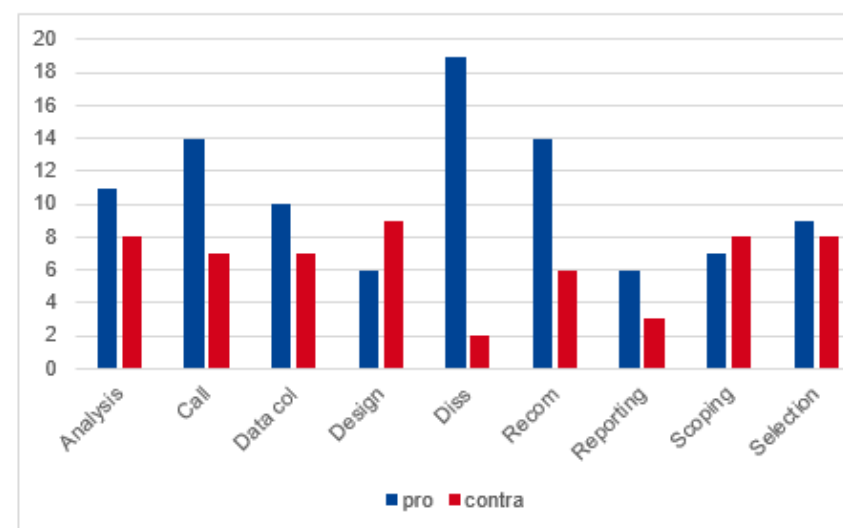
	Data analysts	GCP	HSR	HTA	Supporting staff	Secretariat	Trials
Data analysts	24						
GCP	0	32					
HSR	0	0	54				
HTA	1	0	3	31			
Staff	0	1	1	3	33		
Secretariat	5	0	1	1	2	22	
Trials	3	1	2	2	3	1	40

13.2.1.3 Comparison between research phases

Regarding the number of arguments per research phase we observe that (Figure 5)

- All research phases have more pros than cons except for the design and scoping phase.
- For the dissemination and recommendations phase the pros outweigh the cons the most.

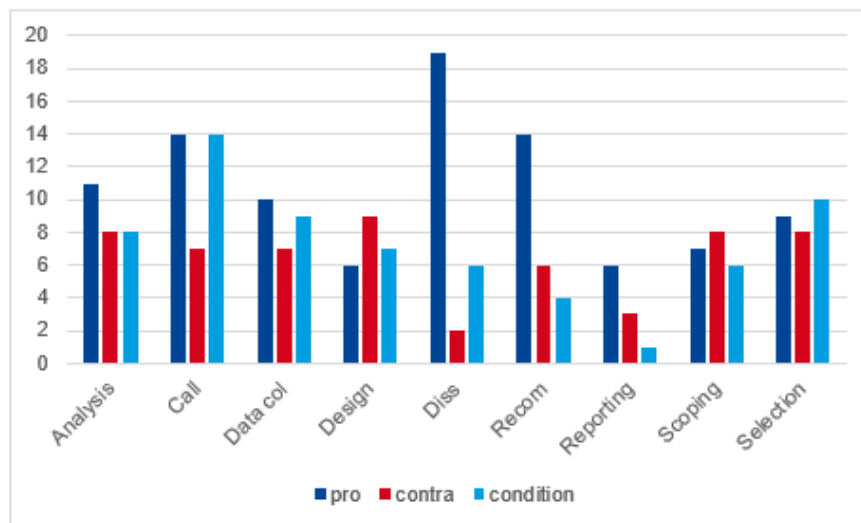
Figure 5 – Number of arguments for and against patient involvement for each research phase



When, in addition to the arguments pro and contra patient involvement, also the conditions are added to the graph, we see that most conditions apply to the call phase (Figure 6).



Figure 6 – Number of arguments for and against patient involvement and number of conditions for each research phase



13.2.2 Qualitative findings

A few interesting observations were made during the initial coding:

- The same argument is sometimes mentioned as an argument for patient involvement in research, and sometimes as an argument against patient involvement in research
- The same arguments might return in different phases of the research cycle, for example, potential conflict of interest, time consumption, lack of knowledge.
- The major arguments chosen by the different groups for the plenary discussion differed from the most frequently cited arguments.

After recoding the initial nodes with similar content and within the same research phase into larger clusters, the total number of nodes was reduced from 219 to 140 ($219 - 120 + 44 - 3 = 140$). 120 nodes have been merged and 3 nodes (referring to the observation that patients were already involved in a certain research phase at KCE and hence did not give specific arguments or formulate conditions) have been deleted. Some examples of how nodes were merged and reformulated into a new node are provided in Table 20.

Table 20 – Examples of nodes merged in the second coding round

New node merging initial nodes 1 and 2	Initial node 1	Initial node 2
Analysis_Contra_Risk of misinterpretation by lack of knowledge	Analysis_Contra_Patients are not scientists	Analysis_Contra_Risk of misinterpretation by lack of knowledge
Analysis_Contra_P might be too focussed on own situation (miss broader perspective)	Analysis_Contra_Patients have a conflict of interest	Analysis_Contra_Patients might be too focussed on own situation (miss broader perspective)
Analysis_Pro_patients can help to interpret findings	Analysis_Pro_patients can help to interpret findings	Analysis_Pro_Patients can validate the interpretation of the results
Call_Condition_If call is accessible to patients in terms of language used and submission requirements	Call_Condition_If call is easily accessible	Call_Condition_If call is understood by patients



In addition to the re-coding of initial nodes, (re-formulated re-coded) nodes that represented these same theme were clustered into overarching themes. Themes were identified across research phases, meaning that one theme could be relevant in different research phases. We identified 25 overarching themes:

1. Acceptability/Acceptance of study report
2. Adequate language use
3. Anonymity, especially of topic proposals
4. Appropriateness and relevance of patient involvement
5. Complementarity of patient involvement to other research aspects
6. Conflict of interest
7. Credibility of the research
8. Early in the research process
9. Ethics in research: different weight to different stakeholders, right to be involved, dealing with frustrations/negative impact on patients
10. Experts by experience
11. Extensiveness: involvement of patients in all projects or not
12. Feasibility
13. Impact on KCE procedures
14. Implementation of recommendations
15. Innovation in research approaches
16. Ownership of research results
17. Patient education about scientific methods
18. Patients' knowledge and competences
19. Priority setting in study proposals
20. Quality of the research
21. Representativeness of patients involved

22. Social capital
23. Subjectivity of patient input
24. Time issues
25. Visibility and image building of KCE

A complete list of all nodes classified under each of the themes is provided in Appendix 4.1.

Table 21 presents for each research phase (columns) the themes (rows) reflected upon by all staff members. A colour code was given to the cells, indicating whether the arguments were in favour of patient involvement (green), against patient involvement (red), mixed pro/con (orange), or related to (a) condition(s) for patient involvement (yellow). In cases of pros and/or cons combined with a condition, a 'c' is added to the red, green or orange cell.

The complete table with all arguments is presented in Appendix 4.2.

13.2.2.1 Concerns related to patient involvement

Some themes covered mainly arguments against patient involvement, such as appropriateness, time issues, subjectivity and potential conflicts of interest.

The expectation seems to be that patient involvement might reduce the appropriateness of the research activities performed by KCE. For example, it was brought up that the call should primarily target policy makers, not patients. This is rather peculiar, because the current processes at KCE allow every citizen, organisation or institution to submit proposals. Because of the concerns related to appropriateness, several conditions were identified for patient involvement in research, such as "patients should only be involved in a project if data have to be collected in patients", "patients should not be involved if the study relies on quantitative data analysis only", "patient involvement in the analysis phase might be appropriate but should be a free choice". There was disagreement amongst KCE people regarding the appropriateness of involving patients in the recommendations phase. For some, it was easy to involve patients in this phase, whereas others



considered this not appropriate because patients are rarely a direct target group of the recommendations.

Patient involvement is expected to have a huge impact on time needed for the research. It seems to be a major concern and reason for resistance against patient involvement at KCE, given that “patient involvement is time consuming” was mentioned for all research phases (except reporting, recommendations and dissemination).

Concerns were also raised about patients’ subjectivity, emotions and lack of ability to distinguish between their personal problems and the more macro-oriented issues that need to be addressed by KCE, thereby slowing down the research process.

With respect to conflicts of interest, it was highlighted that some patient organisations are fully funded by the pharmaceutical industry, and might therefore have a conflict of interest when they contribute to, for instance, an HTA on a particular pharmaceutical product or when they submit topic proposals. Also possible conflicts of interest due to patients’ focus on their own concerns and needs, were mentioned. Patient involvement in the formulation of recommendations was considered acceptable if patients would declare their conflicts of interest (condition).

There seemed to be some concerns amongst the KCE people about the anonymity of study proposals. For example, the use of specific patient-submission forms for topic proposals, might allow reviewers of the topics to indirectly identify the submitter. If patients would be involved in the selection of topics for research at KCE, this might bias their judgement.

Finally, the issue of representativeness of patients involved was raised for several research phases. Heterogeneity within patient populations was considered as a barrier for patient involvement in the call for proposals, the scoping of the research study and the design. However, if specific conditions with respect to representativeness could be met, the balance could move to more support for patient involvement, according to the KCE people. For example, if it can be assured that the patients involved are representative of a sufficiently large patient population, patient involvement in the selection of research topics could be beneficial. For the design phase, a possible

condition could be to always include the same group of patient representatives, or to apply the same selection procedures for patients as for subcontractors. In the analysis phase, a condition imposed upon the patients involved could be that they should have consulted at least a certain number of other patients to inform their contributions to the research process.

13.2.2.2 Advantages of patient involvement in different research phases

KCE personnel also saw several possible advantages of patient involvement for the procedures of KCE. For the call, for instance, the involvement of patients was considered to be beneficial to avoid that topics submitted relate mainly to highly prevalent conditions or exclusively scientific topics. Involving patients in the design phase might allow to include or test other methods for data collection than those the researchers would initially think of. In the dissemination phase, patients could help developing patient summaries. Several conditions were, however, also mentioned, e.g. that patients’ role and expected input is clearly specified, and researchers are still free to decide what to do with the input of patients.

Besides the potential positive impact of patient involvement on KCE procedures, the involvement of patients to incorporate the experience of experts by experience was also emphasized as a major advantage. It may help to identify the major needs of patients, evidence gaps, and relevant information on patient-related issues. Involvement of patients as experts by experience was considered beneficial for almost all research phases. The overall quality of studies is expected to improve if patients give input on the issues to address. However, it was surprisingly not mentioned as a possible advantage in the scoping phase. This might relate to the fact that the call and selection phases were discussed first, and the arguments of ‘improving the identification of gaps or needs’ and ‘giving information about patient-relevant issues’ had already been mentioned in these phases, but remain relevant in the scoping phase (once you have the information on the needs and gaps, you can use this information in the scoping phase as well). Nevertheless, we should conclude from these results that KCE people would



rather not see the advantage of involving experts by experience in the scoping phase.

The acceptability and acceptance of study results is expected to increase through patient involvement and, as a consequence, the likeliness of implementation of the study results. This in part due to increased ownership of the study results. Patient involvement might also contribute positively to the image of KCE: rather than being a group of scientists in an ivory tower, KCE is reaching out to patients (and other stakeholders) to make sure its work is relevant and realistic.

Patient involvement is also thought to lead to potential innovation at KCE, for example, KCE may learn from patient representatives about new scoring algorithms for research proposals (selection phase) or learn from the experiences of patient (organisations) with surveys or other data collection methods.



Table 21 – Matrix of 25 themes and their occurrence in each research phase

	Call	Selection	Scoping	Design	Data collection	Analysis	Reporting	Recommendation	Dissemination
Impact on KCE procedures	c	c	c		c	c			
Experts by experience						c	c		
Ownership									
Innovation									
Social capital									
Visibility and image building of KCE									
Implementation									
Patient education									
Acceptability/Acceptance of study report									
Adequate language use									c
Early in the research process		c			c	c			c
Extensiveness		c							
Complementarity						c			
Feasibility									c
Priority setting									
Quality of the research	c			c				c	
Ethics		c				c			
Credibility									
Anonymity	c								
Conflict of interest					c	c		c	



13.2.2.3 Summary matrix of main arguments

Main arguments in favour of patient involvement were chosen for the very early phases of a research project (call, scoping) and for the last phases (recommendations and dissemination or results). In the early phases, it is considered that patients can bring in important knowledge on what is important to them, what their unmet needs are and what their priorities are. In the recommendations phase, they can bring in the daily life perspective, to make sure the recommendations formulated by KCE make sense in practice. In the dissemination phase, they can help with the creation of patient fiches, if these are relevant (not for all research projects).

Main conditions for patient involvement brought forward by the different groups were spread across all phases of the research process. Conditions relate to disclosure of possible conflicts of interest, scientific knowledge and educational background of patients involved, clear procedures and processes for patient involvement (who to involve, how to involve them) and availability of resources (time and budget).


Table 22 – Main conditions for and arguments pro and contra patient involvement in different research phases brought forward by KCE personnel

	PRO	CONTRA	CONDITION
Call	<ul style="list-style-type: none"> Patients know what is important (making research patient relevant) Patients can highlight their unmet needs Patients have another image / point of view than researchers 	<ul style="list-style-type: none"> Patients cannot always make the distinction between individual problems and societal problems that are solved by a research project 	
Selection		<ul style="list-style-type: none"> Risk of bias: patients might want to favor their own interests / subjects 	<ul style="list-style-type: none"> Conditional upon the guarantee that the patient is not “piloted” by private interests (industry)
Scoping	<ul style="list-style-type: none"> Patients have the experience and can bring in arguments and information that differ from those of the medical doctors Patient involvement can help to identify the priorities of patient needs 		<ul style="list-style-type: none"> You need a representative that knows the jargon, and that masters the topic
Design		<ul style="list-style-type: none"> Not for the methodology, this is for experts Patients do not have sufficient methodological knowledge, should be done by KCE experts 	
Data collection		<ul style="list-style-type: none"> Time consuming 	<ul style="list-style-type: none"> Screening of patients' conflict of interest KCE has to study how to involve patients / which methods to use for patient involvement in which situation
Analysis		<ul style="list-style-type: none"> Not feasible to involve patients in analysis, because this is time consuming Independence of patients (organisations) – conflict of interest 	
Reporting			
Recommendations	<ul style="list-style-type: none"> Patients can add elements of feasibility, applicability in daily life, what is feasible, the practical side of things, connection with real life 		<ul style="list-style-type: none"> Define clearly who to involve and how to involve them (patient associations) Beware of conflicts of interest



	<ul style="list-style-type: none">Recommendations will always affect the patient, even indirectly	
Dissemination	<ul style="list-style-type: none">Creation of patient fiches (by KCE)	<ul style="list-style-type: none">Only if patients have been involved beforeMake choices: if KCE is to communicate to patients itself, then KCE should create the conditions for doing this (time, resources...)

13.3 Conclusion with respect to the actual patient involvement culture at KCE

In general, we should conclude that although KCE people are principally in favour of patient involvement their research and see some advantages of patient involvement, there is also some resistance. Several employees, in particular those with experience with patient involvement in their research, tend to emphasize the advantages, whereas the less experienced staff members mainly have concerns.

The major advantages that are shared by the majority of employees encompass the focus of studies on patient needs by bringing in experience and knowledge from people with experience. Concerns relate to the impact patient involvement might have on the duration of studies, the time investment, the subjectivity and credibility of their work and the added value of patient involvement. Also potential conflicts of interest of patients is an issue for several people. However, it seems that KCE staff is open to patient involvement if good procedures and processes are developed for it, and resources are made available.



14 THE CURRENT PLACE OF PATIENT INVOLVEMENT IN KCE PROJECTS

In the previous chapter, we tried to capture the patient involvement culture at KCE by consulting the employees of KCE. This could be considered as an exploration of the implicit place of patient involvement in KCE's work.

In addition, the position of KCE as an organisation vis à vis patient involvement is also partly reflected in its past work, strategic decisions and policy. The patient, as the end-beneficiary of healthcare, is and always has been at the centre of all KCE concerns. Besides the fact that all KCE reports aim to make recommendations for a better healthcare system, the place of the patient and the patient involvement in the research of KCE has been developed in its reports throughout the years.

14.1 Patient involvement in defining health policy

In 2013, we examined through a qualitative study which models for citizen- and patient involvement in health policy were considered acceptable and feasible for different stakeholders in health policy (report 195).³ The study showed that stakeholders considered citizen and patient involvement to be important. They would see them mainly in a consultative role. Citizens would be consulted for more strategic decisions, e.g. about healthcare priorities, whereas patients would be consulted more in the context of operational decisions, e.g. specific drug reimbursement decisions.³

In report 234, we defined, based on a scientific process with expert consultation, the relevant criteria for the appraisal of therapeutic and societal need.²⁴¹ We also measured the relative importance according to the Belgian general public of these criteria for the appraisal of therapeutic and societal need as well as for the appraisal of added therapeutic value. We performed a large population survey using discrete choice experiments. The general public was hence consulted about the relative importance of decision criteria, as recommended in report 195, and thereby increases the accountability for reasonableness of decision makers who decide on behalf of the public.

The identified criteria and their weights are two essential components of the multi-criteria decision analysis (MCDA) for the appraisal of medical needs that was applied in a pilot study aiming at appraising and ranking therapeutic and societal needs in healthcare (report 272).²⁴²

Another example of research oriented towards patient involvement in health care policy is the report on patient-reported outcome measures (PROMs) and patient-reported experience measures (PREMs).²⁴³ Measuring health outcomes and experiences as seen by patients, aims to improve the quality of care, be it at the national level, the institutional level or the individual patient level, and places patients' needs at the centre. It has now been proven that collecting PROMs and PREMs have a positive effect on the doctor-patient relationship... at least if a culture of patient-oriented care is already in place. KCE has formulated recommendations to implement PROMs and PREMs in Belgium.

Finally since 2015, patient representatives of the 3 umbrella organisations of patient associations in Belgium (Flemish, French-speaking and German-speaking) are members of the **KCE Board of directors**, besides representatives of public bodies active in healthcare, and health insurance. At each Board meeting new KCE reports are presented and their political recommendations, based upon the scientific work, are discussed, sometimes nuanced, and approved by a simple majority, when voting turns out to be necessary.



14.2 Patient involvement in the development of a tool for shared decision making

KCE has developed expertise with shared decision-making processes. In this process patient and physicians are invited to talk about the pros and cons of an examination, a test, a screening process or a treatment. Both users have to be satisfied with the tool because they will use it together, for shared decision making. As end-users, patients were involved in the development of the tool, in order for the tool to respond to their expectations, both in terms of information content and in terms of the form (media, visual, etc.).

14.3 Patient involvement in the different domains of KCE research

KCE research covers several domains where patient involvement theoretically makes sense.

Health Services Research (HSR) focuses on the organisation and financing of health care in the largest sense. Studies are often complex and based on an extensive analysis of the scientific literature and of a variety of examples from different countries. The various stakeholders, including patients, are to be involved in the main steps of the project because they are affected by decisions based on HSR as users of the health care system.

KCE also develops **Guidelines for Good Clinical Practice (GCP)**, i.e. tools designed for the daily practice of health professionals. The guidelines are developed by a multidisciplinary group: the Guideline Development Group (GDG). When developing an oncology guideline, the GDG generally includes patient representatives. Members of the GDG define the clinical question, identify critical and important outcomes, give feedback on the content of the guideline, judge indirectness of evidence, and give feedback on the draft recommendations. In the last phase of the guideline development, patient representatives are invited to review the draft recommendations.

The Guideline development for primary care professionals is part of the Evidence Based Practice (EBP) Network, developed by KCE. The principal task of this network is to identify, develop or refine all processes, procedures, responsibilities and roles of all partners and structures in the EBP Network, in terms of prioritisation, development, validation, dissemination, and implementation of guidelines or EBP products, as well as evaluation, policymaking, management and feedback of the EBP Network. Existing EBP partners, representatives from government and administration, health care professionals as well as patients are involved in the whole process.

Health Technology Assessments (HTA) encompasses the analysis of the safety, effectiveness, cost-effectiveness, organizational impact, ethical impact and patient issues of a health technology (a drug, a vaccine, medical equipment, a medical device and new treatment path ...). The assessment of the impact of a health technology needs to take the benefits (relevant) for the patient into account, e.g. the impact on health-related quality of life, the convenience of the treatment. Possible other patient issues are also part of the assessment. Patient involvement might help to identify relevant outcomes or ways to address or examine patient issues related to a health technology.

“Trials” is the newest activity domain of KCE (since 2015). The KCE trials programme is a publicly funded pragmatic clinical trials programme that emerged from the observation that many questions in healthcare are currently not or insufficiently studied in clinical trials, despite their high societal importance. It was concluded that public funding of pragmatic clinical trials, subject to conditions, would be beneficial to stimulate research with respect to such questions.²⁴⁴ The KCE selects and funds the trials. Generally, academic hospitals or other research organizations that can act as a non-commercial sponsor under the Belgian law, will organize and conduct the trials.

An absolute requirement for funding is that the results of the trial must have an immediate impact on patient care or on the efficient use of healthcare resources. Patient involvement in the development of the trial design is one of the assessment criteria of the study proposals submitted for funding.



14.4 Methodological reports useful to involve patients

KCE has published **methodological manuals** with validated work methods for all researchers in the field of healthcare and public health. A first manual describes how to involve stakeholders in a broad sense.²⁴⁵

In the KCE process book that compiles all KCE research processes,²⁴⁶ qualitative research methods are also described. Qualitative research methods are particularly suitable for the collection of patient's perspectives and experiences.

Finally, we plan to develop a methodological report on how to identify patients' needs: knowing what patients really need is important for guiding research and development efforts towards those areas that matter most to patients, and for the appraisal of the added value of new healthcare interventions. It contributes to the creation of a needs-driven healthcare system. However, the identification of patient needs has hurdles and constraints. These will be addressed in the study and solutions will be sought in order to develop a feasible method for identifying patient needs.

14.5 Involvement of patients in recent KCE research

In order to learn from KCE experiences with involving patients in KCE projects, we carried out an internal web-survey on patient involvement among all KCE experts.

14.5.1 Methods

End of December 2018, we sent an email inviting every KCE expert to fill out a questionnaire in LimeSurvey (see Appendix 5).

We used the following definition of patient involvement: *"Patient involvement in research encompasses two distinct but complementary ways to strengthen research by taking patients' perspectives into account: Involving patients to research patient aspects that are relevant for the study (patients' experiences, preferences, perspectives) Involving patients in the research process (scoping, design, data-gathering, analysis, reporting, diffusion and implementation) We want to focus in this survey on active patient*

involvement for one of these purposes. Participation of patients in studies as 'units of investigation', e.g. as in a trial, is not considered. Whenever we talk about patient involvement, we mean consultation, active participation in discussions, and co-decision making. Just informing patients is not enough to be considered as involving patients."

We asked the experts to judge, based on this definition, whether they had involved patients in the projects they worked on in the last 5 years.

After basic description of their expertise at KCE, questions were targeting:

- The rationale of patient involvement in the different study domains (HSR, HTA, GCP, Methods or KCE trials)
- The 'type' of patients involved
- The way to involve patients
- The project phase in which patients were involved
- How many patients were involved
- Selection and recruitment of patients
- Positive experiences with patient involvement
- Negative experiences with patient involvement
- Solutions to remedy negative aspects

These topics were also tackled for ongoing projects.

The four last topics focused overall on practical aspects that will nourish the KCE procedures.

Additional questions were dedicated to discontinued attempts to involve patients. The extensive questionnaire is available upon request.



14.5.2 Results

Twenty-seven KCE experts participated in the survey, mainly experts working for more than 5 years at KCE. Due to the size of the observed sample, and according to the aim of the survey, results have to be considered as qualitative information.

14.5.2.1 Rationale of patient involvement in the different study domains

Patients were mainly involved in HSR and GCP projects, while no HTAs or methods projects have involved patients in the last 5 years. The KCE trials programme foresees systematic patient involvement in the future.

In the HSR projects identified in our survey, patients were involved to:

- Collect data about:
 - information or content related to the research question
 - experience with a disease, a treatment or the healthcare system
 - perception on the topic
 - preferences and values
- Validate research aspects from the patient's perspective:
 - the scope of the project, to check that the patient point of view is not missed
 - the results of the project
 - the recommendations

For GCP, patient involvement adds another perspective, and allows to take into account patients' preferences and values in elaborating recommendations. This is crucial for guidelines and quality indicators.

In KCE trials, patients are involved in order to make the trials more relevant for patients e.g. regarding the choice of the outcome parameters.

14.5.2.2 Types of patients involved

A central question is the type of patients to involve in the research, i.e. who represents the patient? The results by type of research are presented in Table 23.

Table 23 – Types of patients involved in the research

	HSR	GCP	Trials
Individual patient(s) with the disease/condition	X	X	X
Patient association(s)	X	X	X
Patient umbrella organization(s)	X	X	X
Other	Informal caregiver, family of patients with the disease		X

KCE involves individual patients, generally less than 25, as well as patients' representatives from specific patient associations (1 to 3 associations) or the umbrella organizations. In the latter case, both the LUSS and the VPP are contacted. In some cases, where patients themselves cannot be involved, the patient's informal caregivers or family members are involved as proxies.

14.5.2.3 Selection and recruitment of patients

Recruitment strategies vary. Patients are recruited by phone, e-mail, social media, specialized media... Collaboration with the stakeholders is essential. For example, healthcare practitioners' are useful and sometimes essential resources to:

- Identify patient associations
- Help in the recruitment of individual patients



It seems that, in KCE research, there is generally no need to select candidates to represent patients due to the limited numbers of potential participants. They are mainly volunteers. It could be questioned to what extent they represent the 'usual patients'.

14.5.2.4 Project phases

The involvement of the patients differs according to the research domain, the project phase and the type of patients, as shown in Table 24.

Table 24 – Project phases with patient involvement

	Individual patient(s)	Patient association(s)	Patient umbrella organization(s)	Other
Scoping	HSR	HSR, GCP	HSR, GCP	
Design				Trials
Outcome definition	HSR			HSR, Trials
Data collection	HSR, GCP	HSR	HSR	HSR
Conclusions	HSR, GCP	HSR, GCP	HSR	
Recommendations	HSR, GCP	HSR, GCP	HSR, GCP	

There is no clear pattern of involvement in each research domain, except that KCE trials in the only domain where it is systematically required that individual patients are involved in the design of the research. More precisely, patients' representatives are involved into the evaluation process of the submissions.

14.5.2.5 Patient involvement methods

In general, all classical methods used to collect data were also used to involve the patients in KCE research (Table 25).

Table 25 – How are patients / (umbrella) organizations involved in the projects

	HSR	GCP	Trials
During meetings (face-to-face, skype, phone...)	X	X	X
(Online) surveys	X	X	
Individual interviews	X	X	
Focus groups	X	X	
Delphi panel	X		
Nominal Groups			
(Online) forum	X		
Other	Workshops	GDG meetings	Patient representatives are member of the Trials panel and Trials Board and prioritization group

HSR projects use a larger variety of more methods than projects in other domains. Specific methods of inclusion of patients in specific 'tools' of the research domains are reported: the GDG, for example, is a formal meeting group where patients are included.



The rhythm of the involvement differs across the research domains and the type of patients involved (Table 26). Continuous collaboration is more sustained for KCE Trials than for the other projects.

Table 26 – Rhythm of patient involvement

	Once during the entire project	More than once	Continuous collaboration
Individual patient(s)	HSR, GCP	HSR	Trials
Patient association(s)	GCP	HSR, GCP	Trials, GCP
Patient umbrella organization(s)	HSR	GCP	Trials
Other		HSR	Trials

In the EBP Network, patients are involved in different phases: they give input in the prioritisation process, they participate in the stakeholders meeting for the development of guidelines or EBP products, and they are members of Board of Directors of the EBP Network Foundation and members of the Advisory Board. Last but not least, they are the end-beneficiaries of EBP Network content.

In KCE trials, patients are included in the trials panel and the trials board. These advisory groups are composed of independent individuals with the broad spectrum of knowledge, skills and experience needed to get a well-rounded view of research needs and research assessment. . In general terms they help to ensure that research is high quality, scientifically robust, represents good value for money and meets the needs of patients, the Belgian health system and the wider public.

Trials Board members read Expressions of Interest and Full Research proposals and critically assess the documents. They may also review draft trial reports. KCE Trials panel assists KCE Trials in selecting the research questions that are most relevant for routine clinical practice and the Belgian healthcare system.

14.5.2.6 Perceptions of KCE researchers on patient involvement in the projects

We found no clear differences in the perceptions of KCE experts with respect to patient involvement according to the research domains. KCE researchers reported mainly to be satisfied with the content of the information and discussions they had with patients or patient associations. Patients or patient representatives were felt to be really willing to participate in the project and share their personal experiences. The patients involved were found to be really active in their participation.

Some difficulties were however pointed out:

- In the recruitment:
 - Depend on the goodwill of the practitioners
 - How to avoid selection bias and, in case of patients associations, conflict of interest?
- In the involvement with experts:
 - Language was an issue: meetings in KCE are held in English, or each participant use his/her own mother tongue (French or Dutch). Patients are not always able to understand other languages than their mother tongue.
 - The number of patients in a meeting is always smaller than the number of other experts and/or other stakeholders. This could generate a disbalance between the different points of view and the patient's could feel somewhat isolated.
- In the active involvement: it is sometimes less easy to actively involve patient representatives during meetings, sometimes they even do not come or give any comment.
- In the interest to participate: umbrella organisations are not always interested in participating in projects, or they do not have the capacity.
- In the openness of the patients to listen to and and participate in discussions about aspects that differ from their own experiences.



- In the researcher's skills: researchers are not completely ready to cope with the expression of the emotions and pain of the patients they meet.
- Ethical issues: involving patients requires the approval from an ethical committee. This is a long process.
- Resource requirements: patient involvement is time consuming, particularly if they are expected to be involved in the data collection phase (need of ethical committee approval). Also recruitment is sometimes difficult and also takes additional time.

Some solutions have already been proposed or implemented to overcome the difficulties:

- increasing time for the project
- multiplying the sources for recruitment
- involving caregivers or patient associations in recruitment
- preparing the GDG meetings via web-survey, avoiding patients (representatives) to be influenced by the physicians. This approach enhanced an anonymous voting system, which reduces the potential feeling of patients of being blamed for not being experts in medical matters.

14.5.3 Conclusions

While patient involvement has a place in all KCE research domains, no recent HTA study has involved patients. For HSR and HTA projects, there is no clear process to decide why, when and how to involve patients. It is 'tailored', depending on the project and the team. Creativity is often required because of the complexity of the topics, especially in case of HSR projects. Various methods and techniques for patient involvement have been applied in this kind of projects.

The question of who is the best representative of the patient is not solved in KCE research. It depends on the research question, the way to involve patients (method, frequency...) and practical issues. It has been suggested

that patient involvement could be facilitated by involving caregivers in the identification and recruitment of patients to be involved.

In general, solutions to improve patient involvement in KCE projects were found to overcome the barriers, but they are found to be time and resource consuming.



15 FORMULATION OF POSITION STATEMENTS

15.1 General approach

For the formulation of the position statements of KCE with respect to patient involvement in its research, a two-step approach was taken. **First**, the research team (authors of this report) formulated a set of draft position statements based on the research activities the team performed and described in this report, i.e. literature review, description of international initiatives or patient involvement at organizations similar to KCE, interviews with Belgian organizations or groups with experience in patient involvement, description of culture at KCE and activities to date.

Then, these draft statements were presented to the entire staff of KCE, in order to assess the level of support for these position statements. Based on this second activity, statements were withdrawn or adaptations were made to the statements to accommodate for the concerns of the KCE employees, in order to reach a maximum support from KCE personnel for the final published statements. This was considered of utmost importance, as the statements will have a direct impact on people's work.

15.2 First draft statements

Based on the different research activities described above, 10 draft position statements on patient involvement in KCE research were formulated:

1. KCE perceives the fundamental ethical, as well as the instrumental and procedural rationales for patient involvement decisive enough to take a positive position towards patient involvement in health policy research. Patients have the democratic right to be involved in research about

them, and they can contribute a unique perspective to the research from their personal experience, competences and knowledge.

2. Patients should not necessarily be involved in all policy research projects. The relevance and need for patient involvement in research projects should be assessed project by project.
3. Patient involvement in health policy research is complementary to the review of scientific evidence and primary data collection, not a substitute for it.
4. Sufficient resources (human, financial, time) should be made available to ensure and support effective patient involvement in health policy research. A central point of contact should support both patients and researchers and coordinate all patient involvement activities at KCE.
5. The planning of the projects has to be adapted to implement patient involvement in an optimal way.
6. Training should be organised for researchers and patients/patient organisations to effectively involve patients or be involved in health policy research.
7. Patient involvement activities in health policy research should be regularly evaluated and procedures revised when appropriate.
8. Patient contributions and potential impact should be reported in the research report.
9. Patient representatives who have been involved should receive feedback from KCE and provide feedback to KCE to potentially improve future collaboration.
10. It is important to make appropriate and informed choices about who to involve, in what role, in which phase of the research project and for what purpose (see Table 27)



Table 27 – First draft statements about who to involve, when, for what and at what level

	Individual patients	Patient organisations	Umbrella organisations	Sickness funds
Call*	Yes, accessible template required	Yes	Yes	Yes
Selection*			Yes	Yes
Scoping	Describe the context Define elements that need to be addressed in the research to support researchers in the formulation of the research questions	Describe the context		
Design	Method selection, by patient expert*	Selection of outcomes to consider in the research	Method selection	Method selection
Data collection		Recruitment Instrument selection and testing	Recruitment	Recruitment
Analysis	Minimal important difference in patient-relevant outcomes Interpretation of results, by patient expert			
Reporting		Synthesis review	Synthesis review	Synthesis review
Recommendations			Verification of the appropriateness of formulation	Verification of the appropriateness of formulation
Dissemination	Defining dissemination strategies Actively disseminate	Defining dissemination strategies Actively disseminate	Defining dissemination strategies Actively disseminate	Defining dissemination strategies Actively disseminate

* as is – no changes recommended compared to current KCE procedures

Consultation

Co-production

User-led decision

* A patient expert is a patient with knowledge about scientific approaches (trained or acquired through frequent involvement in research projects)



15.3 Assessment of the support for the statements by the KCE members

The draft statements were presented to KCE employees. The tenth statement referred to a table specifying who to involve, when in the research process, for what purpose, and hence actually encompassed several specific statements. We drafted separate statements for every type of involvement included in the table. This led to 11 additional statements.

15.3.1 Method

A Delphi method was used to assess the support for the position statements within KCE.

Nineteen of the 21 statements were explained and submitted to consensus. Two statements were not submitted because they are already part of the KCE work processes (involvement of all types of patients in the call for topic proposals and involvement of patient umbrella organizations and sickness funds in the formulation of the recommendations^{kkkkk}). Some statements were made more concise for presentation purposes. The complete list of statements presented, with the exact formulation is shown in Table 28.

The 19 statements were presented during a meeting to which all KCE members were invited. Forty-eight out of 71 invited employees participated in the meeting, i.e. 66% of the staff (including researchers, management, supporting and administrative staff).

An electronic voting system called Turningpoint^{lllll} was used, showing directly the results of the votes on a PowerPoint® presentation during the meeting.

^{kkkkk} With the three umbrella organisations of patient associations being member of the Board of KCE, the patients actually already get the opportunity to review the recommendations now.

1st voting round:

After a short introduction about the purpose of the exercise, each statement was voted upon by the participants. Participants were given four response options:

- "agree" if the respondent would agree to put the statement in its current form in the position paper.
- "almost agree" if the respondent agrees with the principle, but would suggest to reformulate the statement somewhat.
- "disagree somewhat" if the respondent would rather not put the statement in the position paper but does not particularly feel strongly about it.
- "strongly disagree" if the respondent has strong feelings against the statement.

People who have no opinion on a particular statement were asked not to vote. The votes were anonymous.

We defined consensus for acceptance as "75% or more of the respondents voted "agree or almost agree" AND less than 10% voted "strongly disagree"". If consensus was reached, the statement was adopted. Nevertheless everyone had the opportunity to comment or make suggestions for all statements in a later stage via an online questionnaire (see further).

If no consensus was reached, or there was a consensus on disagreement (> 75% votes "disagree or disagree somewhat" AND < 10% votes "agree"), the statement was submitted to a 2nd voting round *after* all statements had been voted upon and a plenary discussion on that statement had taken place.

^{lllll} <https://www.turningtechnologies.com/turningpoint/>

**2nd voting round:**

The statements that did not reach consensus at the first round were discussed. After the discussion, participants were invited to re-vote using the same response categories. The statement was not modified, even if suggestions were made for reformulation. The objective of the discussion was to learn from each other why some were for against a specific statement.

If consensus was reached, the statement was adopted. If still no consensus was reached after the second voting round, statements were rewritten according to the suggestions and comments made during the discussion and the online questionnaire distributed after the meeting.

The general management was asked to decide on the acceptability of the reformulated final position statements that did not reach consensus. If the management agreed with the statement, the statement was adopted, if not, the statement was rejected.

15.3.2 Results

After the 1st round, 14 out of 19 statements reached consensus for agreement and one for rejection (Table 28). The detailed results for the different response categories per statement are presented in Appendix 6.1. Discussions about statements that did not reach consensus were limited to about 15 minutes for each to stay within the time slot foreseen for the entire Delphi exercise.

Table 28 – Draft position statements presented to KCE employees for voting, results 1st and 2nd round of voting

Position statement	Voting round 1	Voting round 2
1 KCE perceives the fundamental ethical, as well as the instrumental and procedural rationales for patient involvement decisive enough to take a positive position towards patient involvement in health policy research.	Consensus agreement	/
2 The relevance and need for patient involvement in research projects should be assessed project by project.	Consensus agreement	/
3 Patient involvement in health policy research is complementary to the review of scientific evidence and primary data collection, not a substitute for it.	Consensus agreement	/
4 Sufficient resources (people, time, and budget) should be made available to ensure and support effective patient involvement in health policy research.	Consensus agreement	/
5 The planning of the projects has to be adapted to implement patient involvement on an optimal way.	Consensus agreement	/
6 Training should be organised for researchers and patients/patient organizations to effectively involve patients or be involved in health policy research.	Consensus agreement	/
7 Patient involvement activities in health policy research should be regularly evaluated and procedures revised when appropriate.	Consensus agreement	/
8 Patient contributions and potential impact should be reported in the study report.	Consensus agreement	/
9 Patient representatives who have been involved should receive feedback from KCE and provide feedback to KCE to potentially improve future collaboration.	Consensus agreement	/



10	Individual patients and/or patient organisations should be <u>consulted</u> in the scoping of the KCE projects to allow researchers to better describe the context.	Consensus agreement	/
11	Individual patients should contribute to the scoping by <u>co-producing</u> the elements that need to be addressed in the research project.	Dissensus	Dissensus
12	Individual patient 'experts' and/or patient umbrella organisations should be <u>consulted</u> on the selection of <u>methods</u> for the projects.	Consensus disagreement	/
13	Patient organisations should be <u>consulted</u> in the selection of the <u>outcomes</u> to be included in the study.	Consensus agreement	/
14	Patient organisations and/or patient umbrella organisations and/or sickness funds could <u>co-decide</u> on the approaches for recruitment of participants if primary data collection in patients or users is needed.	Consensus agreement	/
15	Patient organisations should be <u>consulted</u> to select and test the data collection instrument(s).	Dissensus	Dissensus
16	Individual patients and/or patient organisations should be <u>consulted</u> to define the minimal important difference in patient outcomes.	Consensus agreement	/
17	Individual 'expert' patients should be <u>consulted</u> to interpret results of analyses.	Dissensus	Dissensus
18	Patient organisations and/or patient umbrella organisations should be given the opportunity to review the KCE synthesis and give feedback before publication (=consultation).	Dissensus	Dissensus
19	Individual patients and/or patient organisations and/or patient umbrella organisations and/or sickness funds should <u>collaborate</u> on the dissemination of the results of the KCE project.	Consensus agreement	/

After the discussion of the 4 statements for which there was dissensus in the first round, dissensus remained in the second round (Table 28). Detailed results can be found in Appendix 6.2.

The arguments given by participants during the discussion on the four statements at the meeting are summarized in Table 29.

**Table 29 – Summary of discussion points raised after the first voting round**

Statement	Discussion points
Individual patients should contribute to the scoping by <u>co-producing</u> the elements that need to be addressed in the research project.	Why individual patients and not patient organizations? Co-producing is too much, certainly if this would apply to all projects; maybe 'consultation' is a better phrasing. Beware of conflict of interest
Patient organisations should be <u>consulted</u> to select and test the data collection instrument(s).	Why patient organizations and not individual patients? Statement contains two elements 'select and test'; better to split this in 2 statements. It will require much time to justify all choices made during a research process.
Individual 'expert' patients should be <u>consulted</u> to interpret results of analyses.	'Should' is too strong, better 'could' Counter-argument: 'could' is too open-ended and not binding enough. We should be firm in a position statement. KCE-management commits themselves to find the (financial and human) resources necessary to realize patient involvement in KCE-research. The term "expert patients" could also be understood as individual patients with experience (expert by experience). This is not what is meant here. Here the patient expert is a patient with a scientific background or educated in scientific approaches.
Patient organisations and/or patient umbrella organisations should be given the opportunity to review the KCE synthesis and give feedback before publication (=consultation).	What is exactly is meant by 'before publication'; is this before presenting to the board of KCE? 'Should be given the opportunity' is too soft; why not 'should review'. Must this be done in all cases? Or only if patients were involved in preceding steps? This will lengthen the procedures. Expectations from patients and expectations from researchers should be clearly discussed at the beginning of a project.

Additional comments were raised in the open questionnaire about *all* statements. Forty people responded to the survey. Their comments are summarized in Table 30.

**Table 30 – Summary of comments provided via web-survey on the full set of original position statements**

Statement	Summary of comments
KCE perceives the fundamental ethical, as well as the instrumental and procedural rationales for patient involvement decisive enough to take a positive position towards patient involvement in health policy research.	General support for this statement. Someone refers to 'a general trend around the world'. Someone else would even suggest a stronger wording, like "taking a leading position".
The relevance and need for patient involvement in research projects should be assessed project by project.	Agreement that for each project automatically the reflection of the relevance and need for patient involvement should be made. At the same time it should be considered in which phase of the project patient involvement would be relevant and needed. As a caveat, it was mentioned that there might be a risk of arbitrariness: some researchers will more easily consider patient involvement less relevant or needed than others. Therefore, it needs to be clarified which aspects need to be considered in the relevance/need judgment to keep the process transparent and consistent.
Patient involvement in health policy research is complementary to the review of scientific evidence and primary data collection, not a substitute for it.	No comments received.
Sufficient resources (people, time, budget) should be made available to ensure and support effective patient involvement in health policy research.	Agreement about the statement but some comments regarding the proportionality of the resources used for patient involvement. Resources for patient involvement should remain proportional to the overall resources that are available to KCE to fulfil its mandate. Making more resources available for patient involvement will either result in less resources for other parts of the project (e.g. data analysis), or fewer projects per year. The added value of patient involvement should be carefully evaluated, not per se, but in relation to its "opportunity costs" in terms of, for instance, lower quality of other parts of the project due to less resources for these parts, or increased duration of the projects. Nevertheless, someone mentioned that patient involvement does not necessarily require more resources, at least not when compared to the cost of other types of stakeholder involvement. One respondent also emphasised the importance of providing the right resources for supporting patient involvement activities, e.g. experts with knowledge on how to involve patients.
The planning of the projects has to be adapted to implement patient involvement on an optimal way.	Agreement about the statement. Comments were in line with the ones related to resources: the impact of patient involvement on the project planning depends on the level of involvement; additional time investment should be balanced against other uses of this time and ability of KCE to respond timely to policy questions.
Training should be organised for researchers and patients/patient organizations to effectively involve patients or be involved in health policy research.	Many practical concerns: who is going to organise these trainings, is this the role of KCE, time consuming, lack of experience with this kind of training, etc. Some respondents disagree that patients need to be trained. Others question whether patients are willing to be trained. A suggestion was made to discuss the need for training on an ad hoc basis.



	Whether or not patients or researchers need to be trained will depend on the research, topic, and methods.
Patient involvement activities in health policy research should be regularly evaluated and procedures revised when appropriate.	General agreement but some request to implement patient involvement gradually. The need for the development of a structure and procedures for patient involvement which are regularly reviewed based on experiences, is mentioned.
Patient contributions and potential impact should be reported in the study report.	<p>Some doubts are expressed about the feasibility of assessing and reporting the impact (except for GCP projects, where this is done already). Usually patients contribute by discussing with others. It is not always possible from there on to isolate the impact of patients' contributions from that of other people's contributions or the research group in general. The suggestion has been made to report the contributions of patients in the same way as other stakeholders' contributions.</p> <p>Someone fears that this could lead to eternal discussions with patients if they do not agree with the decisions made or feedback given. It may imply a lot of additional work for the researchers to justify every research decision made.</p>
Patient representatives who have been involved should receive feedback from KCE and provide feedback to KCE to potentially improve future collaboration.	<p>Questions were asked for clarification: feedback about what? About the collaboration (communication, modes of collaboration...), about the content of the patients' contributions or about the choices made during the research project?</p> <p>Several respondents raised concerns about treating patients differently from other stakeholders in this respect (this comment was repeated at several statements). KCE does not give or ask for feedback from other stakeholders. Some respondents use this as an argument to say that we should hence not do this for patients either, while others use this to argue that we should also improve this for other stakeholders. One respondents even argues that we should go further for patients, and explain to them how their contributions served the project.</p> <p>Two respondents suggested to do this informally to keep it feasible, rather than via formal written procedures.</p>
Individual patients and/or patient organisations should be consulted in the scoping of the KCE projects to allow researchers to better describe the context.	<p>In general, people had issues with the distinction between the different types of patient representatives (e.g. individual patients, patient organisations, umbrella organisations...) in this and the next nine statements (comment not repeated).</p> <p>Another general comment applying to this and the next 9 statements is that it should be made clear that these only apply <i>if</i> patient involvement is considered relevant and necessary in this research phase (comment not repeated in the next statements).</p> <p>One respondent commented that it is OK to involve patients to describe the context, but it may become complicated to involve patients in the real scoping phase of the project, where decisions are made on the in- or exclusion of some patient categories. We might run into troubles if we exclude some patients represented by the organisations or if we include patients for which there is not representation at the scoping meeting.</p>



Individual patients should contribute to the scoping by co-producing the elements that need to be addressed in the research project.	<p>Several respondents commented that they would agree with this statement if patients would not be 'co-producing' the elements to be studied but would be 'consulted' to identify these elements. Related to this, one respondent explained that this is due to KCE not having a culture of cooperation (yet). Some experts feel that their expertise should prevail. Indeed, 21 comments state that 'the researchers should take the final decision' and 'consultation is acceptable, but not co-production'. It might be difficult to change this mind-set. In any case, this process will take time.</p> <p>A suggestion was made to implement this gradually. In short term, patients could be "consulted" while in the long term, patients could be involved as research partners. This requires training and experience: patients need to feel confident enough to be involved and KCE experts need to build up experience with the benefits of patient involvement for the quality of their research. Implementing this too fast might jeopardise the approach.</p> <p>One respondents emphasised that patients should be well informed about the general scope of KCE work, which is always related to health(care). This "health care filter" should be strictly applied during the scoping phase.</p>
Individual patient 'experts' and/or patient umbrella organisations should be consulted on the selection of methods for the projects.	<p>Similar to the previous statement, some resistance against involving patients in the methods selection was found in the comments, because it is felt that the researchers have sufficient expertise to make the methodological choices. Lack of scientific knowledge on the part of the patients is mentioned several times.</p> <p>It was suggested by a few people not to ask patients which method they consider appropriate but rather explain the methodological choices made and the reasons for these choices in the context of the project. Patients could then help to identify gaps in the proposed methods, highlight potential risks, help to fasten the process, etc.</p> <p>It was also felt that patients should only be consulted for the methods relating to the collection of patients' perceptions, opinions, experiences... and not for methodological choices in other domains.</p>
Patient organisations should be consulted in the selection of the outcomes to be included in the study.	<p>Similar to the previous statement, a suggestion was made for this statement that researchers should pre-select the (scientifically sound and relevant) outcomes and then ask patients' opinions about this selection of outcomes. According to two respondents, the input from patients in the selection of outcomes should be limited to the selection of patient-reported or patient-relevant outcomes, not all outcomes.</p>
Patient organisations and/or patient umbrella organisations and/or sickness funds could co-decide on the approaches for recruitment of participants if primary data collection in patients or users is needed.	<p>Several respondents suggest to replace 'co-decide' by 'be consulted', with the final decision to be made by the researchers.</p>
Patient organisations should be consulted to select and test the data collection instrument(s).	<p>Many concerns were raised about this statement. First, it needs to be clarified that it concerns instruments for collecting patient-relevant data (patient perspectives, preferences, impact on quality of life etc). Second, selection and testing of the instruments should not be done by the same patient representative. Expert patients could be involved in the selection, patient organisations in the testing.</p>



	<p>Third, researchers should make a pre-selection of scientifically valid instruments before consulting patients about the selection. Fourth, testing might be redundant if the instruments have already been extensively tested and evaluated in literature.</p> <p>Three respondents do not see the value in consulting patients for the selection of the instruments if patients have already been involved in the design phase (choice of methods). They would prefer not to involve patients in the selection, although involvement might be useful for the testing of the instruments.</p> <p>Several respondents support the statement, and highlight the value of testing the instruments with patients from their personal experience.</p>
Individual patients and/or patient organisations should be consulted to define the minimal important difference in patient outcomes	<p>Comments mentioned that this is not always relevant or applicable. A few respondents asked for clarification of this statement. Moreover, two respondents consider this to be part of the design phase and not the analysis phase.</p> <p>Some respondents consider that patients should only be consulted about the minimal important difference in patient outcomes when researchers are not sure of their interpretations or when researchers feel that some complementary inputs are necessary to understand some results. Notes of cautions were formulated when consulting only one or two patients about the minimal important difference in outcomes. Research designs should not be changed based on the opinion of a few patients only. One respondent stated in this context that “scientific data supersede patients opinions”. Others considered that the wording might even be stronger.</p>
Individual ‘expert’ patients should be consulted to interpret results of analyses.	<p>Mixed opinions and diverging comments on this statement.</p> <p>Several respondents prefer to see it clarified in the statement that it only concerns cases where the results are unexpected or strange. Then, patients can possibly, but not necessarily, help to interpret the results. It was noted by one respondent that this usually already happens during the stakeholder meetings, to which patients are/should be invited. Consulting patients separately from that stakeholder consultation would imply an extra, unnecessary step in the research process. Three other respondents confirmed this conclusion and felt that study results do not have to be confirmed by patients. Someone referred to the possible links of some patient organisations with the industry, and that we “wouldn’t let the industry help us interpret our results, so we should be careful about this”. On a general note, this respondent added that we cannot put aside the input of patients once we have asked for it. If our conclusion goes in a different direction, we should justify. This will take a huge amount of time if we involve patients in all research phases. Therefore, we should carefully select the steps in which patients’ input will be asked, to avoid creating false expectations.</p> <p>One respondent commented that in the phase of interpretation of analyses, triangulation is needed with different sources of information. Patient consultation could (not “should”) be one of them. A challenge might be to find a patient-expert who is able to provide the input we need in this phase.</p>



Patient organisations and/or patient umbrella organisations should be given the opportunity to review the KCE synthesis and give feedback before publication (=consultation).

Several respondents commented that this actually already happens, with the patient umbrella organisations being member of the Board of KCE and therefore this statement is redundant. Also making an exception for patients as compared to other stakeholders in this phase, does not seem appropriate to many respondents. Others think it is a good idea to also give other patient representatives the opportunity to review the synthesis, especially by patients who were already involved in previous phases of the research project. Nevertheless, one respondent argued that –if patients were involved in the previous phases of a KCE report (scoping, methods, etc.) - the synthesis should already reflect this involvement. This is the remit of the communication experts and the research team, there is no need for additional review consultation by patient organisations.

Practical concerns were raised for this statement. For example, it would require a translation of the synthesis in three languages: French, Dutch and German. It would be difficult to get these translations done at a moment where the time pressure on the researchers is already very high. Moreover, any changes following the review, would then have to be introduced in four language versions (including also the English version).

Not only the language issue as such but also the need to integrate the comments in the synthesis and give feedback to the patient-reviewer might considerably delay the publication of the report.

Individual patients and/or patient organisations and/or patient umbrella organisations and/or sickness funds should collaborate on the dissemination of the results of the KCE project.

Overall agreement that patients can play an important role in the dissemination phase. However, they cannot be obliged to collaborate, hence the ‘should’ is too strong and should be replaced by “should be invited to” or “should be encouraged to”.

One concern was raised that patients may be more willing to disseminate “positive” results than for example studies which conclude we should not reimburse a drug or device. Specifically in some fields the link between the industry and patients is very strong.



Based on the comments (both during the meeting and in the web-survey), we discussed within the research team:

- For statements that reached consensus in the second Delphi round: would re-wording clarify statements without changing the basic idea of the statement? If so, a reformulation was proposed, and it was assumed that in these cases, consensus would still be reached for the clarified statement.
- For statements that did not reach consensus after the second Delphi round: Is a reformulation possible that would increase the agreement amongst the KCE staff? This was only done when all comments for a particular statement went into the same direction.
- More general topics: some comments tackled a more general issue, e.g. related to the type of 'patient representative' to be involved (individual patient, patient organisation, umbrella organisation), or to operational aspects. It was decided to address these comments by adding a few general notes that precede the statements to help the reader putting the position statements in the right perspective (cfr infra).

Finally, when the comments went in different directions, no changes were made to the statement. The final decision about the reformulated statements was made by the general management.

The results of the team discussion and the final decision by the general management regarding the position statements are presented in Table 31.

- In terms of the general topics, the following considerations were made:
- It should be emphasized that **the management of KCE endorses** the set of position statements

- It should be made clear that the position statements relate primarily to **patient involvement** in KCE research **for the purpose of better addressing patient issues** in a research project, meaning that the study of some aspects of a research topic might not require patient involvement. The use of the term 'should' should be considered in that perspective: "if patient involvement is relevant (because there are potential patient issues), then patients should be involved in such or such way in this or that research phase".
- A general principle is that **expectations from the involvement**, both from the patients' side and the researchers' side, should be discussed openly at the beginning of the involvement process with all those involved.
- We should add that the **operationalisation** of the patient involvement, i.e. how it should be decided who can represent the patient, is **still open for discussion** and will be further specified in the (yet to be developed) KCE process book on patient involvement. The current statements remain therefore rather high level.
- The **distinction between individual patients, patient experts, patient organisations, patient umbrella organisations and sickness funds** must be **abandoned** in the high-level statements. The specification of who best to involve should be addressed in the process notes. Therefore, the statements will speak about "patients" as an overarching concept, encompassing different types of representatives


Table 31 – Reformulation of statements based on comments provided by KCE personnel (orange cells: statements for which there was dissensus)

Original statement	Re-formulated/clarified statement	Management decision
1 KCE perceives the fundamental ethical, as well as the instrumental and procedural rationales for patient involvement decisive enough to take a positive position towards patient involvement in health policy research.	Keep as is	Agree
2 The relevance and need for patient involvement in research projects should be assessed project by project.	The relevance and need for patient involvement in research projects should be assessed in every project .	Keep original formulation “project by project”
3 Patient involvement in health policy research is complementary to the review of scientific evidence and primary data collection, not a substitute for it.	Keep as is	Agree
4 Sufficient resources (people, time, budget) should be made available to ensure and support effective patient involvement in health policy research.	Keep as is	Agree
5 The planning of the projects has to be adapted to implement patient involvement on an optimal way.	The planning of the projects has to be adapted to implement patient involvement in an optimal way.	Agree with correction
6 Training should be organised for researchers and patients/patient organizations to effectively involve patients or be involved in health policy research.	Keep as is	Agree
7 Patient involvement activities in health policy research should be regularly evaluated and procedures revised when appropriate.	Keep as is	Agree
8 Patient contributions and potential impact should be reported in the study report.	Keep as is	Agree
9 Patient representatives who have been involved should receive feedback from KCE and provide feedback to KCE to potentially improve future collaboration.	In case of a collaboration with patients during a research project, patient representatives who have been involved should receive feedback from KCE and provide feedback to KCE about the collaboration , to potentially improve future collaboration.	Agree with clarification
10 Individual patients and/or patient organisations should be consulted in the scoping of the KCE projects to allow researchers to better describe the context.	Patients should be consulted in the scoping of the KCE projects to allow researchers to better describe the context.	Agree with re-formulation



11	Individual patients should contribute to the scoping by co-producing the elements that need to be addressed in the research project.	Patients should be consulted in the scoping phase to define the elements that need to be addressed in the research project.	Agree with re-formulation
12	Individual patient 'experts' and/or patient umbrella organisations should be consulted on the selection of methods for the projects.	Patients should be consulted on the selection of methods to be used in a project to study patient issues.	Reject statement
13	Patient organisations should be consulted in the selection of the outcomes to be included in the study.	Patients should be consulted in the selection of the outcomes to be included in the study.	Agree with re-formulation
14	Patient organisations and/or patient umbrella organisations and/or sickness funds could co-decide on the approaches for recruitment of participants if primary data collection in patients or users is needed.	Patients could co-decide on the approaches for recruitment of participants if primary data collection in patients or users is needed.	Agree with re-formulation
15	Patient organisations should be consulted to select and test the data collection instrument(s).	Patients should be consulted to select and test the data collection instrument(s).	Agree with re-formulation
16	Individual patients and/or patient organisations should be consulted to define the minimal important difference in patient outcomes.	Patients should be consulted to define the minimal important difference in patient outcomes.	Agree with re-formulation
17	Individual 'expert' patients should be consulted to interpret results of analyses.	Patients should be consulted to interpret results of analyses.	Reject statement
18	Patient organisations and/or patient umbrella organisations should be given the opportunity to review the KCE synthesis and give feedback before publication (=consultation).	Remove statement (reason: comments highlighted several practical issues making this approach actually unfeasible. Moreover, some considered that patients are already given the opportunity to review the synthesis, with patient umbrella organisations being part of the KCE Board)	Agree, remove statement
19	Individual patients and/or patient organisations and/or patient umbrella organisations and/or sickness funds should collaborate on the dissemination of the results of the KCE project.	Patients are invited to collaborate on the dissemination of the results of the KCE project.	Agree with re-formulation

Yellow rows encompass statements for which there was dissensus. Statement 11 achieved consensus for rejection. Reformulated words are in bold.



The discussion with the management about the reformulated position statements and about the general notes, led to the following results:

- The management agreed with the team suggestions regarding reformulation, clarification, rejection or removal of 16 statements.
- One additional statement was rejected by the management (regarding the consultation of patients in the interpretation of the results).
- For one statement, the management decided to keep the original formulation and not to follow the team's suggestion (regarding the assessment of the relevance and need of patient involvement project by project, rather than 'for every project').
- The management rejected the statement for which consensus for disagreement was reached at the second Delphi round with the KCE employees and did not follow the team's suggestion to reformulate this statement.

Regarding in particular the statements for which there was dissensus amongst KCE employees at the second Delphi round, the following decisions were taken:

- The statement for which all the comments went into the same direction (not agreeing with "*co-production* of elements that need to be addressed in the project" but would agree with "*consultation* of elements that need to be addressed in the project") was retained after re-formulation.
- One statement was retained by the management. It concerned the statement about the consultation of patients to select and test data collection instruments.
- The statement about the consultation of patients for the interpretation of the study results was rejected

- The statement about giving the patients the opportunity to review the synthesis and give feedback before publication was removed. The reason is twofold. First, comments highlighted several practical issues making this approach actually unfeasible, especially if this opportunity would be given to individual patient organisations. Belgium is a country with three official languages. It is not feasible nor efficient to translate the synthesis in three languages before review and to revise all three language versions after the review. This would take much time and resources. Second, currently patient umbrella organisations actually already have the opportunity to review the synthesis before publication, as member of the KCE Board. The syntheses and full reports are sent to the Board for review before the publication. Moreover, the results are presented during a face-to-face meeting with the Board members.

The final position statements are listed in chapter 16. Besides the statements that were discussed with the employees of KCE and retained, two position statements –confirming and perpetuating an actual situation– are included to strengthen the position of KCE on these points. They relate to the role of patients in the call for proposals and the formulation of the recommendations.



16 KCE'S POSITION STATEMENTS REGARDING PATIENT INVOLVEMENT IN HEALTH POLICY RESEARCH

KCE wants to involve patients as much as possible in its research projects, in order to support choices to be made during the research process about the (best) ways to evaluate patient-related aspects. This will improve the quality of its research about patient-related issues. Below are KCE's position statements on how it intends to do this. These are inextricably linked, and must therefore be considered as a whole.

KCE always has to find the balance between its commitments towards the patients and its legal remit. The management of KCE will try to maintain this balance in the most efficient way when making its choices.

A next step is the development of a process book with practical guidance for patient involvement in health policy research. This will cover several aspects, such as who to involve in which research phase, how to select the patient (representative) to be involved and which method to use to guarantee meaningful patient involvement.

1. KCE perceives the fundamental ethical, as well as the instrumental and procedural rationales for patient involvement decisive enough to take a positive position towards patient involvement in health policy research. Patients have the democratic right to be involved in research about them, and they can contribute a unique perspective to the research from their personal experience, competences and knowledge.
2. KCE aims to involve patients in all research phases if this is relevant and appropriate for the project. Patients should not necessarily be involved in all policy research projects. The relevance and need for patient involvement in research projects should be assessed project by project.
3. Patient involvement in health policy research is complementary to the review of scientific evidence and primary data collection, not a substitute for it.
4. Sufficient resources (human, financial, time) should be made available to ensure and support effective patient involvement in health policy research. KCE aims to assure this availability.
5. The planning and processes of the projects have to be adapted to implement patient involvement in an optimal way.
6. Researchers and patients or patient organisations should be trained to effectively involve patients or be involved in health policy research.
7. Patient involvement activities in health policy research should be regularly evaluated and procedures revised when appropriate.
8. Patient contributions and their potential impact on the research process should be reported in the research report.
9. Patients and KCE researchers should give feedback to each other about the collaboration, to potentially improve future collaboration.



- 10. Everybody, hence also patients, can already today submit topic proposals to KCE. This possibility should be maintained.**
- 11. Patients should be consulted in the scoping of the KCE projects to allow researchers to better describe the context of the research topic, taking patient issues into account.**
- 12. Patients should be consulted in the scoping phase to define the patient-related elements that need to be addressed in the research project.**
- 13. Patients should be consulted in the selection of the patient-relevant outcomes to be included in the study.**
- 14. Patients could contribute to the decision about the recruitment of study participants if primary data collection in patients or healthcare users is needed.**
- 15. Patients should be consulted in the selection and for the testing of the data collection instrument(s) to be used in patients or healthcare users.**
- 16. Patients should be consulted to define the minimal important difference in patient-relevant outcomes.**
- 17. Patients should be consulted to get input about the formulation of the policy recommendations. This is currently already the case, thanks to the presence of the Belgian umbrella organisations of patient associations in the Board of KCE. This possibility should be maintained.**
- 18. Patients should be invited to collaborate on the dissemination of the results of the KCE project.**



■ APPENDICES

APPENDIX 1. LITERATURE SEARCH AND CLASSIFICATION

Appendix 1.1. Introduction

We did not aim to find *all* relevant studies about patient involvement in health policy research but aim to find relevant good handles on how to involve patients/public in health policy research and relevant articles on the rationale to involve patients/public. Therefore, this review should be considered as a scoping review, rather than as a full systematic review, although we did apply systematic approaches to classifying the relevant literature identified.

Appendix 1.2. Sources

Appendix 1.2.1. PUBMED

The search is built on two content parts, i.c. 'involving patients/public' AND 'health policy research':

("Patient Participation"[Majr] OR "Community-Based Participatory Research"[Mesh] OR "Stakeholder Participation"[Mesh] OR "Community Participation"[Mesh] OR "Public Opinion"[Mesh] OR "patient engagement" OR "patient participation" OR "patient involvement" OR "public engagement" OR "public involvement" OR "public participation" OR "user engagement" OR "user involvement" OR "user participation" OR "community engagement" OR "community involvement" OR "community participation")

AND

("Health Policy"[Mesh] OR "Health Services Research"[Mesh] OR "Public Health Systems Research"[Mesh] OR "Outcome Assessment (Health Care)"[Mesh] OR "Health Care Reform"[Mesh] OR "Delivery of Health Care"[Mesh] OR "Biomedical Research"[Mesh] OR "Comparative Effectiveness Research"[Mesh])

This search gives on 10/07/18 44196 hits and consequently necessitates some limits:

1. Time limit: 2008-2018, resulting in 20883 hits



2. Publication type limit: review[ptyp], resulting in 1997 hits

In addition, there is a journal that is specifically dedicated to patient/public involvement in research: **"Research Involvement and Engagement"** *is an interdisciplinary, health and social care journal focussing on patient and wider involvement and engagement in research, at all stages. The journal is co-produced by all key stakeholders, including patients, academics, policy makers and service users* (<https://researchinvolvement.biomedcentral.com/>). Therefore, this journal is added to the search strategy.

This leads to the final Pubmed search, resulting in 2094 hits on 10/07/18

((("Patient Participation"[Mesh] OR "Community-Based Participatory Research"[Mesh] OR "Stakeholder Participation"[Mesh] OR "Community Participation"[Mesh] OR "Public Opinion"[Mesh] OR "patient engagement" OR "patient participation" OR "patient involvement" OR "public engagement" OR "public involvement" OR "public participation" OR "user engagement" OR "user involvement" OR "user participation" OR "community engagement" OR "community involvement" OR "community participation") AND ("Health Policy"[Mesh] OR "Health Services Research"[Mesh] OR "Public Health Systems Research"[Mesh] OR "Outcome Assessment (Health Care)"[Mesh] OR "Health Care Reform"[Mesh] OR "Delivery of Health Care"[Mesh] OR "Biomedical Research"[Mesh] OR "Comparative Effectiveness Research"[Mesh]) AND Review[ptyp]) OR "Research involvement and engagement" [TA]) AND ("2008/01/01"[PDat]: "2018/12/31"[PDat])

Appendix 1.2.2. Google

health AND policy AND research AND "patient public involvement" filetype:pdf limited to 2008-2018

N: 127, of which 20 possibly relevant

Appendix 1.2.3. Websites

- European Patients Forum: <http://www.eu-patient.eu/>
- European Patient's Academy on Therapeutic Innovation: <http://eupati.be/>
- Patient-Centered Outcomes Research Institute: <https://www.pcori.org/>
 - <https://www.pcori.org/engagement/what-we-mean-engagement>
 - <https://www.pcori.org/about-us/our-programs/engagement>
- Healthtalk:
 - <http://www.healthtalk.org/peoples-experiences/medical-research/researchers-experiences-patient-public-involvement/reasons-involving-patients-and-public-research>
- Zonmw www.zonmw.nl
- National Institute for Health and Care Excellence (NICE): <https://www.nice.org.uk/>
- Agency for Healthcare Research and Quality: <https://www.ahrq.gov/>
- Haute Autorité de santé: <https://www.has-sante.fr/portail/jcms/1249588/fr/accueil>
- International Alliance of Patients' Organizations: <https://www.iapo.org.uk/patient-involvement-research>
- NHS National Institute for Health Research :
 - <https://www.nihr.ac.uk/news/new-national-standards-launched-across-the-uk-to-improve-public-involvement-in-research/8141>
 - <https://www.nihr.ac.uk/about-us/CCF/funding/how-we-can-help-you/RDS-PPI-Handbook-2014-v8-FINAL.pdf>
 - <http://www.invo.org.uk/find-out-more/what-is-public-involvement-in-research-2/>
 - http://www.invo.org.uk/wp-content/uploads/2014/11/9938_INVOLVE_Briefing_Notes_WEB.pdf



- <https://www.phc.ox.ac.uk/ppi/information-for-researchers>
- Kingsfund:
 - https://www.kingsfund.org.uk/topics/patient-involvement?gclid=CjwKCAjwj4zaBRABEiwA0xwsPx9iregEokl_QUcsu2W-hc4NuFmI3NUROPDvPAIG0-IFfmPm-DeJhoC4KkQAvD_BwE
 - https://www.kingsfund.org.uk/sites/default/files/field/field_pdf/Library-reading-list-public-involvement-Aug2014.pdf
- INVOLVE: <http://www.invo.org.uk/>
- GIN: <https://www.g-i-n.net/document-store/working-groups-documents/g-i-n-public/toolkit/toolkit-2015>
 - Canadian Institutes of Health Research: <http://www.cihr-irsc.gc.ca/>
 - Canadian Health Services Research Foundation: <https://www.cfhi-fcass.ca/>
 - James Lind Alliance: <http://www.jla.nihr.ac.uk/>
 - L'Institut de recherche et documentation en économie de la santé (Irdes) : <http://www.irdes.fr/>

Done on 16/08/2018 ; resulted in 37 publications.

Documents/references from above searches were merged, de-duplicated and assessed on relevance first on title/abstract, then on full-text.

Appendix 1.2.4. Citing search

Since we might miss relevant articles due to the limitations in above searches/sources, additional searches will be performed in Google scholar (via Publish or Perish interface), by searching articles that cited relevant publications that were included from the other searches.

Appendix 1.3. Inclusion criteria

- About patient/public involvement somewhere in health research policy process
- AND
- About method for PPI in research
- OR
- About rationale for PPI in research
- OR
- About effects of PPI in research
- AND
- In western country
- AND
- English/French/Dutch

Appendix 1.4. Classification of retained papers

Appendix 1.4.1. References related to definitions and terminology in patient involvement

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Appendix 1.5. Summary of findings from reviews on the application, benefits, risks and challenges of patient involvement in research

Author	Summary of findings
Backhouse et al. (2016) ¹⁸¹	<ul style="list-style-type: none">• Older care-home residents can be successfully involved in the research process.• Small-scale studies involved residents as collaborators in participatory action research, whereas larger studies involved residents as consultants in advisory roles.• There are multiple facilitators of and barriers to involving residents as patient and public involvement members.
Bailey et al. (2015) ¹⁶²	<ul style="list-style-type: none">• Positive impacts of involvement for disabled children included increased confidence, self-esteem and independence.• Positive impacts for research were identified.• Involving disabled children in research can present challenges; many of these can be overcome with sufficient time, planning and resources.• Although a range of positive impacts were identified, the majority of these were authors' opinions rather than data.
Blackburn et al. (2018) ¹³⁹	<ul style="list-style-type: none">• About half of the studies included patient and public involvement to develop research ideas and during the study itself. Common activities included designing study materials, advising on methods, and managing the research.• Researchers reported beneficial impacts of patient and public involvement.• Beneficial impacts of patient and public involvement in designing studies and writing participant information was frequently reported. Less impact was reported on developing funding applications, managing or carrying out the research.• The main cost of patient and public involvement for researchers was their time.
Boote et al. (2010) ¹¹⁶	<ul style="list-style-type: none">• Contributions that members of the public made to research design were: review of consent procedures and patient information sheets; outcome suggestions; review of acceptability of data collection procedures; and recommendations on the timing of potential participants into the study and the timing of follow-up.• Numerous barriers, tensions and facilitating strategies were identified.
Boote et al. (2011) ¹¹⁹	<ul style="list-style-type: none">• The public was found to contribute to systematic reviews by: refining the scope of the review; suggesting and locating relevant literature; appraising the literature; interpreting the review findings; writing up the review.• Numerous tensions, facilitating strategies and recommendations were identified.
Boote et al. (2015) ²⁴⁷	<ul style="list-style-type: none">• For those papers where it was possible to determine the research stages in which the public was involved, the following stages were identified: identification of question or prioritisation (n = 41); research design (n = 27); data collection (n = 23); peer review of proposals (n = 11); commissioning and/or funding of research (n = 6); membership of study advisory group (n = 6); data analysis and interpretation (n = 6).



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- Brett et al. (2014)⁷³**
- The positive impacts identified were enhanced quality and appropriateness of the research.
 - Impacts were reported for all stages of research, including the development of user-focused research objectives, development of user-relevant research questions, development of user-friendly information, questionnaires and interview schedules, more appropriate recruitment strategies for studies, consumer-focused interpretation of data and enhanced implementation and dissemination of study results.
 - Some challenges were also identified.
 - Much of the evidence base concerning impact remains weak.
-
- Brett et al. (2014)¹⁶⁸**
- Service users reported feeling empowered and valued, gaining confidence and life skills.
 - Researchers developed a greater understanding and insight into their research area, gaining respect and a good connections with the community.
 - The community involved in research became more aware and knowledgeable about their condition.
 - Lack of preparation and training led some service users to feel unable to contribute to the research, while other service users and communities reported feeling overburdened with the work involved.
 - Researchers reported difficulties in incorporating patient and public involvement in meaningful ways due to lack of money and time.
-
- Brett et al. (2010)¹⁶⁹**
- The evidence base underpinning patient and public involvement in health and social care research is complex reflecting the wide diversity of the patient and public involvement landscape and activities. It is comprised of mainly qualitative or case study reflections of patient and public involvement, or cross-sectional studies reporting individual or organisational views of patient and public involvement, with relatively little critical evaluation.
 - The main ways in which the impact and outcomes of patient and public involvement are represented is through narrative description, which is usually too brief to provide a full understanding of impact.
 - The evidence base appears to be relatively weak in relation to the quality and detail of impact reporting, and needs significant enhancement.
 - There has been little focus on developing robust instruments capable of capturing or measuring patient and public involvement impact and this area is characterised by an absence of formal capture or measurement.
 - Despite the limitations in the evidence base, it was possible to identify patient and public involvement impacts in relation to the following areas: research and the research process, users, researchers, research participants, community, journals, policy makers and funders.
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- Camden et al. (2015)¹⁷⁰**
- There is a great interest in rehabilitation to engage stakeholders in the research process.
 - Stakeholder engagement outcomes were rarely formally evaluated.
 - Perceived outcomes of stakeholder engagement included the creation of partnerships, facilitating the research process and the application of the results, and empowering stakeholders.
 - Further evidence is needed to identify effective strategies for meaningful stakeholder engagement that leads to more useful research that positively impacts practice.
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- Cargo et al. (2008)³⁰**
- Consistent evidence demonstrates that insider knowledge can enrich academic partners' understandings of the needs, priorities, and health concerns of communities, organizations, and the public health system and lead to refined and new research questions.
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- Engaging with non-academic partners in shaping the research purpose has the advantage of enhancing contextual readiness for research implementation.
- Participatory research approaches enhance the relevance and importance of the research for non-academic partners' needs and circumstances.
- Growing evidence from participatory research studies employing different designs, methodologies, and methods shows that participatory research can improve research quality by increasing recruitment and retention rates, reducing reporting bias, and reducing measurement error from survey and interview questions that are not culturally aligned.
- Non-academic partners benefit primarily through the development of their capacity, empowerment, and ownership.
- Key benefits to academic partners include enriched interpretation of research findings through integrating different stakeholder perspectives, the potential for wider dissemination and translation of research results, enhanced research capacity for participatory research, and opportunities to train students in state-of-the-art participatory research approaches and community development.
- For non-academic partners, scientific knowledge can be applied to improve existing programs or to create new programs, practices, services, and policies.
- Integrating stakeholder perspectives with research results can lead to research products that are tailored to meet the needs of implementing systems, implementers, and end users.
- Partnering organizations can also enhance their credibility and leverage additional resources by forming alliances with academic partners.

Chen et al. (2010)¹⁴⁸

- In CBPR, dissemination beyond scientific publication is largely occurring.
- Challenges to timely and widespread dissemination remain.
- Current dissemination to community participants and the general public is variable.

Concannon et al. (2014)¹⁷¹

- It was not possible to validate claims of the benefits and challenges related to stakeholder engagement.
- About one in five articles reported that stakeholder engagement improved the relevance of research, increased stakeholder trust in research and researchers, enhanced mutual learning by stakeholders and researchers about each other, or improved research adoption.
- A smaller number reported that engagement improved the transparency of research (6%) and increased understanding of the research process (9%).
- The most common challenge reported was that stakeholder engagement is time consuming (19%), requires researcher flexibility, trust among researchers and stakeholders, commitment from both the researchers and stakeholders to maintain contact and participation, difficulty establishing stakeholder representativeness throughout the course of the research program, increased ethical concerns in some institutional review boards, stakeholder distress while participating (particularly with patients and family members), and difficulty overcoming cultural differences between stakeholders and researchers (all between 3 and 6% of the articles).

Conklin et al. (2012)¹⁷²

- Sound empirical evidence of the outcomes of public involvement activities in health care remains underdeveloped.
- The concept and indicators used to examine and determine outcomes remain poorly specified and inconsistent, as does the reporting of the evidence.
- There was some evidence for the developmental role of public involvement, such as enhancing awareness, understanding and competencies among lay participants.
- Evidence for instrumental benefits of public involvement initiatives was less well documented.



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- Despite the growing body of work on public involvement in health-care policy, evidence of its impact remains scarce; thus, firm conclusions about involvement activities that are appropriate and effective for policy development are difficult to draw.
 - However, focus on outcomes risks missing the normative argument that involving the public in the health-care policy process may be seen to be of intrinsic value.
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Cook et al. (2008)¹⁷³

- In 14 of the 20 studies reviewed, CBPR led to community-level action to improve the health and well-being of the community members.
 - Observational studies that investigated problems posed by the affected community and that incorporated qualitative methods were more likely to lead to action.
 - The collaboration among government scientists, university researchers and community partners emerged as a new model of CBPR partnerships that effectively integrates research and action.
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De las Nueces et al. (2012)⁸⁸

- Significant publication gaps remain between CBPR and other interventional research methods.
 - CBPR may be effective in increasing participation of racial and ethnic minority subjects in research and may be a powerful tool in testing the generalizability of effective interventions among these populations.
 - CBPR holds promise as an approach that may contribute greatly to the study of health care delivery to disadvantaged populations.
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Deja et al. (2014)¹⁷⁴

- There is limited empirical evidence to support claims of impacts of patient involvement in research. The majority of the papers is theoretical or based on anecdotal evidence.
 - Improved relevance, credibility and acceptability appear to be the most commonly listed benefits.
 - The experiential knowledge that representatives contribute gives credence to their ability to make judgment calls on how relevant the purpose and outcomes of a research project are to the targeted population. This is believed to increase the project's usability to both its participants and the wider research community. This perceived usability in turn gives the research increased credibility and acceptability, making it more likely to influence practice and improve healthcare. While this intuitively makes sense, there is limited evidence to support these claims.
 - It has been suggested that patient and public involvement can improve the clarity of participant information, removing jargon and making it more salient to potential participants.
 - Patient and public involvement can facilitate more representative sampling.
 - An additional benefit is the potential to increase recruitment. However, evidence of increased recruitment is largely anecdotal as it is difficult to measure.
 - It has been suggested that patient and public involvement can expedite ethical approval and data collection, shortening the time frame of the research.
 - Patient and public involvement is claimed to broaden opportunities for dissemination, increasing the impact of research.
 - Reporting of negative impacts of patient and public involvement on the research processes are limited and are mostly described as barriers or challenges to implementing patient and public involvement rather than negative impacts on research.
 - Added cost, time and complexity of patient and public involvement are the main barriers to its implementation reported in the literature.
 - Only a handful of the papers reviewed provided empirical evidence on the effect of being involved in research on the representatives.
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		<ul style="list-style-type: none">• Representatives report increased confidence in their own capabilities and influence, feeling valued, improved self-esteem and an overall sense of well-being as outcomes of their roles. They also value gaining knowledge of their condition and the research process, alongside new skills and experiences.• While there is limited evidence about the effects of patient and public involvement on the research process and the representatives there is even less on the effect on the professionals.• There is currently little in-depth empirical evidence on the impact of patient and public involvement and the evidence that is available appears to be limited as it often overlooks negative or challenging aspects of patient and public involvement.
Domecq (2014)³⁴	et al.	<ul style="list-style-type: none">• In general, engagement was feasible in most settings and most commonly done in the beginning of research (agenda setting and protocol development) and less commonly during the execution and translation of research.• No comparative analytic studies to recommend a particular method were found.• Patient engagement increased study enrolment rates and aided researchers in securing funding, designing study protocols and choosing relevant outcomes.• The most commonly cited challenges were related to logistics (extra time and funding needed for engagement) and to an overarching worry of a tokenistic engagement.
Drahota (2016)¹⁷⁵	et al.	<ul style="list-style-type: none">• Most studies involved case studies using qualitative methods to collect data on the collaborative process.• The most frequently reported hindering factor was excessive time commitment.• The next most common hindering factors were unclear roles and/or functions of partners, followed by excessive funding pressures or control struggles.• Additional hindering interpersonal process factors were poor communication among partners (13%); inconsistent partner participation or membership (11.1%); a high burden of activities/tasks (9.3%); lack of shared vision, goals, and/or mission (9.3%); mistrust among partners (7.4%); lack of a common language or shared terms (7.4%); differing expectations of partners (7.4%); and a bad relationship among partners.• Forty-two (77.8%) of the articles reported that the community-academic partnerships had one or more proximal outcomes, such as partnership synergy (18.5%), knowledge exchange (25.9%), or tangible products (72.2%), with the most common proximal outcome reported being the development or refinement of a tangible product. Eighteen (33.3%) of the articles reported one or more distal outcomes, such as the development of or an enhanced capacity to implement programs or interventions (13%), improved community care (18.5%), sustainable community-academic partnership infrastructure (5.6%), and changed community context (1.9%).
Duffett (2017)¹⁷⁶		<ul style="list-style-type: none">• Reports of impact were primarily self-reported by the research team and robust measurements and validation for outcomes are largely lacking across all studies.• Reported positive impact of patient engagement include: improved relevance of research to patient priorities, significant contributions to trial design (deciding on comparators, outcomes, protocols), improved patient information material and/or informed consent documents, improved clinical trial enrolment and decreased attrition, improved dissemination and/or implementation of research findings, and increased public trust in research.• Challenges or negative impacts included: increased time; increased cost, fear of tokenism, changes to research scope that were unfeasible, and uncertainty on how to resolve conflicts.



- A report was commissioned by the UK's National Institute for Health Research (NIHR) to evaluate the impact and outcome of policy changes requiring patient engagement in funded research. In a UK national scoping review of studies funded or completed within the prior 2 years, 51% (92 studies) had some evidence of patient engagement. A survey of chief investigators found that the strongest influence on the extent of patient engagement related to funding requirements and study design. A case study and in depth realist evaluation of 22 studies was then conducted. Outcomes of patient engagement included research priority/question setting, study marketing, changes to the design including the interventions, and ensuring participant safety and recruitment. Studies with the most embedded patient engagement demonstrated inclusion of more patient-related outcomes. One study reported an objective rise in recruitment rate following a change to participant information sheet that was made through patient contributions.
- A similar program evaluation was performed by the US Patient Centered Outcomes Research Institute (PCORI). Reported contributions to the research included: changes to the study outcome or goals, changes to methods, enhanced access to study populations or settings, modifications to study intervention, and refinement of the study instrument.
- The most frequently reported challenges were lack of time by both patients and researchers, and lack of resources and training. The impact of engagement on the trial results could not yet be assessed.
- While most reported experiences with patient engagement in research has been positive, potential hazards should not be minimized and it is possible that some degree of selective reporting may be present.

Forsythe et al. (2014)⁷⁰

- No empirical assessments of engagement practices and their effectiveness were found, although authors reported benefits of engagement and identified changes to their research processes.

Frankena et al. (2015)⁸⁹

- The presence of two elements of added value was determined in each paper: expected and experienced added value. The reported expected added value was unspecific and mainly focussed on demands by policy and funding bodies or ethical grounds (i.e., ethical notion and giving people with intellectual disabilities a voice). The experienced added value was more concrete and focused on the gains attributed to an inclusive methodology for specific stakeholders.
- People with intellectual disabilities were empowered; gained skills; gained confidence; gained experiences; employment; felt they could contribute; felt respected; experienced personal development; and experienced mutual understanding.
- Research(ers) experienced increased quality and validity; developed appropriate research materials; facilitated research with people with intellectual disabilities; safeguarded ethical standards; developed relevant research and outcomes; learned new skills; improved data analysis; facilitated recruitment; improved data dissemination; and experienced mutual understanding.
- For health care professionals, inclusive health research resulted in awareness of people with intellectual disabilities' needs.
- Papers were inconsistent in terms of the information provided on expected and experienced added value.
- None of the added value was formally measured.

Gagnon et al. (2011)¹⁷⁷

- Although many examples retrieved in this review showed that patients' or the public's perspectives could add important dimensions to the evaluation of health technologies and clinical interventions, the need remains for systematic and rigorous empirical studies of patient and public involvement in HTA.

Puts et al. (2017)³¹

- Patient engagement has shown to improve the conduct of studies by making the study design more relevant and feasible, and improving recruitment rates and uptake of research findings by patients.
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- A review of 65 papers, mostly from the UK and the USA, showed there were several personal benefits for patients involved. These consisted of feeling listened to and empowered, feeling valued, feeling part of a team, having improved access to information, being able to engage with researchers (which helped the patients understand research better and develop a more positive attitude towards research), and gaining a number of skills such as public speaking, group working and interviewing.
 - Negative impact of involvement for patients included frustration due to feeling not valued or listened to, feeling marginalized, feeling not being taken seriously, apprehension about engaging in something, increased emotional burden due to having to recall their own experiences and listening to those of other patients.
 - In a systematic review on the impact of patient engagement, 35 papers were identified that studied the impact on researchers. The benefits of engaging patients included the research team gaining new insights into the research issues and a greater understanding of patients' needs.
 - Including patients on the team also led to greater diversity and sometimes even less workload for researchers, whose role changed from researcher to advisor.
 - The most commonly identified challenges were needing more time to engage patients, having to work on patient relationships, and needing more funds to implement patient engagement.
 - Researchers also sometimes felt uncomfortable when patients' ideas did not match their expert vision, particularly when researchers and patients had a different vision of what constitutes good research.
 - Sensory and communication difficulties, the fluctuating health state of patient participants, cognitive impairment, dominance of some patients in meetings and low energy of patients to participate can be challenging for researchers.
 - Further challenges included: lack of predefined roles and expectations, difficulty sharing power, and dealing with patients who have with their own agenda.

Shen et al. (2017)¹²⁸

- A robust evidence base is currently lacking on how to effectively engage parents as co-researchers.
- The success of parental engagement in research is based on anecdotal comments, surveys, individual interviews or research diary data.

Staley (2015)¹⁷⁸

- This review reflects on the use of quantitative approaches to evaluating impact. It concludes that the statistical evidence is weakened by not paying sufficient attention to the context in which involvement takes place and the way it is carried out.
 - The impact of involvement is highly context dependent. If patients are involved in reviewing a clinical trial protocol then the impacts are most likely to be related to research design and recruitment strategies, but if they are involved in dissemination of research results, then the impact will most likely be on implementation and changes to practice.
 - One of the commonly reported impacts of involvement is an increase in recruitment rates.
 - Reported impacts of involvement on research:
 1. Impact on the research agenda—the topic, research question and funding decisions
 2. Impact on research design and delivery—influencing the research design, tools and choice of method, recruitment, data collection and analysis, writing-up and dissemination.
 3. Impact on research ethics—the consent process and developing ethically acceptable research
 4. Impact on the people involved
 5. Impact on the researchers
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- 6. Impact on participants
 - 7. Impact on the wider community
 - 8. Impact on community organisations
 - 9. Impact on implementation and change
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**Tierney
(2016)**¹⁷⁹

et al.

- Authors' appraisals of their work were mostly positive.
 - The most consistent claim made was that service users offered a unique and practical expertise that added credibility to the work with positive impacts on service delivery of research. Many authors reported that service user involvement added real-world connection to their research, and changed the mind-sets of researchers.
 - There were reported benefits for service users: increase in confidence and self-knowledge, in confidence in making health-care decisions, the sense of power, and participants learned how to speak up and talk back. Equality in the research process led to positive interactions and equality of interaction. Interestingly, only one paper provided data from service users directly to support these claims.
 - Negative outcomes were rarely reported.
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APPENDIX 2. EXAMPLES OF PATIENT INVOLVEMENT STRUCTURES IN HTA AGENCIES

Appendix 2.1. National Institute for Health and Care Excellence (NICE)

Appendix 2.1.1. Rationale and objectives for patient and public involvement

NICE has an explicit commitment with regard to patients, service users, carers and public involvement.^{mmmm} Involving patients in policy research is part of this commitment. In its guide to public involvement in the development of “NICE guidances and standards”, NICE states that the views of all members of the NICE committees are given equal weight during discussions about interpretation of the evidence.

Policy-research related statements included in the commitment are (verbatim²¹³):

- NICE will ensure that all NICE advisory committees and working groups have at least two lay members (patients, service users, carers or members of the public)
- NICE will provide opportunities for patients, service users, carers and the public to give evidence and testimony that can inform the development of our guidance and quality standards
- NICE will offer support and training to lay people who contribute to NICE's work

^{mmmm} <https://www.nice.org.uk/about/nice-communities/public-involvement/patient-and-public-involvement-policy>

ⁿⁿⁿⁿ <http://indepth.nice.org.uk/PIP-annual-review/index.html>

- NICE will offer payment to lay members of NICE advisory committees and working groups in recognition of their contribution

The rationale for involving patients and citizens in its work processes is that “NICE believes that lay people and the voluntary and community sector organisations that represent their interests should have opportunities to contribute to developing NICE guidance, advice and standards”.ⁿⁿⁿⁿ

NICE's policy for public and patient involvement relies on two main principles:

1. lay people, and organisations representing their interests, should have opportunities to contribute to developing NICE guidance, advice and quality standards, and support their implementation, and
2. because of this contribution, NICE's guidance and other products have a greater focus and relevance for the people most directly affected by our recommendations.

The objective of patient and citizen involvement is to establish guidelines and recommendations with a focus and relevance for the people most directly affected by them, i.e. the people who use health and social care services, their carers, families and the public.

Appendix 2.1.2. NICE's public involvement policy

NICE has developed a specific policy to promote patient and citizen involvement in the development NICE guidelines and HTA.^{oooo} The levels of involvement range from information (publishing guidelines) to consultation (participation in workshops to discuss the scope, public consultations).

Patients can contribute to the guideline development process by^{pppp}:

^{oooo} <https://www.nice.org.uk/about/nice-communities/public-involvement/patient-and-public-involvement-policy>

^{pppp} <https://www.nice.org.uk/process/pmg22/chapter/how-you-can-get-involved>



- encouraging people with relevant skills and experience to apply to join the committee working on a guideline
- attending a workshop, if one is held, to discuss the scope (which lists what the guideline will and will not cover)
- providing evidence if the guideline developer makes a 'call for evidence'
- commenting on the draft scope and the draft guideline, including on equality issues
- helping NICE to promote the guidelines and put them into practice
- telling NICE about reasons a guideline might need updating earlier than planned and contributing to consultation on whether to update a guideline

All stakeholder organisations have to register their interest to be involved in the guideline development on a particular topic via an online form.

In the guideline development committees, people are included for their individual experience and do not represent their organisations. But in all guideline committees, also at least 2 lay members are involved. Lay members are considered to be part of the public stakeholders. Public stakeholders encompass national patient, service user, carer and community organisations that represent the interests of people whose health or care is covered by the guideline, and local Healthwatch organisations. Citizens are involved in a Citizen Council, consisting of 30 people who represent the social diversity of the British population.

For participation in the workshops, NICE mainly works with expert patient representatives, i.e. people that represent an organisation and are experts in guideline development. Organisations are asked to nominate one delegate to coordinate and consolidate the input from the members of the organisation. Also for the guideline consultation, organisations are asked to combine their comments into one response.

The developer of a guideline might assess the relevance and acceptability of a guideline with people affected by the guideline. This can happen through

a group discussion, interview or survey or through feedback on the draft guideline.

Once a draft guideline is ready, a public consultation takes place. Consultation is defined by NICE as giving a chance to stakeholders to comment on a draft scope or guideline. The committee then considers for each comment whether they require changes in the draft guideline. The committee formulates a response to each comment. If changes in the guideline have been made, this is made clear in the response, if not, it is explained why no changes have been made. All comments and responses are published on the NICE web-site.

Appendix 2.1.3. Organization and coordination of patient involvement activities

A specific patient involvement unit (the Patient Involvement Programme, PIP) coordinates and supports patient involvement through information and training activities. The programme works across all NICE activities to make sure that patients and the public can participate meaningfully in NICE's activities (i.e. not limited to research). The unit is also in charge of the recruitment and accreditation, financial support, the development of versions for patients of the reports and the evaluation of the activities.

PIP supports between 200 and 250 individual lay committee members and experts. They identify experts to offer their expertise to the committees. In 2017-2018, they identified 82 patient experts for technology appraisal, highly specialized technologies and medical technologies committees and 23 patient experts for the Scientific Advice Programme.

Appendix 2.1.4. Experience with patient involvement and evaluation

In 2017-2018, NICE evaluated its Public Involvement Programme (PIP).

An evaluation was made of the participation of expert patients in NICE's committees. The perceptions of patients varied depending on the treated subject and previous experience with the expert committees. However, 91% rated their experience as 'good' or 'excellent' in 2017-2018. Highlighted issues were the technical language used regarding clinical aspects, quantitative evidence being preferred over qualitative evidence. The



important role of the moderator was also highlighted by the participants. The contributions of patients thus depend, to a large extent, on the moderator's abilities to actively involve them in the debate.

Based on the results of the evaluation, concrete actions were taken, such as producing guidance on selection of lay members for the recruiting team, developing advice for moderators on best practice for lay involvement, and revising lay member information packs and checklists for phone calls with lay members to ensure that issues important to them are covered.

New since 2018 is the voluntary and community sector (VCS) evidence submissions for diagnostic and interventional procedures. The submissions enable VCS organisations to share patient data, perspectives, and issues that might complement or inform the published evidence and committee discussions. Previously only individual patients were invited to contribute evidence.

A few changes to the recruitment have been made as well:

- increasing the proportion of advisory committee positions of black, Asian and minority ethnic groups
- establishment a PIP Expert Panel of patients and the public.

It was found that the representation of black, Asian and minority ethnic groups was insufficient up to now. Therefore, PIP designed an engagement project to identify barriers to these population groups to be involved as lay members at NICE. They consulted representative organisations and learnt that in order to fully engage with these communities, they need to work at a local or regional level to meet people in their local communities and change the language of the recruitment materials (less detailed, less technical, and easier to understand).

The aim of the PIP Panel is to provide an expanding pool of patient and public expertise with knowledge and experience of NICE's work to contribute to NICE committees, which makes it easier to identify people with specialist input (as (patient) experts or reviewers) or members, without having to go through an open recruitment process on each occasion. Nevertheless, the idea is to refresh the panel on a regular basis.

Involvement of patients through questionnaires (i.e. committees requesting written information from patients with experience with the procedures considered) was also assessed. Committee members' views on patients' input were asked and received for 7 out of 17 product assessments for which patient input was sought. According to the respondents, the input from patients had an impact on the committee's decision-making. Assessment of 'impact' varied across committee members but the majority agreed it reinforced the other evidence. The mere fact of measuring the impact of patient input also seems to have a direct impact.

Two examples of patient input in health technology assessments are described in Box 10.

Box 10 – Examples of patient input in health technology assessments at NICE

Example 1: HTA of joint fusion surgery for low back pain

NICE received 15 questionnaires from patients who had had joint fusion surgery for low back pain. The published evidence demonstrated the procedure to be safe and effective. Information from patients identified that people commonly had to use crutches for a number of weeks following surgery. The committee added a comment to the guidance to reflect this.

Example 2: HTA of radiation therapy for Dupuytren's disease

NICE received 34 questionnaires from patients who had had radiation therapy for Dupuytren's disease. The committee noted that the patient feedback demonstrated a lack of understanding from the patients of the purpose of the procedure. The committee included a comment in the guidance suggesting clinicians should provide patients with clear, written information about the procedure and its purpose.



Important conclusions from the assessment of the patient involvement programme were:

- that it is important to formally feed back to the contributors (both individuals and organisations) about the impact of their involvement;
- that staff should routinely check with candidates about any special requirements related to illness or disability, at interview and on appointment;
- to avoid the use of jargon;
- during meetings, to provide an agenda slot for patient or lay contributors to give their input (potentially by means of a presentation);
- to develop activities on social media to reach more members of the public and different communities and to have conversations with people that would normally not be reached

Appendix 2.2. Healthcare Improvement Scotland^{qqqq}

Objectives of patient involvement

By means of patient group submissions, the Scottish Medicines Consortium (SMC) aims at understanding the experiences of patients, their families and carers. Patients, members of their families and carers can provide information about what it is like to live with a condition, and advantages and disadvantages of medicines that may not be available in the published literature. This information may complement standard quality of life measures.

How are patients involved?

The SMC works in partnership with patient groups to gather this information through **patient group submissions**.

Companies submitting a file to SMC must include a 'Summary Information for Submitting Patient Groups' in their submission, using a specific form.

This summary information is used by the SMC Public Involvement Team to inform relevant patient groups about ongoing appraisals for which patient group submissions are requested. The SMC Public Involvement Team identifies patient groups for each appraisal, and encourages and provides support to them to provide input.

The 'Summary Information for Submitting Patient Groups' prepared by the companies should be a patient/public friendly version of their submission. Companies are advised to focus on the impact and implications for patients, such as:

- Severity of the condition
- Need for the medicine, including level of unmet need and how the medicine addresses it
- Added value of medicine for patient and patient's carer/family including secondary trial end-points including those related to Quality of Life
- Key side effects and the impact on Quality of Life

Representatives of patient groups identified by the SMC Public Involvement Team may wish to obtain additional information from the submitting company about the treatment(s) under consideration.

The Association of the British Pharmaceutical Industry (ABPI) has developed a code of practice for the development of Summary Information for Submitting Patient Groups, and relationships with patient groups. The code of practice specifies, amongst others, that information about prescription only medicines made available to the public must be factual and presented in a balanced way. Companies should be able to substantiate information with scientific evidence.

The submission should be sufficiently concise (5-10 pages), structured as questions and answers formulated in plain non-technical English.

^{qqqq} <https://www.scottishmedicines.org.uk/making-a-submission/>



The submission should contain the following parts:

- Front page: including the approved and proprietary name of the product, the submission date and name of the company, name and position of the main contact person for patient groups.
 - Question 1: What condition is the medicine to be used for? Brief overview of the condition and the target population and selected sub-group of the licensed indication.
 - Question 2: How is this condition currently managed in Scotland? Outline of the current patient pathway and current treatment(s) likely to be displaced by the medicine under review, which may include non-medicine treatment options. Consideration of the severity of the condition and the implications for patients.
 - Question 3: How does the medicine work? How might the medicine be different and why might this be relevant to the way patients are managed?
 - Question 4: How effective is this medicine and is it different from other medicines currently available to treat this condition? Detail of any unmet need and how this is addressed by the medicine. Brief and simple summary of the clinical trial results. Description of outcomes that are likely to be most important to the patient. Advantages and disadvantages from a patient perspective compared to current treatment(s)? Factual information and balanced presentation in a balanced way. Presentation of current body of evidence relating to the medicine and its benefit/risk profile.
 - Question 5: How is the medicine administered and how will this affect patients and carers? Form, frequency, handling and self-administration/or otherwise. Consideration of the impact on patient care, such as avoiding the need for hospital visit.
 - Question 6: What are the side effects of this medicine and how are they managed? Main side effects that are likely to be experienced.
 - Question 7: What is the quality of life impact of this medicine on patients and their carers? What is likely to be most important for

the patient and patient's carer/family. Added value of the medicine for patients and carers compared to current treatment(s)?

Further online information about the medicine which patient groups may find useful might also be provided, such as published clinical trial data, publicly available regulatory documents regarding this medicine (e.g. Public Assessment Report), patient information materials and websites.

Appendix 2.3. Canadian Agency for Drugs and Technologies in Health

The Canadian Agency for Drugs and Technologies in Health (CADTH) has developed different mechanisms and structures for patient involvement.

CADTH includes patient input in its scientific advice development process. Patient input is collected in two ways:

1. Via information provided by the companies that apply for scientific advice in the context of the CADTH Scientific Advice Program. Companies have to submit information about their patient engagement activities, i.e. any consultations or interactions with patients or patient groups related to their product development program regarding input on the design of the clinical trials.
2. Via patient interviews. CADTH itself contacts at least one relevant patient group for each application for scientific advice, to gather information from patients directly about current therapies and remaining unmet needs. Interviews are performed with patient groups or, if none exists for the condition under consideration, individual patients with the condition. Interviewees are financially compensated for their involvement.

CADTH asks applicants (companies) to complete a "Patient Drug Information Form", which is a template for providing information about the drug and the planned phase 3 clinical trials. The completed form is used by CADTH during their conversation with patient(s) representative(s) about the application. Patients are required to sign a non-disclosure agreement and report possible conflicts of interest. Confidential information provided by the



applicant is shared with patient representatives only if the applicant has given permission for this.

Objectives of the involvement are to identify the needs and to assess the first draft of the HTA report. The participation is open to any agent interested in the assessed topic but can vary depending on the type of product and subject. The opening of an assessment report is actively communicated through "CADTH e-mail-Alerts" and its Twitter accounts. There are, however, guidelines on who can participate and electronic forms to submit contributions. Patients and consumers can participate individually through the website. The contributions are valued by an independent evaluation committee who considers whether they should have an influence on the recommendations.

Experience with patient involvement and evaluation

In 2011, CADTH commissioned an external evaluation on the involvement of patients in the HTA process. Evaluation was performed through forms, with feedback obtained from patient groups, evaluation experts and industry. The number of forms sent ranged between none and 9 depending of the technologies evaluated, with an average of 1,8. Most experts found that the information provided by the patients was relevant. However, the patients indicated that there was not enough time to complete the forms (15 business days). They also point out that the Patient Drug Information form was not extensive enough to cover a large number of aspects, such as the psychological impact.

CADTH recognizes that best practices for involving patients in the early scientific advice program are not yet established but the process will evolve as more experience is built up. I

APPENDIX 3. INTERVIEW GUIDES FOR THE SEMI-STRUCTURED INTERVIEWS ABOUT PROJECTS INVOLVING PATIENTS

Appendix 3.1. Interview guide for research centres

Question guide

- Could you describe your project?
- How do you refer to your target group?
- Which patients have been involved in your project?
- How were the patients identified and recruited?
- Why did you choose to involve patients?
- Are there conditions for patient involvement?
- How do you convince patients to participate?
- Do you offer (financial) incentives for participation in the involvement activities?
- In which step(s) of the research are the patients involved?
- Is there any task / research activity in which you would not involve patients?
- Do you think that we can involve patients in every topic / every theme?
- Did you measure the impact of the patient involvement on your research?
- Did you ask the patients for feedback?
- What are the lessons learnt for involving patients?
- What are the pros and the cons of involving patients?
- Do you have any best practice to recommend?



Appendix 3.2. Interview guide for sickness funds

Interview guides with the sickness funds

- According to your expertise, do you think it is necessary to involve patients in health research? What should be the level of involvement of patient in research projects?
- In which step(s) of the research are the patients involved?
- Which patients should be involved in the project?
- Why should it be necessary to involve patients?

- What are the pros and the cons of involving patients?
- Do you think that we can involve patients in every topic / every theme?

- How do you refer to your target group?

- Are there conditions for patient involvement?
- Do we need (financial) incentives for participation in the involvement activities?
- Do we need to train the patients?
- Is there any task / research activity in which you would not involve patients?

- Do you currently have research projects in which patients are involved? Could you describe it?
- What are the lessons learnt for involving patients?
- Did you measure the impact of the patient involvement on your research?
- Did you ask the patients for feedback?

- Do you have any best practice to recommend?



APPENDIX 4. KCE CULTURE DATA ANALYSIS

Appendix 4.1. Overarching themes and corresponding nodes

THEME	INCLUDED NODES*
Acceptability/acceptance	Reporting_Pro_Patients can enhance acceptability of reports
Adequate language use	<ul style="list-style-type: none">• Data Collection_Pro_Patients can explain the logic of questionnaires better• Data Collection_Pro_Patients can train researchers to use appropriate wording• Diss_Condition_If adapted communication to Patients• Recommendations_Pro_To make sure formulations are comprehensible for patients• Reporting_Contra_Vulgarisation no use in reporting phase• Reporting_Pro_patients can help to report at patient level• Scoping_Pro_Patients can check appropriateness of wording of instruments
Anonymity	<ul style="list-style-type: none">• Call_Condition_If anonymity is respected• Call_Contra_Use of a specific patients form might allow identification of submitter• Selection_Contra_threat for anonymity of the proposals
Appropriateness	<ul style="list-style-type: none">• Analysis_Condition_If patient involvement is a free choice in function of the project• Call_Contra_Call should target policy makers (only)• Data Collection_Condition_If not for each project• Design_Condition_If not in quantitative designs• Design_Condition_Only if data collection in patients• Diss_Condition_If relevant• Diss_Contra_Creation of patient fiches not always possible• Recommendations_Contra_patients are rarely a direct target group for the recommendations• Recommendations_Pro_easy to involve patients in this stage• Scoping_Condition_If patient organisation which have expertise on the topic• Scoping_Condition_only for patient related topics• Selection_Contra_Negative experience with stakeholder involvement in the past



Complementarity	<ul style="list-style-type: none">• Analysis_Condition_If patients are not the only source of information• Analysis_Pro_ patients can help to interpret findings• Diss_Contra_Possible clash between KCE and patient organisations' recommendations• Recommendations_Pro_Allows recom to go beyond the purely medical aspects• Scoping_Contra_Potential clash between patients' and decision makers' needs• Scoping_Pro_ patients provide unique information• Selection_Pro_Complementarity - patients highlight aspects not identified by scientists
Conflict of interest	<ul style="list-style-type: none">• Analysis_Condition_If professional activity of patients is taken into account• Analysis_Contra_Patients might be too focussed on own situation (miss broader perspective)• Data Collection_Condition_If patients' conflicts of interest are screened• Recommendations_Condition_If openness about conflict of interest of patients• Recommendations_Contra_patients have a conflict of interest• Selection_Condition_If it can be ensured that patients are not driven by commercial or industrial interests• Selection_Contra_patients have a conflict of interest
Credibility	<ul style="list-style-type: none">• Data Collection_Contra_reduces scientific credibility• Diss_Pro_ patients are more credible than researchers• Reporting_Contra_reduces scientific rigour of the report• Selection_Pro_Selection procedure will be taken more seriously
Early in the research process	<ul style="list-style-type: none">• Analysis_Condition_If involved from the beginning• Call_Pro_Good to involve patients from the start of the research process• Data Collection_Condition_If patients are involved from the beginning of the research process• Diss_Condition_Only if patients have been involved in previous phases• Selection_Condition_If also involved in other research phases
Ethics	<ul style="list-style-type: none">• Analysis_Condition_if patient exhaustion is taken into account• Recommendations_Contra_Risk of frustrated patients if recommendations are not implemented• Recommendations_Pro_Patient is affected stakeholder like any other• Reporting_Contra_KCE should not privilege one stakeholder group• Selection_Condition_If also other stakeholders are involved



Experts by experience	<ul style="list-style-type: none">• Analysis_Condition_If patients are mainly involved in interpretation, not in analysis itself• Analysis_Pro_patients can define main variables and clinical significance levels• Analysis_Pro_Patients' feedback on crude results allows refinement of analysis• Call_Pro_Identification of gaps or needs informed by experience• Data Collection_Pro_patients can help identify sources of information• Data Collection_Pro_patient experience allows better technology evaluation• Design_Pro_Allows better definition of (sub)populations• Reporting_Condition_If only at the end of reporting phase to confirm the presentation of the results• Selection_Pro_patient organisations as information source to help with specific patient related issues
Extensiveness	Selection_Condition_If not for all projects, maximum one third
Feasibility	<ul style="list-style-type: none">• Analysis_Contra_Unclear how to involve patients in this stage• Analysis_Contra_patients might be dominant in meetings• Diss_Condition_If we create the conditions to do it in house• Recommendations_Pro_Patients are reality check for recommendations
Impact on KCE procedures and resources	<ul style="list-style-type: none">• Analysis_Condition_If added value is evaluated• Analysis_Pro_Feedback to patients in case of qualitative research• Call_Condition_If call is accessible to patients into language and submission requirements• Call_Condition_If something is actually done with patient proposals• Call_Conditions_if call effectively reaches patients or patient organisations and patients are encouraged to submit• Call_Pro_Allows to favour topics with low prevalence• Call_Pro_Avoids dealing with only exclusively scientific topics• Call_Pro_patients can add relevant selection criteria• Data Collection_Condition_If KCE expert with competences in patient communication, recruitment and persuasion is available and can help in preparing patient involvement• Data Collection_Pro_Diversification of methods• Design_Pro_Allows the testing of different data collection methods• Diss_Pro_Creation of patient summaries• Scoping_Condition_If patients and experts are treated as separate groups• Scoping_Condition_If researchers can still decide what to do with patient info• Scoping_Condition_If their role and expected input is explained



	<ul style="list-style-type: none"> • Selection_Condition_If new selection criterion is introduced • Selection_Pro_patients can ensure patients are involved during project
Implementation	Scoping_Pro_Increase in implementation after the project
Innovation	<ul style="list-style-type: none"> • Design_Pro_Learn from patients (organisations) experience with surveys or other data collection methods • Selection_Pro_discovery of new scoring methods
Ownership	<ul style="list-style-type: none"> • Diss_Pro_Better ownership over the message and consequently effectiveness of dissemination and implementation of results/recommendations • Diss_Pro_patients pay for it (public money) • Recommendations_Pro_Allows to explain patients the reason for a recommendation, reducing the paternalisation in medicine • Selection_Pro_patients pay (taxes)
Patient education	Design_Pro_patients learn about our methods
Patients' knowledge and competences	<ul style="list-style-type: none"> • Analysis_Contra_Risk of misinterpretation by lack of knowledge • Design_Condition_If not to define the methods • Design_Condition_If patients are trained to be involved • Design_Contra_Patients lack scientific knowledge • Design_Contra_Patients do not know where to collect data • Design_Pro_Patients know where to search for information • Diss_Pro_To make sure patients receive the information • Recommendations_Contra_Patients are insufficiently familiar with the politics behind the recommendations (i.e. the broader context) • Scoping_Contra_Patients lack scientific knowledge • Selection_Condition_If patients are aware of the budget • Selection_Contra_Patients have no idea of budgetary aspects of a study • Selection_Contra_Patients cannot correctly score topic proposals • Selection_Contra_Some topics are not understandable for patients (e.g. HTA)
Priority setting	<ul style="list-style-type: none"> • Call_Contra_Risk of too many topics • Reporting_Pro_Patients can make sure patient-relevant key points are reported • Selection_Pro_allows bottom-up prioritisation



Quality of the research	<ul style="list-style-type: none">• Call_Condition_if patients get assistance from KCE or patient organisations to ensure eligibility/quality of the submission• Design_Condition_If the choice of the method does not impact the results• Design_Contra_Treat for scientific independence• Recommendations_Condition_If patients have read the report and received additional explanations• Scoping_Contra_Threat of overambitious scoping• Scoping_Pro_Certain problems can be avoided by including P
Representativeness	<ul style="list-style-type: none">• Analysis_Condition_If patients have consulted minimum X other patients• Call_Condition_If patient organisations or sickness funds are really representing the patients concerned• Call_Contra_KCE should not privilege one stakeholder group• Data Collection_Contra_Potential introduction of bias due to conflict of interest or lack of representativeness• Design_Condition_If always the same patient group is involved• Design_Condition_If selection of patients is based on the same procedure as for subcontractors• Design_Contra_Treat for representativeness• Diss_Pro_P are the recipients/target group of the messages• Recommendations_Condition_If clear determination of who will be involved and how• Scoping_Contra_Patient group is heterogeneous• Scoping_Contra_KCE should not privilege one stakeholder group• Selection_Condition_If clear which patients should be chosen• Selection_Condition_If representative of a sufficiently large patient population
Social capital	<ul style="list-style-type: none">• Call_Pro_Allows to broaden dissemination of call to specific networks• Data Collection_Pro_Patient involvement might facilitate access to patients as respondent
Subjectivity	<ul style="list-style-type: none">• Call_Contra_Patients confuse micro and macro problems• Design_Contra_Real-life experience is not objective• Scoping_Contra_Risk of complicating meetings because of patients' emotions
Time issues	<ul style="list-style-type: none">• Analysis_Contra_Time consuming• Call_Contra_More work for KCE experts to complete patients' proposal• Data Collection_Condition_If researchers get sufficient time• Data Collection_Contra_Time consuming• Design_Contra_Time consuming• Design_Pro_The more patient involvement, the less time consuming it becomes• Scoping_Contra_Time consuming


Visibility and image building

- Call_Pro_Positive image building for KCE
- Diss_Pro_Increasing visibility of KCE or project
- Diss_Pro_To strengthen the image of KCE as a public service

** The nodes are named as follows: the first part refers to the research phase (e.g. call, diss (=dissemination), analysis...); the second part refers to whether it relates to an argument pro or contra patient involvement or to a condition for patient involvement: the third part describes the content of the argument or condition.*

Appendix 4.2. Full list of arguments for or against patient involvement and conditions for patient involvement in different phases of the

	Call	Selection	Scoping	Design	Data collection	Analysis	Reporting	Recommendations	Dissemination
Acceptability							Pro_P can enhance acceptability of reports		
Adequate language use			Pro_Patients can check appropriateness of wording of instruments		Pro_P can explain the logic of questionnaires better		Contra_Vulgarisation no use in reporting phase	Pro_To make sure formulations are comprehensible for patients	Condition_If adapted communication to P
					Pro_P can train researchers to use appropriate wording		Pro_P can help to report at P level		
Anonymity	Condition_If anonymity is respected	Contra_threat for anonymity of the proposals							
	Contra_Use of a specific P form might allow								

	identification of submitter								
Appropriateness	Contra_Call should target policy makers (only)	Contra_Negative experience with stakeholder involvement in the past	Condition_If P organisation which have expertise on the topic	Condition_If not in quantitative designs	Condition_If not for each project	Condition_If P involvement is a free choice in function of the project		Contra_P are rarely a direct target group for the recommendations	Condition_If relevant
			Condition_only for patient related topics	Condition_Only if data collection in patients				Pro_easy to involve P in this stage	Contra_Creation of P fiches not always possible
Complementarity		Pro_Completeness - P highlight aspects not identified by scientists	Contra_Potential clash between patients' and decision makers' needs			Condition_If P are not the only source of information		Pro_Allows recom to go beyond the purely medical aspects	Contra_Possible clash between KCE and P organisations' recommendations
			Pro_P provide unique information			Pro_patients can help to interpret findings			
Conflict of interest		Condition_If it can be ensured that P are not driven by commercial or industrial interests			Condition_If P conflicts of interest are screened	Condition_If professional activity of P is taken into account		Condition_If openness about conflict of interest of P	



		Contra_P have a conflict of interest		Contra_P might be to focussed on own situation (miss broader perspective)		Contra_P have a conflict of interest			
Credibility		Pro_Selection procedure will be taken more seriously		Contra_reduces scientific credibility		Contra_reduces scientific rigour of the report		Pro_P are more credible than researchers	
Early in the research process	Pro_Good to involve P from the start of the research process	Condition_If also involved in other research phases		Condition_If P are involved from the beginning of the research process	Condition_If involved from the beginning		Condition_Only if P have been involved in previous phases		
Ethics		Condition_If also other stakeholders are involved		Condition_if P exhaustion is taken into account		Contra_KCE should not privilege one stakeholder group	Contra_Risk of frustrated P if recom is not implemented		
								Pro_P is affected stakeholder like any other	
Experts by experience	Pro_Identificati on of gaps or needs informed by experience	Pro_P organisations as information source to help with specific patient related issues		Pro_Allows better definition of (sub)population s	Pro_P can help identify sources of information	Condition_If P are mainly involved in interpretation, not in analysis itself	Condition_If only at the end of reporting phase to confirm the presentation of the results		



					Pro_P experience allows better technology evaluation	Pro_P can define main variables <i>and clinical significance levels</i>	
						Pro_Patients' feedback on crude results allows refinement of analysis	
Extensiveness		Condition_If not for all projects, max one third					
Feasibility						Contra_Unclear how to involve P in this stage	Pro_P are reality check for recom
							Condition_if we create the conditions to do it in house
						Contra_P might be dominant in meetings	
Identification of needs							
Impact on KCE procedures	Condition_If call is accessible to patients ito language and submission requirements	Condition_If new selection criterion is introduced	Condition_If patients and experts are treated as seperate groups	Pro_Allows the testing of different data collection methods	Condition_If KCE expert with competences in P communication, recrutement and persuasion is available and can help in	Condition_If added value is evaluated	Pro_Creation of P summaries



preparing P
involvement

Condition_If something is actually done with P proposals	Pro_P can ensure P are involved during project	Condition_If researchers can still decide what to do with P info	Pro_Diversification of methods	Pro_Feedback to P in case of qualitative research
Conditions_if call effectively reaches patients or P organisations and P are encouraged to submit		Condition_If their role and expected input is explained		
Pro_Allows to favour topics with low prevalence				
Pro_Avoids dealing with only exclusively scientific topics				
Pro_P can add relevant selection criteria				



Implementati on		Pro_Increase in implementabilit y after the project		
Innovation	Pro_discovery of new scoring methods		Pro_Learn from P (organisations) experience with surveys or other data collection methods	
Ownership	Pro_P pays (taxes)			Pro_Allows to explain P the reason for a recom, reducing the paternalisation in medicine
				Pro_Better ownership over the message and consequently effectiveness of dissemination and implementation of results/recomm endations
				Pro_P pay for it (public money)
Patient education			Pro_P learn about our methods	



Patients' knowledge and competences	Condition_If P are aware of the budget	Contra_P lack scientific knowledge	Condition_If not to define the methods	Contra_Risk of misinterpretation by lack of knowledge	Contra_P are insufficiently familiar with the politics behind the recommendations (i.e. the broader context)	Pro_To make sure P receive the information
	Contra_P have no idea of budgetary aspects of a study		Condition_If P are trained to be involved			
	Contra_Patients cannot correctly score topic proposals		Contra_P lack scientific knowledge			
	Contra_Some topics are not understandable for patients (e.g. HTA)		Contra_P do not know where to collect data			
			Pro_P know where to search for information			
Priority setting	Contra_Risk of too many topics	Pro_allows bottom-up prioritisation			Pro_P can make sure patient-relevant key points are reported	




Quality of the research	Condition_if P get assistance from KCE or patient organisations to ensure eligibility/quality of the submission		Contra_Threat of overambitious scoping	Condition_If the choice of the method does not impact the results			Condition_If patients have read the report and received additional explanations	
			Pro_Certain problems can be avoided by including P	Contra_Treat for scientific independence				
Representativeness	Condition_If P organisations or sickness funds are really representing the P concerned	Condition_If clear which P should be chosen	Contra_P group is heterogenous	Condition_If always the same P group is involved	Contra_Potential introduction of bias due to conflict of interest or lack of representativeness	Condition_If P have consulted minimum X other patients	Condition_If clear determination of who will be involved and how	Pro_P are the recipients/target group of the messages
	Contra_KCE should not privilege one stakeholder group	Condition_If representative of a sufficiently large P population	Contra_KCE should not privilege one stakeholder group	Condition_If selection of P is based on the same procedure as for subcontractors				
					Contra_Treat for representativeness			



Social capital	Pro_Allows to broaden dissemination of call to specific networks			Pro_P involvement might facilitate access to P as respondent	
Subjectivity	Contra_Patients confuse micro and macro problems	Contra_Risk of complicating meetings because of P emotions	Contra_Real-life experience is not objective		
Time issues	Contra_More work for KCE experts to complete P proposal	Contra_Time consuming	Contra_Time consuming	Condition_If researchers get sufficient time	Contra_Time consuming
			Pro_The more P involvement_the less time consuming it becomes	Contra_Time consuming	
Visibility and image building	Pro_Positive image building for KCE				Pro_Increasing visibility of KCE or project
					Pro_To strengthen the image of KCE as a public service



APPENDIX 5. QUESTIONNAIRE ABOUT PAST AND ONGOING PATIENT INVOLVEMENT ACTIVITIES AT KCE



Section A: Who are you

A1. Please fill in your tryptic or name

A2. When did you start working on at KCE (year)?

2008 or before ☐

2009 ☐

2010 ☐

2011 ☐

2012 ☐

2013 ☐

2014 ☐

2015 ☐

2016 ☐

2017 ☐

2018 ☐

A3. Which type of projects have you been working at KCE (as team's member)?

EPB ☐

HSR ☐

HTA ☐

GCP ☐

Method ☐

KCE Trials ☐

A4. What is your main role on the KCE trials projects?

Member of the KCE trials team ☐

Member of the board ☐



Section B: KCE Reports

Patient involvement in research encompasses two distinct but complementary ways to strengthen research by taking patients' perspectives into account:

Involving patients to research patient aspects that are relevant for the study (patients' experiences, preferences, perspectives)

Involving patients in the research process (scoping, design)

We want to focus in this survey on *active* patient involvement for one of these purposes. Participation of patients in studies as 'study objects', e.g. as in a trial, is not considered. Whenever we talk about patient involvement, we mean consultation, active participation in discussions, and co-decision making. Just informing patients is not enough to be considered as involving patients.

Please indicate in which project(s) patients were actively involved, as defined above (only check the box if you also worked on the project and followed the patient involvement activities)

B1. Please indicate in which project(s) patients were actively involved, as defined above (only check the box if you also worked on the project and followed the patient involvement activities)

Adriaenssens, J., N. Benahmed, M. Eysen, D. Paulus and R. Mertens (2018). Towards an integrated evidence-based practice plan in Belgium – Part 1: literature, Belgian situation and end-user needs KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 291. ☐

Adriaenssens, J., M.-J. Parfan-Portet, N. Benahmed, L. Kohn, C. Dubois, S. Devienne, M. Eysen and C. Ricour (2018). How to improve the Organisation of Mental healthcare for older adults in Belgium? KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 301. ☐

Desomer, A., K. Van den Heede, M. Triemstra, J. Paget, D. De Boer, L. Kohn and I. Cleemput (2018). Use of patient-reported outcome and experience measures in patient care and policy. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 303. ☐

Eysen, M. and B. J. Sildken (2018). Towards an integrated evidence-based practice plan in Belgium – Part 4: EBP Implementation in primary health care in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 291. ☐

Hanquet, G., I. Vinck and N. Thiry (2018). The use of whole genome sequencing in clinical practice: challenges and organisational considerations for Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 300. ☐

Rogiers, S., B. Kokkeleir, K. Marichal, I. Omeny and M. Eysen (2018). Towards an integrated evidence-based practice plan in Belgium – Part 3: Professional leadership and change management as a catalyst for EBP Implementation. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 291. ☐

San Miguel, L., C. Dubois, S. Gerkens, J. Harrison and F. Huisman (2018). MammaPrint® test for personalised management of adjuvant chemotherapy decisions in early breast cancer. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 298. ☐

Sikken, B. J., B. Kokkeleir and M. Eysen (2018). Towards an integrated evidence-based practice plan in Belgium – Part 5: Performance management for EBP implementation in primary health care in Belgium KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 291. ☐

Stephani, V., W. Quentin, K. Van den Heede, C. Van de Voorde and A. Geisler (2018). Payment methods for hospital stays with a large variability in the care process. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 302. ☐

Vandenbroeck, P., W. Christiaens, M. Dautvin, R. Wickert, K. Becher, E. Hendrickx, J. Goossens, L. Jenaé and M. Eysen (2018). An evaluation protocol for NHTD conventions. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 299. ☐

Vinck, I., A. Vijverman, E. Vollebregt, N. Broeckx, K. Wouters, M. Pitt, N. Bacie, J. Vloeyen, N. Thiry and M. Neyt (2018). Responsible use of high-risk medical devices: the example of 3D printed medical devices. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 297. ☐

Vriesacker, K., B. Cambré, B. Kokkeleir, B. van der Kamp, H. de Goeij, A. Terrier, M. Eysen and J. Adriaenssens (2018). Towards an integrated evidence-based practice plan in Belgium – Part 2: Governance Plan KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 291. ☐

B2. Please indicate in which project(s) patients were actively involved, as defined above (only check the box if you also worked on the project and followed the patient involvement activities)

Adriaenssens, J., M. Eysen, R. Mertens, N. Benahmed, D. Paulus, F. Ameye, M. Bouens, C. Caenenberg, A. Coels, K. Doms, E. Eversart, M. Gheysen, M. Heilermans, M. Laloux, H. Malouac, P. Meun, W. Penninx, T. Rossin and M. Walmsley (2017). EBP Plan. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 291. ☐

Benahmed, N., J. Adriaenssens, W. Christiaens and D. Paulus (2017). Towards tailoring of KCE guidelines to end-users' needs. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 284. ☐

Parfan-Portet, M.-J., C. Dubois, P. Mistiaen, A. Cordon, S. Stordeur and K. Van den Heede (2017). Towards an inclusive system for major trauma. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 281. ☐

Gerkens, S., M. Neyt, L. San Miguel, I. Vinck, N. Thiry and I. Cleemput (2017). How to improve the Belgian process for Managed Entry Agreements? An analysis of the Belgian and international experience. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 288. ☐

Jose Boer, P., A. Desreux, B. Dupont, A. Breugnot, M. Brussa, W. Christiaens, E. Cloeckhuyse, C. Donsaert, P. Dupont, P. Fagot, Y. Fauts, L. Gohlis, G. Huis, D. Huis, L. Kohn, P. Marquet, E. Meunier, H. Nuyens, T. Omeny, T. Pabst, B. Pabst, K. Van Broeck, P. Van Leuven, P. Van Leuven, P. Van Leuven, J. Van Leuven, J. Van Leuven, K. Van Leuven and D. Verhaeghe (2017). Low back pain and radicular pain: development of a clinical pathway. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE), 295. ☐



- Lepage-Nekens, I., K. Douw, G. Mantjes, G. de Graaf, R. Leroy and I. Cleemput (2017). Horizon scanning for pharmaceuticals: proposal for the BeNeLuxA collaboration. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 283.
- Leroy, R., C. Camberlin, M. Lefevre, P. Mistiaen, K. Van den Heede, S. Van De Sande, C. Van de Voorde and C. Beguin (2017). Proposals for a further expansion of day surgery in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 282.
- Mistiaen, P., M. Dauvrin, M. Eysen, D. Roberfroid, L. San Miguel and I. Vinck (2017). Health care in Belgian prisons. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 293.
- Neyt, M., S. Devriese, C. Camberlin and J. Vlayen (2017). Bevacizumab in the treatment of ovarian cancer. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 285.
- Oblyn, C., A. Cordon, L. Kohn, C. Devos and C. Léonard (2017). Exploratory steps for the formulation of Belgian health system targets. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 292.
- Van Brabandt, H., S. Gerkens, N. Fairon, C. Dubois, M. Van Der Auwer, C. Vansimpnen and D. Roberfroid (2017). Static automated external defibrillators for opportunistic use by bystanders. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 294.
- Van Brabandt, H., L. San Miguel, N. Fairon, B. Vaes, S. Henrard, A. Boshnakova, R. Cook, R. Davies, A. Karnad, A. Lovell and C. Dubois (2017). Anticoagulants in non-valvular atrial fibrillation. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 279.
- Van de Voorde, C., K. Van den Heede, C. Beguin, N. Bouckaert, C. Camberlin, P. de Bekker, N. Defoornay, J. De Schutter, C. Devos, S. Gerkens, C. Graw, P. Jeurissen, P. M. Kruse, M. Lefevre, Y. Laveau, P. Marissen, A. Vlaendering, E. Van Eycken and E. Van Gansbeke (2017). Required hospital capacity in 2025 and criteria for rationalization of complex cancer surgery, radiotherapy and nursing services. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 309.
- Van den Broeck, K., O. Schmitz, I. Aujoulat, P. Mistiaen, M. Friedel, M. Genet, C. Ricour, L. Kohn and J. Wens (2017). Appropriate care at the end of life. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 296.
- Vinck, I., P. A. Desomer, L. Ailliet, A. Berquin, C. Desmet, B. Depoorter, J. Desautels, M. Dujols, P. Fagard, Y. Friauf, G. Hain, D. Hoste, G. Mollen, J. Michielens, H. Niens, T. Orban, T. Pulevicius, E. Simons, Y. Tobback, J. Van Zandert, P. Vanschebroeck, J. Vanderschueren, J. Vlayen and P. Jonckheer (2017). Low back pain and radicular pain: assessment and management KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 287.
- Vandenbroeck, P., K. Ooms, N. Benahmed, R. Wickert, W. Christiaens and S. Stordeur (2017). Strategies for improving the medical workforce projection model: a stakeholder consultation. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 290.
- Veerman, G., H. Mohammad Nadja, M. Van Leeuwen, R. Scholten and H. Van Brabandt (2017). Management of pancreatic cancer – Part 3: neoadjuvant and induction therapy. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 286.
- Veerman, G., H. Mohammad Nadja, M. Van Leeuwen, R. Scholten and H. Van Brabandt (2017). Management of pancreatic cancer – Part 4: recurrent and metastatic cancer. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 286.
- Veerman, G., H. Mohammad Nadja, M. Van Leeuwen, R. Scholten and H. Van Brabandt (2017). Management of pancreatic cancer: capita selecta – Part 1: Introduction and methodology. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 286.
- Vlayen, J., N. Benahmed and J. Robays (2017). Routine preoperative testing in adults undergoing elective non-cardiothoracic surgery. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 280.
- B3. Please indicate in which project(s) patients were actively involved, as defined above (only check the box if you also worked on the project and followed the patient involvement activities)**
- Benahmed, N., E. Hendrickx, J. Adriaenssens and S. Stordeur (2016). Health workforce planning and midwifery-specific data. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 278.
- Bloomaert, A., G. Hanquet, L. Willem, H. Theeten, N. Thiry, J. Bilcke, J. Verhaegen and P. Beutels (2016). Use of pneumococcal vaccines in the elderly: an economic evaluation. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 274.
- Christian, L., M. Catherine, M. Xavier, V. Ingard, E. Nicolas and S. Sabine (2016). Financial compensation for persons infected by a contaminated blood transfusion – an attempt to reduce discriminations. Brussels, Belgian Health Care Knowledge Centre (KCE).
- Cleemput, I., S. Devriese, W. Christiaens and L. Kohn (2016). Multi-criteria decision analysis for the appraisal of medical needs: a pilot study. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 272.
- De Laet, C., G. Hanquet and E. Hendrickx (2016). Multi criteria decision analysis to select priority diseases for Newborn blood screening. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 267.
- De Pourcq, K., M. De Regge, S. Callens, M. Coëffé, P. Gemmel, K. Van den Heede, C. Van de Voorde and K. Eeckloo (2016). Governance models for hospital collaborations. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 277.
- Desomer, P., P. Mistiaen and M. Eysen (2016). Belnet suite of instruments: an exploratory study on applicability for individual care planning and budget allocation in rehabilitation care. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 262.
- Devriese, S. and C. Van de Voorde (2016). Clustering pathology groups on hospital stay similarity. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 270.
- Gerkens, S., N. Thiry, F. Hulstaert and J. Robays (2016). Economic evaluation of novel direct acting antiviral (DAA) treatment strategies for chronic hepatitis C. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 276.
- Kohn, L., C. Oblyn, J. Adriaenssens, W. Christiaens, X. Van Cauter and M. Eysen (2016). Model for the organization and reimbursement of psychological and orthopedagogical care in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 265.
- Léonard, C., C. Mélotte, X. Marechal, I. Vinck, N. Bistieme and S. Stordeur (2016). Financial compensation for persons infected by a contaminated blood transfusion – an attempt to reduce discriminations. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 273.



- Mistiaen, P., L. Kohn, P. Mambourg, F. Kernerer, C. Tiedtke, M.-C. Lambrechts, L. Godderis, M. Vanmeerbeek, M. Eysen and D. Paulus (2016). Reduction of the treatment gap for problematic alcohol use in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 258.
- Neyt, M., R. Leroy, C. Devos and H. Van Brabandt (2016). Left ventricular assist devices in the treatment of end-stage heart failure. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 264.
- Peters, M., E. Van Cuiem, D. Bielen, A. Boh, P. Denetier, A. D'Hoeke, K. Hausmans, A. Hendrix, A. Lemmers, D. Leonard, F. Penninckx, N. Fairon, J. Robays, K. Holdt Hemmingsen, J. Vlayen and G. Veerman (2016). Guideline on the management of rectal cancer: update of capita selecta – Part 1: Introduction and Methodology. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 260.
- Peters, M., E. Van Cuiem, D. Bielen, A. Boh, P. Denetier, A. D'Hoeke, K. Hausmans, A. Hendrix, A. Lemmers, D. Leonard, F. Penninckx, N. Fairon, J. Robays, K. Holdt Hemmingsen, J. Vlayen and G. Veerman (2016). Guideline on the management of rectal cancer: update of capita selecta – Part 2: Staging. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 260.
- Peters, M., E. Van Cuiem, D. Bielen, A. Boh, P. Denetier, A. D'Hoeke, K. Hausmans, A. Hendrix, A. Lemmers, D. Leonard, F. Penninckx, N. Fairon, J. Robays, K. Holdt Hemmingsen, J. Vlayen and G. Veerman (2016). Guideline on the management of rectal cancer: update of capita selecta – Part 3: Local vs Radical resection for stage I tumours. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 260.
- Peters, M., E. Van Cuiem, D. Bielen, A. Boh, P. Denetier, A. D'Hoeke, K. Hausmans, A. Hendrix, A. Lemmers, D. Leonard, F. Penninckx, N. Fairon, J. Robays, K. Holdt Hemmingsen, J. Vlayen and G. Veerman (2016). Guideline on the management of rectal cancer: update of capita selecta – Part 4: Adjuvant therapy after neoadjuvant therapy. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 260.
- San Miguel, L., N. Benahmed, C. Devos, N. Fairon and D. Roberfroid (2016). The role of biomarkers in ruling out cerebral lesions in mild cranial trauma. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 261.
- Stordeur, S., P. Jonckheer, N. Fairon and C. De Laet (2016). Elective caesarean section in low-risk women at term: consequences for mother and offspring. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 275.
- Van den Heede, K., C. Dubois, S. Devriese, N. Baier, O. Camaly, E. Depuydt, A. Geisler, A. Ghesquiere, S. Mispion, W. Quentin, C. Van Loon and C. Van de Voorde (2016). Organisation and payment of emergency care services in Belgium: current situation and options for reform. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 263.
- Vandenbroeck, P., P. Raeymakers, R. Wickert, K. Becher, J. Goossens, I. Cleemput, F. Hulstaert, L. de Heji and R. Mertens (2016). Future scenarios about drug development and drug pricing. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 271.
- Verhote, J., J. Vlayen, P. Heus, J. P. Hoogenland, J. A. A. G. Dams, P. Van de Weerting, P. Van der Buijn, C. Bourgain, J. De Groot, D. Debruyne, M. Paturel, F. Goffin, M. Houting, J. Kuper, J. Korf, S. Koster, S. Koster, W. Tjalling, P. Van Duijn, Y. Van de Geyse, G. Vliegenhart, N. Fairon, R. P. Zwemmer, L. Hoek, R. Scholten and L. Verleye (2016). Ovarian cancer: diagnosis, treatment and follow-up. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 268.
- Vrijens, F., P. Renard, C. Camberlin, A. Desomer, C. Dubois, P. Jonckheer, K. Van den Heede, C. Van de Voorde, C. Léonard, D. Walckiers and P. Meun (2016). Performance of the Belgian Health System - Report 2015. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 259.
- Vrijens, F., L. Verleye, C. De Gucht, V. Schillema, J. Robays, C. Camberlin, C. Dubois, S. Stordeur, D. Jegou, G. Silverman, E. Van Eycken, I. Wauters and J. Van Meerbeek (2016). Quality indicators for the management of lung cancer. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 266.
- B4. Please indicate in which project(s) patients were actively involved, as defined above (only check the box if you also worked on the project and followed the patient involvement activities)**
- Arbyn, M., A. Haidich, A. Desomer, F. Verhoeft, J. Francart, N. Thiry, G. Hanquet and J. Robays (2015). Cervical cancer screening program and Human Papillomavirus (HPV) testing: part II. Update on HPV primary screening. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 238.
- Baeyens, H., C. Poupez, P. Slegers, I. Vinck, F. Hulstaert and M. Neyt (2015). Towards a guided and phased introduction of high-risk medical devices in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 249.
- Benahmed, N., J. Robays, S. Stordeur, T. Gil, S. Joniau, N. Lumen, L. Remat, S. Roivie, D. Schrijvers, B. Tombal, B. Van den Eynden, G. Villein and S. Rottey (2015). Renal cancer in adults: diagnosis, treatment and follow-up. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 253.
- De Laet, C., N. Thiry, K. Holdt Hemmingsen, S. Stordeur and C. Camberlin (2015). Percutaneous Vertebroplasty and Balloon Kyphoplasty. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 255.
- Deschodt, M., V. Chen, B. Van Groenou, K. Milten, B. Boland, J. Plamung, A. Denis, P. Daele, L. Meignier, C. Devos, P. Mistiaen and K. Van den Heede (2015). Comprehensive geriatric care in hospitals: the role of specialist consultation teams. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 245.
- Desomer, A., S. Gerkens, I. Vinck, C. Léonard, M. Neyt, D. Paulus and H. Van Brabandt (2015). Cardiovascular pre-participation screening in young athletes. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 241.
- Farfán-Portet, M.-L., A. Denis, L. Mergaert, F. Daue, P. Mistiaen and S. Gerkens (2015). Implementation of hospital at home: orientations for Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 250.
- Gerkens, S., S. Van de Sande, R. Leroy, A. S. Mertens, J. Schreiber, D. Van Halbeek, H. Van Brabandt, N. Swaenbroeck and C. Oblyn (2015). Autologous breast reconstruction techniques after mastectomy: time measurements for a potential reevaluation of the surgeon fee. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 251.
- Grégoire, V., L. Rook, H. Pauline, H. Lott, Y. d. W. F. T. R. Spijker, R. J. P. M. Scholten, J. Abkoud, L. Carp, J. Casteleijn, P. Clement, P. Denon, M. Hamon, O. Leusen, S. Nijts, C. Van Laer, J. Vermeulen and J. Vlayen (2015). Oropharyngeal, laryngeal and hypopharyngeal cancer: diagnosis, treatment and follow-up. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 256.
- Gysels, W., P. Jonckheer, N. Almadani, M. Asari, S. Caville, K. Dworzak, L. Gaudet, J. Gien, K. Jones, P. Miller, J. M. Tordella, S. Alexander, K. Alpert, K. Beecham, G. Ceylan, Y. Christiane, N. De Krom, B. De Teyssier, N. De Krom, A. Deryn, P. De Krom, A. Hernandez, E. Mollen, L. Seunig, J. L. Verleye and S. Stordeur (2015). What are the recommended clinical assessment and screening tests during pregnancy? KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 248.
- Holdt Hemmingsen, K., N. Thiry, C. De Laet, S. Stordeur and C. Camberlin (2015). Cervical and lumbar total disc replacements. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 254.
- Leroy, R., N. Benahmed, F. Hulstaert, P. Mambourg, N. Fairon, L. Van Eycken and D. De Ruyscher (2015). Hadron therapy in children – an update of the scientific evidence for 15 paediatric cancers. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 235.
- Neyt, M., T. Christiaens, J. Demotes and F. Hulstaert (2015). Publicly funded Practice-oriented Clinical Trials. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 246.
- Robays, J., S. Stordeur, F. Hulstaert, J.-F. Bourain, L. Brochez, T. Clapham, K. Chen, E. Legius, S. Rottey, D. Schrijvers, D. Kint de Roodenbeke, T. Van Maerke, U. Ullrich and B. Poppel (2015). Oncogenetic testing, diagnosis and follow-up in Birt-Hogge-Dubé syndrome, familial atypical multiple mole melanoma syndrome and acrofibromatosis 1 and 2. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 243.



Robays, J., S. Stordeur, F. Hulstaert, T. van Maerken, K. Chen, N. Jania, G. Matileji, D. Van der Roodenbeke, M. Beldère, H. Wildiers and B. Poppe (2015). Oncogenetic testing and follow-up for women with familial breast/ovarian cancer, Li Fraumeni syndrome and Cowden syndrome. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 236.	<input type="checkbox"/>
Roberfroid, D., M. Dauvin, I. Keyngaert, A. Desomer, B. Kerstens, C. Camberlin, J. Gysen, V. Loran and I. Derhuy (2015). What health care for undocumented migrants in Belgium? KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 257.	<input type="checkbox"/>
Roberfroid, D., L. San Miguel and D. Paulus (2015). Non-invasive markers of subclinical atherosclerosis for predicting a primary cardiovascular event: a rapid systematic review. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 244.	<input type="checkbox"/>
San Miguel, L., J. Vlayen and C. De Laet (2015). Gene expression profiling and immunohistochemistry tests for personalised management of adjuvant chemotherapy decisions in early breast cancer – a Rapid Assessment. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 237.	<input type="checkbox"/>
Van Brabant, H., A. Desomer and B. Tombal (2015). Bladder cancer: an assessment of international practice guidelines. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 247.	<input type="checkbox"/>
Van den Bakker, M., L. San Miguel, R. Salgado, E. De Queckez, H. De Schutter, A. Wuytens, P. Van Den Bergh, S. Tejpar, J. Van Houth, S. Via Lare, B. Mies and F. Hulstaert (2015). Next generation sequencing gene panels for targeted therapy in oncology and haemato-oncology. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 240.	<input type="checkbox"/>
Vannieuwenhuysen, C., P. Slegers, M. Neyt, F. Hulstaert, S. Stordeur, I. Cleemput and I. Vinck (2015). Towards a better managed off-label use of drugs KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 252.	<input type="checkbox"/>
Vlayen, J., M. Bex, B. Bravenboer, K. Chao, B. Lapauw, A. Pennu, K. Poppe, U. Ullmann, T. Van Maerken, L. Vroonen and B. Poppe (2015). Oncogenetic testing for persons with hereditary endocrine cancer syndromes. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 242.	<input type="checkbox"/>
Vrijens, F., L. Kohn, C. Dubois, R. Leroy, I. Vinck and S. Stordeur (2015). Ten years of multidisciplinary teams meetings in oncology: current situation and perspectives. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 239.	<input type="checkbox"/>
B5. Please indicate in which project(s) patients were actively involved, as defined above (only check the box if you also worked on the project and followed the patient involvement activities)	
Anthierens, S., B. Willenue, R. Remmen, O. Schnitz, J. Macq, A. Deckerq, C. Arnant, M. Forest, A. Denis, I. Vinck, N. Defourny and M.-J. Farfan-Portet (2014). Support for informal caregivers – an exploratory analysis. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 233.	<input type="checkbox"/>
Benahmed, N., C. Devos, L. San Miguel, I. Vinck, L. Vankelst, E. Lauwetter, M. Verschueren, C. Obyn, D. Paulus and W. Christiaens (2014). Caring for mothers and newborns after uncomplicated delivery: towards integrated postnatal care. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 232.	<input type="checkbox"/>
Cleemput, I., S. Devriese, L. Kohn, C. Devos, J. van Til, K. Groothuis-Oudshoorn, v. Vandekerckhove and C. Van de Voorde (2014). Incorporating societal preferences in reimbursement decisions - Relative importance of decision criteria according to Belgian citizens KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 254.	<input type="checkbox"/>
Grisque, V., R. Leroy, P. Heus, P. Van de Wetering, R. Scholten, L. Verleye, L. Culp, P. Clement, P. Doron, K. Griffin, M. Hendrickx, R. Hermans, S. Kuz, O. Lemmens, S. Neyts, C. Van Laer, J. Vermeiren, E. Appermont, A. De Prim, E. Hebelhuck, G. Hommez, C. Vandenbroucke, E. Vandalewyck and J. Vlayen (2014). Oral cavity cancer: diagnosis, treatment and follow-up. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 227.	<input type="checkbox"/>
Huquet, G., H. Christensen, E. Agnew, C. Trotter, J. Robays, C. Dubois, S. Devriese, S. Van De Sande and N. Thiry (2014). A quadrivalent vaccine against serogroup B meningococcal disease: a cost-effectiveness study. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 231.	<input type="checkbox"/>
Hulstaert, F., M. Neyt and W. Gyselaers (2014). The non-invasive prenatal test (NIPT) for trisomy 21 – health economic aspects. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 222.	<input type="checkbox"/>
Kayotaki, E., Y. Smit, P. Cuijpers, M. Debauche, T. De Keyser, H. Habraken, W. Pichot, F. Raes, D. Salomez, B. Gillan, N. Fauron, D. Paulus, J. Robays and H. K. Holdt (2014). The long-term efficacy of psychotherapy, alone or in combination with antidepressants, in the treatment of adult major depression KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 230.	<input type="checkbox"/>
Kohn, L., F. Mambourg, J. Robays, M. Albertijn, S. Janssens, C. Hoefnagels, M. Rommans and P. Jonckheer (2014). Informed choice on breast cancer screening: messages to support informed decision. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 216-149 p.	<input type="checkbox"/>
Mambourg, F., L. Kohn, J. Robays, S. Janssens, M. Albertijn and C. De Laet (2014). A decision aid for an informed choice when patient asks for PSA screening. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 224.	<input type="checkbox"/>
Pesters, M., R. Leroy, J. Robays, G. Veremmen, D. Bielen, W. Coelen, E. Dancie, M. De Man, P. Demetter, P. Phlumen, A. Hendrix, J. Simpi, D. Vanbeekroon, E. Via Cunen, Y. Dijk, P. van Gils, L. Verbeek, Y. Smit and L. Verleye (2014). Colon Cancer: Diagnosis, Treatment and Follow-Up. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 218-194 p.	<input type="checkbox"/>
Robays, J. and B. Poppe (2014). Oncogenetic testing for Lynch syndrome and familial adenomatous polyposis KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 220.	<input type="checkbox"/>
Rodens, K., D. Roberfroid, N. Akmadzi, M. Anst, K. Singh, L. Gaudet, S. Alexander, P. Coud, B. de Thyssens, P. Elmont, G. Pison, W. Gyselaers, C. Kirkpatrick, L. Lewi, H. Logghe, A. Niset, V. Riga, I. Tenoy, B. Van Oremette and L. Verleye (2014). Prevention of preterm birth in women at risk: selected topics. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 228.	<input type="checkbox"/>
San Miguel, L., D. Roberfroid, S. Stordeur and N. Swaertenbroeck (2014). Machine perfusion in kidneys from deceased donors – a rapid assessment. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 217-55 p.	<input type="checkbox"/>
Stordeur, S., F. Vrijens, K. Henau, V. Schillemans, C. De Gendt and R. Leroy (2014). Organisation of care for adults with a rare or complex cancer KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 219-130 p.	<input type="checkbox"/>
Tombal, B., A. Desomer, P. Jonckheer, G. Veremmen, C. D'Hont, R. Van Velthoven, A. Peysers, D. Schijvers, T. Gil, L. Renard, G. De Meirleer, S. Rorive, B. Spinaewijn, A. Sereva, N. Van Damme and H. Van Brabant (2014). National practice guideline on the treatment of localised prostate cancer – part 2. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 226.	<input type="checkbox"/>
Van de Voorde, C., K. Van den Heede, R. Mertens, L. Anemaert, R. Bane, S. Chidens, G. Durant, J. Gierman, J. Kip, B. Kreg, J. Magnussen, M. Pison, W. Quentin and W. Sermet (2014). Conceptual framework for the reform of the Belgian hospital payment system. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 229.	<input type="checkbox"/>
Veremmen, G., K. Holdt Henningsen, M. Bynen, N. Benahmed, W. Christiaens, M. H. Bouchet, A. De Roock, N. Doornick, G. De Ijpe, O. Devine, T. Ouyens, M. Hendrix, C. Kip, G. Magnussen, M. Moens, H. Roeyers, S. Schellekens, M. V. Sereva, S. Sereva, J. Sereva, F. Talle, G. Vinck, A. Wuytens, S. Wuytens and J. C. Schobben (2014). Management of autism in children and young people: a good clinical practice guideline. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 233.	<input type="checkbox"/>



Vinck, I. and D. Paulus (2014). Correction of refractive errors of the eye in adults – part 3: organisation and legal framework of extramural surgery centres. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 225.	<input type="checkbox"/>
Vlayen, J., K. Holdt Henningsen, O. d'Archembent, I. Fournau, B. Heyndrickx, P. Kohn, V. Maleux, S. Motte, M. Springer, F. Vermeiren, C. Sharpin, J. Nelson, M. Westly, S. Caville and K. Jones (2014). Revascularization for lower limb peripheral arterial disease. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 221.	<input type="checkbox"/>
B6. Please indicate in which project(s) patients were actively involved, as defined above (only check the box if you also worked on the project and followed the patient involvement activities)	
Beeckman, D., C. Mathet, A. Van Laecker, S. Van Houdt, G. Vanvalleghem, L. Gryson, H. Heyman, C. Thyse, A. Toppets, S. Stordeur and K. Van den Heede (2013). A National Guideline for the prevention of pressure ulcers. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 193.	<input type="checkbox"/>
Beeckman, D., C. Mathet, A. Van Laecker, G. Vanvalleghem, S. Van Houdt, L. Gryson, H. Heyman, C. Thyse, A. Toppets, S. Stordeur and K. Van den Heede (2013). A national guideline for the treatment of pressure ulcers. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 203-119 p.	<input type="checkbox"/>
Boudts, P., Y. Vandendijck, L. Willem, N. Geyvaert, A. Blommaert, K. van Kerckhove, J. Bickel, G. Huquet, P. Neels, N. Thiry, J. Lienenborgh and N. Hens (2013). Seasonal influenza vaccination: prioritizing children or other target groups? - Part II: cost-effectiveness analysis. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 201-247 p.	<input type="checkbox"/>
Camberlin, C., C. Dabois, T. Di Zazzo, R. Prêst, J. Gailhaume, R. Van de Reydt, C. Van de Voorde, S. Van De Sande and S. Devriese (2013). (Coupling of the Permanent Sample with the Hospital Data: Feasibility and data representativeness study). KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 208.	<input type="checkbox"/>
Christiaens, W., L. Kohn, C. Obyn, L. De Winter, S. Gussé, N. Defourny, C. De Laet and D. Paulus (2013). Correction of refractive errors of the eye in adults - Part 1: Perceptions and experiences. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 202-98 p.	<input type="checkbox"/>
Christiaens, W., E. Van de Walle, S. Devriese, D. Van Halewyck, C. Dubois, N. Benahmed, A. Desomer, S. Van de Sande, N. Van Loey, D. Paulus and K. Van den Heede (2013). Organisation of a service for patients with severe burn injuries KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 209-107 p.	<input type="checkbox"/>
De Laet, C., K. Van Den Heede and R. Mertens (2013). General framework for a multidisciplinary quality manual for cardiac care networks. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 213-16 p.	<input type="checkbox"/>
Desomer, A., T. Dilles, S. Steckel, C. Duchesnes, M. Van Meerbeek, L. Peremans, B. Van Rompey, R. Remmen and D. Paulus (2013). Dissemination and implementation of clinical practice guidelines in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 212-164 p.	<input type="checkbox"/>
Eysen, M., N. Benahmed and A. Desomer (2013). Supportive treatment for cancer – Part 3: Treatment of pain: most common practices. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 211-173 p.	<input type="checkbox"/>
Eysen, M., M. Neyt, I. Vinck and F. Hulstaert (2013). Extremity-only MRI. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 205-34 p.	<input type="checkbox"/>
Hulstaert, F., A.-S. Mertens, C. Obyn, D. Van Halewyck, B. van der Straten and Y. Lievens (2013). Innovative radiotherapy techniques: a multicentre time-driven activity-based costing study. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 198-75 p.	<input type="checkbox"/>
Jonckheer, P., S. Van Landeghem, W. Christiaens, L. De Winter, J. Piérart and R. Mertens (2013). The decisional process for the choice of active surveillance in localized prostate cancer. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 210-57 p.	<input type="checkbox"/>
Lapage-Nekens, I., S. Gerkens, I. Vinck, J. Piérart, F. Hulstaert and M.-J. Farfan-Portet (2013). Barriers and opportunities for the uptake of biomedicine medicines in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 199-147 p.	<input type="checkbox"/>
Obyn, C., Y. Smit, P. Post, L. Kohn, N. Defourny, W. Christiaens and D. Paulus (2013). Correction of refractive errors of the eye in adults – Part 2: laser surgery and intraocular lenses KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 215-127 p.	<input type="checkbox"/>
Roberfroid, D., L. San Miguel and N. Thiry (2013). Novel serum biomarkers for the prediction of cardiovascular risk. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 201-100 p.	<input type="checkbox"/>
Roosen, P., T. Willems, R. De Ridder, L. San Miguel, K. Holdt Henningsen, D. Paulus, A. De Sutter and P. Jonckheer (2013). Ankle sprains: diagnosis and therapy. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 197.	<input type="checkbox"/>
Van de Sande, S., N. Swaertenbroeck, C. Van de Voorde, C. Devos and S. Devriese (2013). Evolution of day care: impact of financing and regulations. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 192.	<input type="checkbox"/>
Van de Voorde, C., S. Gerkens, K. Van den Heede and N. Swaertenbroeck (2013). A comparative analysis of hospital care payments in five countries. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 207-130 p.	<input type="checkbox"/>
Vinck, I., M. Lona and N. Swaertenbroeck (2013). Impact of the KCE reports published in 2009-2011. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 214-40 p.	<input type="checkbox"/>
Vlayen, J., C. De Gendt, S. Stordeur, V. Schillemans, C. Camberlin, F. Vrijens, E. Van Eycken and T. Lerut (2013). Quality indicators for the management of upper gastrointestinal cancer. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 200-351 p.	<input type="checkbox"/>
Wauters, L., J. Robays, L. Verleye, K. Holdt Henningsen, F. Hulstaert, T. Berghmans, W. De Weert, Y. Lievens, P. Pauwels, S. Stroobans, P. Van Houtte, J. Van Meerbeek, P. Van Schil, B. Weynaert and J. De Giebre (2013). Non-Small Cell Lung Cancer: Diagnosis, Treatment and Follow-up. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 206-212 p.	<input type="checkbox"/>
Wildiers, H., S. Stordeur, J. Vlayen, R. Scholten, F. van de Wetering, C. Bourgain, B. Carly, M.-R. Christiaens, V. Cocquyt, E. Lefrange, J.-C. Schobben, M. Van Goethem, G. Villiers, E. Van Limbergen and P. Neven (2013). Breast cancer in women: diagnosis, treatment and follow-up. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 143-183 p.	<input type="checkbox"/>
B7. Please indicate in which project(s) patients were actively involved, as defined above (only check the box if you also worked on the project and followed the patient involvement activities)	
Camberlin, C., L. San Miguel, J. Smit, P. Post, S. Gerkens and C. De Laet (2012). Neuromodulation for the management of chronic pain: implanted spinal cord stimulators and intrathecal analgesic delivery pumps. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 189-VI, 258 p.	<input type="checkbox"/>
Cardoso, F., S. Stordeur, J. Vlayen, C. Bourgain, B. Carly, M.-R. Christiaens, V. Cocquyt, E. Lefrange, P. Neven, P. Scalliet, J.-C. Schobben, M. Van Goethem and G. Villiers (2012). Scientific update of the College of Oncology: update of the national guidelines on breast cancer. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 143-XIII, 199 p.	<input type="checkbox"/>



- Christiaens, W., L. Kohn, C. Léonard, A. Denis, F. Daue and I. Cleemput (2012). Models for citizen and patient involvement in health care policy - Part 1: exploration of their feasibility and acceptability. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 195. 85 p. ☐
- Cleemput, I., C. Devos, S. Devriese, M.-I. Farfan-Portet and C. Van de Voorde (2012). Principles and criteria for the level of patient cost sharing: Reflections on value-based insurance. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 186. ☐
- Cleemput, I., M. Neyt, S. Van de Sande and N. Thiry (2012). Belgian guidelines for economic evaluations and budget impact analyses : second edition. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 183. VII. 76 p. ☐
- Farfan-Portet, M.-I., C. Devos, S. Devriese, I. Cleemput and C. Van de Voorde (2012). Simplification of patient cost sharing: the example of physician consultations and visits. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 180. V. 106 p. ☐
- Financ, J., A. Van Den Bruel, B. Decallonne, M. Adam, C. Dubois, H. De Schutter, J. Vlayen and S. Stordeer (2012). Regional differences in thyroid cancer incidence in Belgium: role of diagnostic and therapeutic strategies for thyroid disease. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 177. ☐
- Gerkens, S., N. Martin, N. Thiry and F. Hulstaert (2012). Hepatitis C : Screening and Prevention. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 173. ☐
- Holdt Henningsen, K., A. Desomer, S. Hanssens and J. Vlayen (2012). Supportive treatment for cancer : Part 1 - exercise treatment. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 185. VII. 121 p. ☐
- Kohn, L. and W. Christiaens (2012). The use of Qualitative Research Methods in KCE studies. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 187. IV. 89 p. ☐
- Mambourg, F., P. Jonckheer, J. Piérart and H. Van Brabant (2012). A national clinical practice guideline on the management of localised prostate cancer : - part 1. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 194. 102 p. ☐
- Mambourg, F., J. Robays and S. Gerkens (2012). Breast cancer screening amongst women aged 70-74 years of age. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 176. 51 p. ☐
- Michele, D., Y. Sun, V. Thijs, O. Saka Rasit, D. Hemelsoet, M. Eysen and D. Paulus (2012). Stroke units : efficacy, quality indicators and organisation. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 181. VII. 112 p. ☐
- Paulus, D., K. Van den Heede and R. Mertens (2012). Organisation of care for chronic patients in Belgium : development of a position paper. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 190. VI. 166 p. ☐
- Piérart, J., C. Léonard, P. X. Chalou, F. Daue and R. Mertens (2012). Stakeholder Involvement in KCE working processes. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 174. ☐
- Robays, J., G. Hanquet, D. Roberfroid and N. Thiry (2012). Vaccine safety surveillance in Belgium : place and limits of a background rate approach. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 188. ☐
- Stordeur, S. and M. Eysen (2012). Prophylactic removal of pathology-free wisdom teeth : rapid assessment. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 182. VI. 61 p. ☐
- Swartenbroeckx, N., C. Obyn, P. Guillaume, M. Lona and I. Cleemput (2012). Manual for cost-based pricing of hospital interventions. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 178. ☐
- Van Brabant, H., M. Neyt and C. Devos (2012). Catheter Ablation of Atrial Fibrillation. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 184. VI. 123 p. ☐
- Vandenbroeck, P., R. Dechenne, K. Becher, K. Van den Heede, M. Eysen, G. Goossens and B. Stroud (2012). The organisation of mental health services for children and adolescents in Belgium : development of a policy scenario. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 175. 120 p. ☐
- Verleye, L., A. Desomer, J. Gailly and J. Robays (2012). Identifying women at risk for breast cancer/technical methods for breast cancer screening. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 172. ☐
- Verleye, L., F. van de Weerting, P. Heus, R. Scholten and J. Vlayen (2012). Supportive treatment for cancer - Part 2: Prevention and management of adverse events related to chemotherapy and radiotherapy. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 191. VIII. 348 p. ☐
- Vrijens, F., F. Renard, P. Jonckheer, K. Van den Heede, A. Desomer, C. Van de Voorde, D. Walckiers, C. Dubois, C. Camberlin, J. Vlayen, H. Van Oyen, C. Léonard and P. Meun (2012). Performance of the Belgian Health System. Report 2012. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 196. 30. 152 p. ☐

B8. Please indicate in which project(s) patients were actively involved, as defined above (only check the box if you also worked on the project and followed the patient involvement activities)

- Allen, F., M. Kroes, S. Mitchell, F. Mambourg and D. Paulus (2011). Diagnosis and treatment of varicose veins in the legs. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 164. ☐
- Beutels, P., A. Blommaert, G. Hanquet, J. Billeke, N. Thiry, M. Sabbé, J. Verhaeghe, F. De Smet, M. Callens and P. Van Damme (2011). Cost-effectiveness of 10- and 13-valent pneumococcal conjugate vaccines in childhood. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 155. ☐
- De Gendt, T., A. Desomer, M. Goossens, G. Hanquet, C. Léonard, R. Mertens, J. Piérart, J. Robays, D. Roberfroid, O. Schmitz, A. Van den Bruel, I. Vinck and L. Kohn (2011). Acupuncture: State of affairs in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 153. ☐
- De Gendt, T., A. Desomer, M. Goossens, G. Hanquet, C. Léonard, R. Mertens, J. Piérart, J. Robays, D. Roberfroid, O. Schmitz, I. Vinck and L. Kohn (2011). Homeopathy : State of affairs in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 154. ☐
- De Maess, B., K. Boulanger, P. Schoukens, W. Sermeus, C. Van de Voorde, P. Vrijens and I. Vinck (2011). Elective care for foreign patients : impact on the Belgian healthcare system. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 169. 25 p. ☐
- Goephebeur, E., R. Van Rossem, K. Bunt, K. Vanhoute, T. Boreberg, P. Demette, M. De Ridder, D. Harrington, M. Peeters, G. Storme, J. Verhaert, J. Vlayen, P. Vrijens, S. Vandeplas and W. Creten (2011). Quality Insurance of rectal cancer - phase 3 : statistical methods to benchmark centers on a set of quality indicators. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 161. ☐



- Hanquet, G., P. Jonckheer, J. Vlayen, F. Vrijens, N. Thiry and P. Beutels (2011). Seasonal influenza vaccination : priority target groups - Part I. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 162. ☐
- Huisaert, F., M. Neyt, I. Vinck, S. Stordeer, M. Huijs, S. Sauerland, M. R. Kuijpers, P. Abrihami, H. Vondeling and H. Van Brabant (2011). The pre-market clinical evaluation of innovative high-risk medical devices. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 158. ☐
- Jonckheer, P., C. Dubois, L. Borgemans, E. Verhoeven, E. Rinchard, A.-M. Baudewyns, T. Haezaert, I. Vinck, M. Lona and D. Paulus (2011). After-Hours Primary Care: which solutions? KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 171. ☐
- Jonckheer, P., S. Stordeur, G. Lebeer, M. Rohard, J. De Schampheleire, M. De Troyer, N. Kacemdenbogh, A. M. Offermans, J. Piérart and L. Kohn (2011). Barriers among general practitioners: prevention and management. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 165. ☐
- Kroes, M., S. Garcia-Stewart, F. Allen, M. Eysen and D. Paulus (2011). Dementia : which non-pharmacological interventions? KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 160. ☐
- Monnerey, G., K. Van Den Heede, N. Verhaeghe, N. Swartenbroeckx, L. Annemans, E. Schoenjes and M. Eysen (2011). Organisation of child and adolescent mental health care : study of the literature and an international overview. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 170. IV. 121 p. ☐
- Neyt, M., H. Van Brabant, S. Van de Sande and S. Devriese (2011). Transcatheter Aortic Valve Implantation (TAVI): a Health Technology Assessment Update. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 163. ☐
- 1st print: 4 juillet 2011)). Pharmacological prevention of fragility fractures in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 159. ☐
- Schwiezer, C., N. Thiry, S. Van de Sande, M. Gamil, F. Nevens, I. Collie, Y. Houtmans and F. Huisaert (2011). Economic evaluation of antiviral treatment of chronic hepatitis B in Belgium : Part 2. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 157. ☐
- Van de Voorde, C., L. Kohn and I. Vinck (2011). Entitlement to a hospital insurance for persons with a chronic illness or handicap. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 166. ☐
- Van den Bosch, K., P. Willems, J. Geerts, J. Breda, S. Peeters, S. Van de Sande, F. Vrijens, C. Van de Voorde and S. Stordeer (2011). Residential care for older persons in Belgium : Projections 2011 - 2025. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 167. 24 p. ☐
- Van Meerhaeghe, A., L. Annemans, P. Haentjens, L. San Miguel, B. Buyse, K. Benoit, S. Devriese, S. Stroobant and J. Gailly (2011). Home Oxygen Therapy. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 156. ☐
- Vergote, I., J. Vlayen, J. Robays, D. Stenken, Y. Smit, C. Bougiani, J. De Grève, F. Kridelka, P. Scalliet, P. Simon, S. Stroobant, P. Van Dam, E. Van Limbergen and G. Villeirs (2011). A national clinical practice guideline for the management of cervical cancer. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 168. ☐
- Vlayen, J., S. Stordeer, F. Vrijens and E. Van Eycken (2011). Quality indicators in oncology: prerequisites for the set-up of a quality system. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 152. ☐
- B9. Please indicate in which project(s) patients were actively involved, as defined above (only check the box if you also worked on the project and followed the patient involvement activities)**
- Bikke, J., C. Marais, O. Benon, A. J. van Hoek, O. Lejeune, M. Callens, S. Vancorenand, E. Van Kerschaer, K. Callaert, N. Heus, P. Van Damme and P. Beutels (2010). Cost-utility of Vaccination against Chikungunya in Children and against Herpes Zoster in Elderly in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 151. ☐
- Borgemans, L., C. Dubois, S. Rieppl, S. Vanhaeren, N. Geukens, C. Pilon, F. Clairet, C. Massart, S. Brunet, L. Kohn, J. Piérart and D. Paulus (2010). Impact of academic detailing on primary care physicians. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 125. ☐
- Cardoso, F., S. Stordeer, J. Vlayen, C. Bourgin, B. Cluys, M.-R. Christiaens, Y. Cocquyt, E. Lefrançois, P. Neven, P. Scalliet, J.-C. Schobben, M. Van Goethem and G. Villeins (2010). Scientific support of the College of Oncology: update of the national guidelines on breast cancer. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 143. ☐
- Cleemput, I., C. Beguin, Y. de la Kethulle, S. Gerkens, M. Jadoul, G. Verpoeten and C. De Laet (2010). Organisation and financing of chronic dialysis in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 124. ☐
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- Deboutte, D., M. Smet, V. Walraven, A. Janssens, C. Obyn and M. Leys (2010). Emergency psychiatric care for children and adolescents. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 135. ☐
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- Houtmans, Y., N. Thiry, M. Le Polain, M. Adler, I. Collie, J. Devalde, P. Michielien, H. Orient, P. Van Damme and F. Hulstaert (2010). Cost-effectiveness of antiviral treatment of chronic hepatitis B in Belgium. Part I: Literature review and results of a national study. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 127. ☐
- Le Polain, M., M. Franken, M. Koopmanschap and I. Cleemput (2010). Drug reimbursement systems: international comparison and policy recommendations. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 147. ☐
- Leyts, M., C. Antoine, V. De Jaegere and O. Schmitz (2010). Mental health care reforms: evaluation research of 'therapeutic projects'. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 146. ☐
- Mambourg, F., J. Gailly and W.-H. Zhang (2010). Guideline relative to low risk birth. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 139. ☐



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Proesmans, M., H. Cuppens, M.-F. Vincent, A. Palem, K. De Boeck, K. Diericks, H. Nys, M. Neyt, I. Vinck and P. Lebeque (2010). Is neonatal screening for cystic fibrosis recommended in Belgium? KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 132.

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Roberfroid, D., C. Dubois, F. Vrijens, C. Camberlin and M. Farfan-Protet (2010). Statins in Belgium: utilization trends and impact of reimbursement policies. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 141.

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Spinevue, A., V. Foulon, C. Clarys, J. De Lepeleire, P. Chevalier, F. Desplantes, S. De Winter, C. Dumont, V. Lacroix, S. Simoons, C. Dubois and D. Paulus (2010). Seamless care with regard to medications between hospital and home. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 131.

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Van Brabant, H., M. Neyt, S. Stroobandt, S. Van de Sande and C. Schwierz (2010). Pacemaker therapy for bradycardia in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 137.

Van de Sande, S., D. De Ryck, K. De Gauquier, R. Hilderson, M. Neyt, G. Peeters, N. Swartenbroeckx, W. Tambeur, G. Vanden Boer and C. Van de Voode (2010). Feasibility study of the introduction of an all-inclusive case-based hospital financing system in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 121.

Van Vlaenderen, L., J. Worrall, S. Raza, A. Collie, C. De Vos, D. Steens, Ö. Saka, B. Moore, M. Eysen and D. Paulus (2010). Cardiac rehabilitation: clinical effectiveness and utilization in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 140.

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Yerna, B.-L., C. Mélotte and J.-P. Closon (2010). Indemnification of the victims who contracted HIV or hepatitis C through a contaminated blood transfusion. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 134.

B10. Please indicate in which project(s) patients were actively involved, as defined above (only check the box if you also worked on the project and followed the patient involvement activities)

Ansemans, L., P. Boeckstaens, L. Borgersman, D. De Smedt, C. Duchesnes, J. Heymans, R. Remmen, W. Sermeus, C. Van Den Broeke, P. Van Herck, M. Van Meerbeek, S. Willems and K. De Gauquier (2009). Advantages, disadvantages and feasibility of the introduction of 'Pay for Quality' programmes in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 118.

Beutels, P., J. Layten, O. Lejeune, N. Hens, J. Billeke, K. De Schijver, S. Van de Sande, K. Van Herck and P. Van Damme (2009). Evaluation of universal and targeted hepatitis A vaccination programs in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 98.

Camberlin, C., A. Senn, M. Leys and C. De Laet (2009). Robot-assisted surgery: health technology assessment KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 104.

Ces, S., V. De Jaegere, P. Feys and M. Leys (2009). Mental health care reforms: evaluation research of 'therapeutic projects' - first intermediate report. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 103.

Denis, A., S. Simoons, C. Fostier, L. Mergaert and I. Cleemput (2009). Policies for Orphan Diseases and Orphan Drugs. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 112.

Gailly, J., S. Gerkens, A. Van den Bruel, S. Devriese, C. Obyn and I. Cleemput (2009). Use of point-of-care devices in patients with oral anticoagulation: a Health Technology Assessment. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 117.

Gillain, D., A. Velghe, X. Boman, P. Fecher, P. Gillet, P. Kohn, J. Petermans, N. Van Den Noortgate, L. Kohn, F. Mambourg and C. Léonard (2009). Financing of the Geriatric Day Hospital. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 99.

Hulstaert, F., N. Thiry, M. Eysen and F. Vrijens (2009). Pharmaceutical and non-pharmaceutical interventions for Alzheimer's Disease, a rapid assessment. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 111.

Keirse, E., C. Beguin, M. Desmedt, M. Develghe, J. Menten, S. Simoons, J. Wens, L. Borgersman, L. Kohn, B. Spinevue, A. Cardinale, B. Katten, P. Vanden Berghe and D. Paulus (2009). Organisation of palliative care in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 115.



Léonard, C., G. Hanquet, A. Senn and M. Huybrechts (2009). How to ensure self-sufficiency of stable plasma derivatives in Belgium ? KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 120.

Neyt, M., A. Van den Bruel, J. Gailly, N. Thiry and S. Devriese (2009). Tiotropium in the Treatment of Chronic Obstructive Pulmonary Disease Health Technology Assessment. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 108.

Obyn, C., I. Cleemput, C. Léonard and J.-P. Closon (2009). Magnetic Resonance Imaging: cost analysis. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 106.

Peeters, M., J. Vlayen, S. Stordeur, F. Mambourg, T. Boterberg, B. de Hempen, P. Demetter, P. Deprez, J.-F. Gigot, A. Hooren, E. Van Cunen, B. Van den Eynden, J.-L. Van Laethem and C. Verschuer (2009). Scientific support of the College of Oncology: a national clinical practice guideline for pancreatic cancer. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 105.

Stordeur, S., S. Gerkens and D. Roberfroid (2009). Interspinous implants and pedicle screws for dynamic stabilization of lumbar spine: Rapid assessment. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 116.

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Van Brabant, H. and M. Neyt (2009). Endobronchial valves in the treatment of severe pulmonary emphysema: a rapid Health Technology Assessment. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 114.

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Vlayen, J., S. Stordeur, A. Van den Bruel, F. Mambourg and M. Eysen (2009). Positron Emission Tomography (PET) in Belgium: an update. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 110.

Vrijens, F., K. De Gauquier and C. Camberlin (2009). The volume of surgical interventions and its impact on the outcome: Feasibility study based on Belgian data. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 113.

Vrijens, F., F. Hulstaert, B. Gordts, C. De Laet, S. Devriese, S. Van de Sande, M. Huybrechts and G. Peeters (2009). Nosocomial Infections in Belgium, part 2: Impact on Mortality and Costs. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 102.

Yerna, B.-L., X. Maréchal, M. Demuit, J.-P. Closon and I. Vinck (2009). Compensation for damage due to health care - Phase V: Budgetary impact of the transposition of the French system in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 107.

B11. Please indicate in which project(s) patients were actively involved, as defined above (only check the box if you also worked on the project and followed the patient involvement activities)

Ansemans, L., J.-P. Closon, M.-C. Closon, I. Heymans, R. Lagane, E. Mendes da Costa, C. Moucous and I. Roch (2008). Comparison of the cost and the quality of two financing systems of primary health care in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 85.

Beguin, C., N. Deggou, M. Debary, S. Gerkens, D. Van den Steen, D. Roberfroid, C. De Laet and M.-C. Closon (2008). Hearing aids in Belgium: health technology assessment. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 91.

Berckmans, G., L. Alvarez Irujo, N. Bouzgea, T. Defloor, G. Peeters, S. Stordeur and M. Gobert (2008). Differentiated practice in nursing: opportunities and limits. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 86.

Carels, C., L. De Ridder, N. Van Loock, K. Bogarts, M. Eysen and C. Obyn (2008). Orthodontics for children and adolescents. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 77.

Cleemput, I., M. Neyt, N. Thiry, C. De Laet and M. Leys (2008). Threshold values for cost-effectiveness in health care KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 100.

Cleemput, I., P. Van Wilder, F. Vrijens, M. Huybrechts and D. Ramaekers (2008). Guidelines for Pharmacoeconomic Evaluations in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 78.

Closon, M.-C., M. Gobert, T. Peperack, F. Falez, J.-P. Baeyens, L. Kohn, N. Swartenbroeckx and C. Léonard (2008). Financing of the care program for geriatric patients in classic hospital. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 73.

De Gauquier, K., A. Senn, L. Kohn and I. Vinck (2008). International comparison of reimbursement principles and legal aspects of plastic surgery. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 83.

De Laet, C., C. Obyn, D. Ramaekers, S. Van de Sande and M. Neyt (2008). Hyperbaric Oxygen Therapy: a Rapid Assessment. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 74.

de Walque, C., B. Seumtjens, K. Vermeyen, G. Peeters and I. Vinck (2008). Comparative study of hospital accreditation programs in Europe. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 70.

Devolder, P., M. Demuit, X. Maréchal, B.-L. Yerna, J.-P. Closon, C. Léonard, A. Senn and I. Vinck (2008). Setting a medical index for private health insurance contracts. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 96.

Gillet, P., P. Kohn, W. Sermeus, A. Vleugels, J. Jacques, K. Van den heede and S. Verelst (2008). Detection of adverse events in administrative databases. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 93.

Huybrechts, M., I. Cleemput, S. Devriese and F. Hulstaert (2008). Radioisotopes Reimbursement in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 79.

Leclercq, A., K. De Gauquier, A. Cuppens, J. Boly, D. Van Den Steen and D. Paulus (2008). Consumption of physiotherapy and physical and rehabilitation medicine in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 87.

Legrand, M., V. Coudron, I. Taillieu, N. Rooryck, K. Dupont, P. Boudrez, A. Senn, I. Vinck, G. Peeters and L. Kohn (2008). Video registration of endoscopic surgery: a rapid assessment. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 101.



Lorant, V., C. Geerts, W. D'Hoore, D. Sautew, R. Remmen, L. Peermans, H. Buttiens, C. Duchesnes, L. Rysaert, J. De Maesseneer, I. Violet, J. Goedhuys, V. Soto Rojas and D. Paulus (2008). Making General Practice Attractive: Encouraging GP attraction and retention. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 90. ☐

Obyn, C. and F. Mambourg (2008). Rapid assessment of a selection of new treatments for prostate cancer and benign prostate hypertrophy. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 89. ☐

Peeters, M., T. Lerut, J. Vlietin, F. Mambourg, N. Etcon, P. Deprez, T. Boterberg, J. De Mey, P. Plamen, J.-L. Van Laethem, B. Neyns and P. Pattin (2008). Guideline for the esophageal and gastric cancer: scientific support of the College of Oncology. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 75. ☐

Remmen, R., L. Seunjens, D. Portaux, P. Luyten, K. Knops, J.-B. Lafontaine, H. Philips, L. Lafeyve, A. Van den Bruel and D. Paulus (2008). Quality development in general practice in Belgium: status quo or quo vadis? KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 76. ☐

Roberfroid, D., S. Stordeur, C. Camberlin, C. Van de Voorde, F. Vrijens and C. Léonard (2008). Physician workforce supply in Belgium. Current situation and challenges. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 72. ☐

Scheiris, J., S. Pieters, K. De Block, D. De Graeve, M. Eysen, S. Van Endeghem, D. Deboutte and H. Roeyers (2008). Rehabilitation centres ENT/PSY: target groups, scientific evidence and care organisation. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 97. ☐

Schokkaert, E., J. Guillaume, A. Lecluyse, H. Avalosse, K. Cornelis, D. De Graeve, S. Devriese, J. Vanoverloop and C. Van de Voorde (2008). Effects of the Maximum Billing system on health care consumption and financial access to health care. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 80. ☐

Stordeur, S., N. Thiry and M. Eysen (2008). Fatigue Syndrome: diagnosis, treatment and organisation of care. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 88. ☐

Van Brabant, H., C. Camberlin and I. Cleemput (2008). 64-Slice computed tomography imaging of coronary arteries in patients suspected for coronary artery disease. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 82. ☐

Van Brabant, H. and M. Neyt (2008). Percutaneous heart valve implantation in congenital and degenerative valve disease. A rapid Health Technology Assessment. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 95. ☐

Van den Bruel, A., J. Gailly, F. Vrijens and S. Devriese (2008). Guidance for the use of five ophthalmic tests in clinical practice. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 71: ix, 103 p. ☐

Van Parys, A.-S., C. Lucet, A. Remacle, T. Di Zinno, H. Verstraeten, F. Mambourg, G. Peeters and M. Temmerman (2008). Maternal intensive Care in Belgium. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 94. ☐

Verniest, R., A. Laenen, A. Daems, L. Kohn, G. Vandermeersch, V. Fabri, R. Mertens, C. Van Audenhove and M. Leys (2008). Long stay patients in psychiatry T-beds. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 84. ☐

Vlietin, J., M. Verstreken, C. Mertens, E. Van Eycken and F. Penninckx (2008). Quality insurance for rectal cancer - phase 2: development and testing of a set of quality indicators. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 81. ☐

Vrijens, F., B. Gordts, C. De Laet, S. Devriese, S. Van de Sande, M. Haybrechts, G. Peeters and F. Hulstert (2008). Nosocomial Infections in Belgium, part 1: national prevalence study. KCE Reports. Brussels, Belgian Health Care Knowledge Centre (KCE). 92. ☐

Section C: Description of the patient involvement in the project

"{R2018_1.shown}"

C1. Why did you want to involve patients?

C2. Who did you involve to represent the patient's perspective?

- Individual patient(s) with the disease/condition ☐
- Patient association(s) ☐
- Patient umbrella organization(s) ☐
- Other (e.g. caregiver, family of patients with the disease,...) ☐



C3. Please specify who is 'other'

C4. At which stage were individual patient involved?

- Scoping ☐
- Design ☐
- Outcome definition ☐
- In the data collection ☐
- Conclusions ☐
- Recommendations ☐
- I don't know ☐

C5. How often did you involved them ?

	Once during the entire project	More than once	Continuous collaboration	Other	I don't remember
Individual patient(s)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Patient association(s)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Patient umbrella organisation(s)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
{INSERTANS:64+258X3X+1}	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

C6. How many patients did you involved?

- 0 ☐
- 1-5 ☐
- 6-10 ☐
- 11-15 ☐
- 15-25 ☐
- >25 ☐
- I don't know ☐



C7. How many patient association(s) did you involve?

1 ☐
1-3 ☐
>3 ☐
I don't know ☐

C8. Which umbrella organisation(s) did you involve?

LUSS ☐
VPP ☐
Patienten Rat&Treff ☐
Other ☐

Other

C9. How did you identify ...

Individual patient(s)
Patient association(s)
Patient umbrella organisation(s)
{INSERTANS:644258X3X+1}

C10. How did you recruit ...

Individual patient(s)
Patient association(s)
Patient umbrella organisation(s)
{INSERTANS:644258X3X+1}

C11. How did you select ...

Individual patient(s)
Patient association(s)
Patient umbrella organisation(s)
{INSERTANS:644258X3X+1}



C12. In general, regarding patient involvement in this project, what has been going smoothly?

C13. In general, regarding patient involvement in this project, which difficulties do you experimented?

C14. In general, regarding patient involvement in this project, which solutions do you eventually apply and for what results?

C15. How did you involved individual patients in the scoping phase?

☐
Other ☐

Other

C16. At which stage were patient association(s) involved?

Scoping ☐
Design ☐
Outcome definition ☐
In the data collection ☐
Conclusions ☐



Recommendations ☐
I don't know ☐

Section D: renouncement to PI

D1. Do you remember a project where you, as principal investigator or responsible of the 'patient' part of the project, intended to involve patient but finally have renounced?

Yes ☐
No ☐

D2. In which project(s) do you intended to involve patients but you finally have renounced?

- Towards an integrated evidence-based practice plan in Belgium – Part 1: literature, Belgian situation and end-user needs ☐
- How to improve the Organisation of Mental healthcare for older adults in Belgium? ☐
- Use of patient-reported outcome and experience measures in patient care and policy ☐
- Towards an integrated evidence-based practice plan in Belgium – Part 4: EBP Implementation in primary health care in Belgium ☐
- The use of whole genome sequencing in clinical practice: challenges and organisational considerations for Belgium ☐
- Towards an integrated evidence-based practice plan in Belgium – Part 3: Professional leadership and change management as a catalyst for EBP Implementation ☐
- MammaPrint® test for personalised management of adjuvant chemotherapy decisions in early breast cancer ☐
- Towards an integrated evidence-based practice plan in Belgium – Part 5 – Performance management for EBP implementation in primary health care in Belgium ☐
- Payment methods for hospital stays with a large variability in the care process ☐
- An evaluation protocol for NIHDI conventions ☐
- Responsible use of high-risk medical devices: the example of 3D printed medical devices ☐
- Towards an integrated evidence-based practice plan in Belgium – Part 2: Governance Plan ☐

D3. What was foreseen?



D4. Why did you abandon this idea?

D5. In which project(s) do you intended to involve patients but you finally have renounced?

- EBP Plan ☐
- Towards tailoring of KCE guidelines to end-users' needs ☐
- Towards an inclusive system for major trauma ☐
- How to improve the Belgian process for Managed Entry Agreements? An analysis of the Belgian and international experience ☐
- Low back pain and radicular pain: development of a clinical pathway ☐
- Horizon scanning for pharmaceuticals: proposal for the BeNeLuxA collaboration ☐
- Proposals for a further expansion of day surgery in Belgium ☐
- Health care in Belgian prisons ☐
- Bevacizumab in the treatment of ovarian cancer ☐
- Exploratory steps for the formulation of Belgian health system targets ☐
- Static automated external defibrillators for opportunistic use by bystanders ☐
- Anticoagulants in non-valvular atrial fibrillation ☐
- Required hospital capacity in 2025 and criteria for rationalisation of complex cancer surgery, radiotherapy and maternity services ☐
- Appropriate care at the end of life ☐
- Low back pain and radicular pain: assessment and management ☐
- Strategies for improving the medical workforce projection model: a stakeholder consultation ☐
- Management of pancreatic cancer: capita selecta – Part 1: Introduction and methodology ☐
- Management of pancreatic cancer Part 2: Diagnosis ☐
- Management of pancreatic cancer – Part 3: neoadjuvant and induction therapy ☐
- Management of pancreatic cancer – Part 4: recurrent and metastatic cancer ☐
- Routine preoperative testing in adults undergoing elective non-cardiothoracic surgery ☐

D6. In which project(s) do you intended to involve patients but you finally have renounced?

Health workforce planning and midwifery-specific data ☐



Use of pneumococcal vaccines in the elderly: an economic evaluation	<input type="checkbox"/>
Financial compensation for persons infected by a contaminated blood transfusion – an attempt to reduce discriminations	<input type="checkbox"/>
Multi-criteria decision analysis for the appraisal of medical needs: a pilot study	<input type="checkbox"/>
Multi criteria decision analysis to select priority diseases for Newborn blood screening	<input type="checkbox"/>
Governance models for hospital collaborations	<input type="checkbox"/>
Belrai suite of instruments: an exploratory study on applicability for individual care planning and budget allocation in rehabilitation care	<input type="checkbox"/>
Clustering pathology groups on hospital stay similarity	<input type="checkbox"/>
Economic evaluation of novel direct acting antiviral (DAA) treatment strategies for chronic hepatitis C	<input type="checkbox"/>
Model for the organization and reimbursement of psychological and orthopedagogical care in Belgium	<input type="checkbox"/>
Financial compensation for persons infected by a contaminated blood transfusion – an attempt to reduce discriminations	<input type="checkbox"/>
Reduction of the treatment gap for problematic alcohol use in Belgium	<input type="checkbox"/>
Left ventricular assist devices in the treatment of end-stage heart failure	<input type="checkbox"/>
Guideline on the management of rectal cancer: update of capita selecta – Part: 1 Introduction and Methodology	<input type="checkbox"/>
Guideline on the management of rectal cancer: update of capita selecta – Part 2: Staging	<input type="checkbox"/>
Guideline on the management of rectal cancer: update of capita selecta – Part 3: Local vs Radical resection for stage 1 tumours	<input type="checkbox"/>
Guideline on the management of rectal cancer: update of capita selecta – Part 4: Adjuvant therapy after neoadjuvant therapy	<input type="checkbox"/>
The role of biomarkers in ruling out cerebral lesions in mild cranial trauma	<input type="checkbox"/>
Elective caesarean section in low-risk women at term: consequences for mother and offspring	<input type="checkbox"/>
Organisation and payment of emergency care services in Belgium: current situation and options for reform	<input type="checkbox"/>
Future scenarios about drug development and drug pricing	<input type="checkbox"/>
Ovarian cancer: diagnosis, treatment and follow-up	<input type="checkbox"/>
Performance of the Belgian Health System - Report 2015	<input type="checkbox"/>
Quality indicators for the management of lung cancer	<input type="checkbox"/>
D7. In which project(s) do you intended to involve patients but you finally have renounced?	
Cervical cancer screening program and Human Papillomavirus (HPV) testing, part II: Update on HPV primary screening	<input type="checkbox"/>
Towards a guided and phased introduction of high-risk medical devices in Belgium	<input type="checkbox"/>
Renal cancer in adults: diagnosis, treatment and follow-up	<input type="checkbox"/>
Percutaneous Vertebroplasty and Balloon Kyphoplasty	<input type="checkbox"/>
Comprehensive geriatric care in hospitals: the role of inpatient geriatric consultation teams	<input type="checkbox"/>



Cardiovascular pre-participation screening in young athletes	<input type="checkbox"/>
Implementation of hospital at home: orientations for Belgium	<input type="checkbox"/>
Autologous breast reconstruction techniques after mammary resection: time measurements for a potential reevaluation of the surgeon fee	<input type="checkbox"/>
Oropharyngeal, hypopharyngeal and laryngeal cancer: diagnosis, treatment and follow-up	<input type="checkbox"/>
What are the recommended clinical assessment and screening tests during pregnancy?	<input type="checkbox"/>
Cervical and lumbar total disc replacements	<input type="checkbox"/>
Hadron therapy in children – an update of the scientific evidence for 15 paediatric cancers	<input type="checkbox"/>
Publicly funded Practice-oriented Clinical Trials	<input type="checkbox"/>
Oncogenetic testing, diagnosis and follow-up in Birt-Hogg-Dubé syndrome, familial atypical multiple mole melanoma syndrome and neurofibromatosis 1 and 2	<input type="checkbox"/>
Oncogenetic testing and follow-up for women with familial breast/ovarian cancer, Li Fraumeni syndrome and Cowden syndrome	<input type="checkbox"/>
What health care for undocumented migrants in Belgium ?	<input type="checkbox"/>
Non-invasive markers of subclinical atherosclerosis for predicting a primary cardiovascular event: a rapid systematic review	<input type="checkbox"/>
Gene expression profiling and immunohistochemistry tests for personalised management of adjuvant chemotherapy decisions in early breast cancer – a Rapid Assessment	<input type="checkbox"/>
Bladder cancer: an assessment of international practice guidelines	<input type="checkbox"/>
Next generation sequencing gene panels for targeted therapy in oncology and haemato-oncology	<input type="checkbox"/>
Towards a better managed off-label use of drugs	<input type="checkbox"/>
Oncogenetic testing for persons with hereditary endocrine cancer syndromes	<input type="checkbox"/>
Ten years of multidisciplinary teams meetings in oncology: current situation and perspectives	<input type="checkbox"/>
D8. In which project(s) do you intended to involve patients but you finally have renounced?	
Support for informal caregivers – an exploratory analysis	<input type="checkbox"/>
Caring for mothers and newborns after uncomplicated delivery: towards integrated postnatal care	<input type="checkbox"/>
Incorporating societal preferences in reimbursement decisions - Relative importance of decision criteria according to Belgian citizens	<input type="checkbox"/>
Oral cavity cancer: diagnosis, treatment and follow-up	<input type="checkbox"/>
A quadrivalent vaccine against serogroup B meningococcal disease: a cost-effectiveness study	<input type="checkbox"/>
The non-invasive prenatal test (NIPT) for trisomy 21 – health economic aspects	<input type="checkbox"/>
The long-term efficacy of psychotherapy, alone or in combination with antidepressants, in the treatment of adult major depression	<input type="checkbox"/>
Informed choice on breast cancer screening: messages to support informed decision	<input type="checkbox"/>
A decision aid for an informed choice when patient asks for PSA screening	<input type="checkbox"/>
Colon Cancer: Diagnosis, Treatment and Follow-Up	<input type="checkbox"/>



Oncogenetic testing for Lynch syndrome and familial adenomatous polyposis	<input type="checkbox"/>
Prevention of preterm birth in women at risk: selected topics	<input type="checkbox"/>
Machine perfusion in kidneys from deceased donors – A rapid assessment	<input type="checkbox"/>
Organisation of care for adults with a rare or complex cancer	<input type="checkbox"/>
National practice guideline on the treatment of localised prostate cancer – part 2	<input type="checkbox"/>
Conceptual framework for the reform of the Belgian hospital payment system	<input type="checkbox"/>
Management of autism in children and young people: a good clinical practice guideline	<input type="checkbox"/>
Correction of refractive errors of the eye in adults – part 3: organisation and legal framework of extramural surgery centres	<input type="checkbox"/>
Revascularization for lower limb peripheral arterial disease	<input type="checkbox"/>
D9. In which project(s) do you intended to involve patients but you finally have renounced?	
A National Guideline for the prevention of pressure ulcers	<input type="checkbox"/>
Seasonal influenza vaccination: prioritizing children or other target groups?: Part II: cost-effectiveness analysis	<input type="checkbox"/>
[Coupling of the Permanent Sample with the Hospital Data: Feasibility and data representativeness study]	<input type="checkbox"/>
Correction of refractive errors of the eye in adults : Part 1 : Perceptions and experiences	<input type="checkbox"/>
Organisation of aftercare for patients with severe burn injuries	<input type="checkbox"/>
General framework for a multidisciplinary quality manual for cardiac care networks	<input type="checkbox"/>
Dissemination and implementation of clinical practice guidelines in Belgium	<input type="checkbox"/>
Supportive treatment for cancer – Part 3 : Treatment of pain: most common practices	<input type="checkbox"/>
Extremity-only MRI	<input type="checkbox"/>
Innovative radiotherapy techniques : a multicentre time-driven activity-based costing study	<input type="checkbox"/>
The decisional process for the choice of active surveillance in localized prostate cancer	<input type="checkbox"/>
Barriers and opportunities for the uptake of biosimilar medicines in Belgium	<input type="checkbox"/>
Correction of refractive errors of the eye in adults – Part 2: laser surgery and intraocular lenses	<input type="checkbox"/>
Novel serum biomarkers for the prediction of cardiovascular risk	<input type="checkbox"/>
Ankle sprains: diagnosis and therapy	<input type="checkbox"/>
Evolution of day care: impact of financing and regulations	<input type="checkbox"/>
A comparative analysis of hospital care payments in five countries	<input type="checkbox"/>
Impact of the KCE reports published in 2009-2011	<input type="checkbox"/>



Quality indicators for the management of upper gastrointestinal cancer	<input type="checkbox"/>
Non-Small Cell and Small Cell Lung Cancer: Diagnosis, Treatment and Follow-up	<input type="checkbox"/>
Breast cancer in women : diagnosis, treatment and follow-up	<input type="checkbox"/>
D10. In which project(s) do you intended to involve patients but you finally have renounced?	
Neuromodulation for the management of chronic pain : implanted spinal cord stimulators and intrathecal analgesic delivery pumps	<input type="checkbox"/>
Scientific support of the College of Oncology : update of the national guidelines on breast cancer	<input type="checkbox"/>
Models for citizen and patient involvement in health care policy - Part I : exploration of their feasibility and acceptability	<input type="checkbox"/>
Principles and criteria for the level of patient cost sharing: Reflections on value-based insurance	<input type="checkbox"/>
Belgian guidelines for economic evaluations and budget impact analyses : second edition	<input type="checkbox"/>
Simplification of patient cost sharing : the example of physician consultations and visits	<input type="checkbox"/>
Regional differences in thyroid cancer incidence in Belgium: role of diagnostic and therapeutic strategies for thyroid disease.	<input type="checkbox"/>
Hepatitis C : Screening and Prevention	<input type="checkbox"/>
Supportive treatment for cancer : Part 1 - exercise treatment	<input type="checkbox"/>
The use of Qualitative Research Methods in KCE studies	<input type="checkbox"/>
A national clinical practice guideline on the management of localised prostate cancer : - part 1	<input type="checkbox"/>
Breast cancer screening amongst women aged 70-74 years of age	<input type="checkbox"/>
Stroke units : efficacy, quality indicators and organisation	<input type="checkbox"/>
Organisation of care for chronic patients in Belgium : development of a position paper	<input type="checkbox"/>
Stakeholder Involvement in KCE working processes	<input type="checkbox"/>
Vaccine safety surveillance in Belgium : place and limits of a background rate approach	<input type="checkbox"/>
Prophylactic removal of pathology-free wisdom teeth : rapid assessment	<input type="checkbox"/>
Manual for cost-based pricing of hospital interventions	<input type="checkbox"/>
Catheter Ablation of Atrial Fibrillation	<input type="checkbox"/>
The organisation of mental health services for children and adolescents in Belgium : development of a policy scenario	<input type="checkbox"/>
Identifying women at risk for breast cancer/technical methods for breast cancer screening	<input type="checkbox"/>
Supportive treatment for cancer - Part 2 : Prevention and treatment of adverse events related to chemotherapy and radiotherapy	<input type="checkbox"/>
Performance of the Belgian Health System. Report 2012	<input type="checkbox"/>



Section E: Ongoing projects

E1. Are you currently working on (a) project(s) where you intend to involve patients / are involving patients?

Yes ☐

No ☐

I don't know ☐

3

1

5

E2. Which one(s)

Title project 1									
Title project 2									
Title project 3									

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APPENDIX 6. SUPPORT OF POSITION STATEMENTS BY KCE EMPLOYEES

Appendix 6.1. Results of the first voting round of the Delphi process

Statements	N	Agree %	Almost agree %	Disagree somewhat %	Strongly disagree %	Total Agree %	Total Disagree %
KCE perceives the fundamental ethical, as well as the instrumental and procedural rationales for patient involvement decisive enough to take a positive position towards patient involvement in health policy research.	45	76	24	0	0	100	0
The relevance and need for patient involvement in research projects should be assessed project by project.	48	77	17	2	4	94	6
Patient involvement in health policy research is complementary to the review of scientific evidence and primary data collection, not a substitute for it.	46	83	9	7	2	91	9
Sufficient resources (people, time, and budget) should be made available to ensure and support effective patient involvement in health policy research.	46	85	13	2	0	98	2
The planning of the projects has to be adapted to implement patient involvement on an optimal way.	46	63	28	9	0	91	9
Training should be organised for researchers and patients/patient organizations to effectively involve patients or be involved in health policy research.	45	49	38	11	2	87	13
Patient involvement activities in health policy research should be regularly evaluated and procedures revised when appropriate.	46	76	22	2	0	98	2
Patient contributions and potential impact should be reported in the study report.	46	74	22	4	0	96	4
Patient representatives who have been involved should receive feedback from KCE and provide feedback to KCE to potentially improve future collaboration.	46	50	35	15	0	85	15



Individual patients and/or patient organisations should be <u>consulted</u> in the scoping of the KCE projects to allow researchers to better describe the context.	45	56	33	11	0	89	11
Individual patients should contribute to the scoping by <u>co-producing</u> the elements that need to be addressed in the research project.	46	17	30	41	11	47	52
Individual patient 'experts' and/or patient umbrella organisations should be <u>consulted</u> on the selection of <u>methods</u> for the projects.	45	18	33	29	20	51	49
Patient organisations should be <u>consulted</u> in the selection of the <u>outcomes</u> to be included in the study.	46	28	54	11	7	82	18
Patient organisations and/or patient umbrella organisations and/or sickness funds could <u>co-decide</u> on the approaches for recruitment of participants if primary data collection in patients or users is needed.	48	40	48	10	2	88	12
Patient organisations should be <u>consulted</u> to select and test the data collection instrument(s).	45	29	40	29	2	69	31
Individual patients and/or patient organisations should be <u>consulted</u> to define the minimal important difference in patient outcomes.	46	30	52	13	4	82	17
Individual 'expert' patients should be <u>consulted</u> to interpret results of analyses.	46	17	33	41	9	50	50
Patient organisations and/or patient umbrella organisations should be given the opportunity to review the KCE synthesis and give feedback before publication (=consultation).	44	25	18	25	32	43	57
Individual patients and/or patient organisations and/or patient umbrella organisations and/or sickness funds should <u>collaborate</u> on the dissemination of the results of the KCE project.	47	64	30	4	2	94	6

Green: consensus for agreement

Yellow: dissensus

Red: consensus for rejection



Appendix 6.2. Results of the second voting round of the Delphi process

Statements	N	Agree %	Almost agree %	Disagree somewhat %	Strongly disagree %	Total Agree %	Total Disagree %
Individual patients should contribute to the scoping by <u>co-producing</u> the elements that need to be addressed in the research project.	45	13	22	49	16	35	65
Patient organisations should be <u>consulted</u> to select and test the data collection instrument(s).	48	15	48	29	8	63	37
Individual 'expert' patients should be <u>consulted</u> to interpret results of analyses.	47	13	38	47	2	51	49
Patient organisations and/or patient umbrella organisations should be given the opportunity to review the KCE synthesis and give feedback before publication (=consultation).	47	11	23	40	26	44	66



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