Priority Medicines for Europe and the World
"A Public Health Approach to Innovation"

Update on 2004 Background Paper

Background Paper 8.5
Patient and Citizen Involvement

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## Table of Contents

1. **Introduction** .................................................................................................................................................. 3

2. **Approach** .......................................................................................................................................................... 3
   2.1 Literature review: bibliography of patient and citizen involvement in health system decision making .... 3
   2.2 Expert meetings ............................................................................................................................................... 4
   2.3 Survey: Experiences of patient organizations & reimbursement authorities with involvement ............... 4

3. **Drivers for involving patients and citizens priority setting** ........................................................................... 5

4. **Terminology: 'patients and citizens'? or 'the public'?** ..................................................................................... 6

5. **Levels of involvement** ..................................................................................................................................... 7

6. **Structures for involvement** ............................................................................................................................. 10
   6.1 Initial approaches: Surveys and citizens juries ............................................................................................. 10
   6.2 Institutionalized involvement: NICE, EMA and FDA .................................................................................... 11
   6.3 Toolkits: G-I-N, INVOLVE, The Participatory Methods Toolkit and Value+ Toolkit .................................. 12
   6.4 Process models: Dialogue Model and Priority Setting Partnerships ............................................................. 14
   6.5 Quality criteria checklists: two examples by Viergever and Saunders ..................................................... 14
   6.6 Other examples ............................................................................................................................................... 15

7. **Roles and expertise of patients and citizens in health research & policy** ...................................................... 19

8. **Evaluating the impact of patient and citizen involvement** ............................................................................. 20
   8.1 Available evidence: what does it say? ............................................................................................................. 20
   8.2 A framework for evaluating patient and citizen involvement ...................................................................... 23

9. **Conclusion and future strategies** .................................................................................................................. 24
   9.1 Validity and representativeness ..................................................................................................................... 24
   9.2 Framework development ............................................................................................................................... 25
   9.3 Evaluate and learn ......................................................................................................................................... 27
   9.4 Empowerment and capacity building ......................................................................................................... 27
   9.5 Dealing with conflict of interest ................................................................................................................... 29

References.................................................................................................................................................................. 30

Annexes.................................................................................................................................................................... 37
   Annex 8.5.1: Bibliography patient and citizen involvement in health research and policy .............................. 37
   Annex 8.3.2: Meeting report ............................................................................................................................... 55
   Annex 6.8.3: Survey results ............................................................................................................................... 60
   Annex 8.5.4: Viergever’s Checklist for health research priority setting ......................................................... 67
   Annex 8.5.5: Saunders’ criteria and rating scales .............................................................................................. 69

Appendix ................................................................................................................................................................. 71
1 Introduction

This chapter serves as background document for discussion on the involvement of patients and citizens in priority setting for pharmaceutical innovation. At the time of the development of the WHO Priority Medicines for Europe and the World Report in 2004, patient and citizen participation in priority setting was uncommon and knowledge about and experience with the effects of such participation was limited. Currently, involvement of patients and citizens in health research and policy is supported by legal and regulatory requirements. Moreover, there is a substantial body of literature on the topic and much work has been done to realize patient and citizen involvement. This progress indicates that the need for patient and citizen involvement is widely acknowledged by stakeholders in the pharmaceutical innovation process. However, there is a general lack of overview, which hampers further development of best practices for patient and citizen involvement.

The aim of this background paper is to contribute to meaningful patient and citizen involvement in priority setting for pharmaceutical innovation by providing an overview of the current state of knowledge and opinion, and to propose future strategies to improve involvement. We broadened the scope of the paper beyond priority setting for pharmaceutical innovation to the field of health care policy and research. This choice was motivated by the limited literature on patient and citizen involvement specific to the topic of priority setting for pharmaceutical innovation, combined with the rich experience reported in articles about patient and citizen involvement in connected areas (i.e. research design, marketing authorization decisions).

In this background paper, we first describe the approach undertaken to capture the scientific literature on, and the experience with, patient and citizen involvement in health research and policy (Section 2). Next, we briefly discuss the most important drivers for patient and citizen involvement. Subsequently, we look into the question of the difference between ‘patients’ and ‘citizens’ when involvement is desired (Section 4). We then describe ‘the state of field’ with regard to patient and citizen involvement, in four sections: levels of involvement (Section 5), structures for involvement (Section 6), the roles and expertise of patients and citizens (Section 7), evaluating the impact of involvement (Section 8). We finish the paper with a conclusion and suggestions for future research and evaluation (Section 9).

2. Approach

The status with regard to patient and citizen involvement has been investigated using three sources: a review of the literature, two international expert workshops and a survey among patient organizations and public bodies.

2.1 Literature review: bibliography of patient and citizen involvement in health system decision making

The literature on patient and citizen involvement in priority setting is difficult to capture; there is a lack of a common structure, no common terminology, and much of what is known is published in grey literature. For our purpose, we made a bibliography which had a broad
approach: not only literature on priority setting for pharmaceutical innovation was included, but also literature on patient and citizen involvement in a broader sense, such as in biomedical research, guideline development and health policy decision making. We limited our investigations to the health care research and policy setting (including regulatory decision making). See Annex 8.5.1.

Methods

A second source of literature was the NHS/INVOLVE Evidence library (updated September 2012). This is an electronic database of references (reports and articles) that cover:

- the nature and extent of public involvement in research, for example mapping public involvement;
- the impact of public involvement on research;
- reflections on public involvement in research.

Additionally, a manual search of reference lists of papers and grey literature identified in the searches described above was conducted. Grey literature was also obtained by contact with key experts in the field.

In total, 353 articles were extracted. Titles and abstracts were screened and 94 articles were excluded because the topic of patient and citizen involvement was not discussed. In most cases, these articles focused on assessment of systems of pharmaceutical innovation, without reference to patient and citizen involvement. Another 28 articles were not accessible. The bibliography for this background document contains 231 articles and documents.

2.2 Expert meetings
Two meetings with experts were organized. The first was held on 27 September 2012 in Brussels with the aim was to identify critical issues and strategies to overcome barriers. The second meeting was on 22 February 2013. At this meeting a draft version of this background paper was discussed. Both meetings were hosted by Belgium’s National Institute for Health and Disability Insurance (RIZIV/INAMI). Meeting reports of both meetings are in Annex 8.5.2.

2.3 Survey: Experiences of patient organizations and reimbursement authorities with involvement
Patient organizations (IAPO and EPF members) and members of the Pharmaceutical Pricing and Reimbursement Information (PPRI) network were asked to complete a survey on their experiences with patient involvement in priority setting and other forms of decision-making related to pharmaceutical innovations. We received completed survey forms from 17 patient organizations and nine members of the PPRI network. An overview of the results is presented in Annex 8.5.3.
3. **Drivers for involving patients and citizens priority setting**

A variety of underlying motivations drives the efforts to involve patients and citizens in priority setting for pharmaceutical innovation. A first category is political and stems from the desire to promote democratic ideals of legitimacy, transparency and accountability. In 2000, The Council of Europe declared that the right of the public to be involved in the decision making processes affecting health care is a basic and essential part of any democratic society. This democratic right is echoed in government reports, legislation and in statements from patient and citizen groups. Setting (research) priorities affects the use of limited public resources and research demonstrates that values and ethical considerations play a role in recommendations on, for example, guideline development. Therefore, societal values should be considered and decisions should be informed by input from patients and citizens, since they are affected by the decisions. If patients are involved in deciding about priorities in pharmaceutical research, the legitimacy of the studies conducted is enhanced. Additionally, there is some evidence that the inclusion of multiple perspectives is seen as an important element in priority setting.

These motivations are supported by trends towards the empowerment of vulnerable and marginalized groups. Patients are being encouraged to exercise greater control over their own health care and to become more involved in the development of health services. This may also be inspired by a commitment to the principle of respect for autonomy, which would prohibit health researchers to conduct research ‘on’ people as opposed to ‘with’ them. Recent policy reforms in health services in western countries stress the importance of public involvement. Such involvement is organized in local health councils for example in Australia, Canada and the United Kingdom; public consultation, in the state of Oregon (USA) and the province of Ontario (Canada); and regional health conferences, as found in France and Thailand. The United Kingdom has required increased public involvement in the decision making of most health care organizations, their Research Governance Framework for Health and Social Care (2nd edition, 2005) states: ‘Research [should be] pursued with the active involvement of service users and carers including where appropriate, those from hard to reach groups such as the homeless.’ At the EU regulatory level, the European Medicines Agency identified ‘a need and expectation for public bodies to listen to the views and experiences of patients who are the ones most affected by the regulatory decisions and engaging with these stakeholders gives the Agency and the public more confidence and reassurance in its outcomes’.

A second category of motivations are health-related motivations that stem from the need to better align pharmaceutical innovation with the unmet needs of patients. Pharmaceutical innovations do not always meet the needs of patients effectively. According to the National Institute for Health Research in the United Kingdom, ‘involving patients and members of the public in research can lead to better research, clearer outcomes, and faster uptake of new evidence’. Biases within the health research system may tend to favour certain research and topics over others. This could result for example in a lack of interdisciplinary and integral approaches and little attention paid to recovery of patient function. In addition, important questions may be overlooked because of emphasis on: chronic but not acute conditions; severe but not common health problems; and disease-specific but not crosscutting issues, such as social care, improved surgery, and anaesthesia.
Evidence shows that health professionals’ values of different health states and research priorities differ from those of patients.\textsuperscript{19, 20} For example, the values physicians assign to stroke as an outcome and to adverse consequences (e.g., gastrointestinal bleeding) of treatment to prevent stroke in patients with atrial fibrillation differ from those of patients.\textsuperscript{21} Patients at high risk for atrial fibrillation placed more value on the avoidance of stroke and less value on the avoidance of bleeding than did physicians who treat patients with atrial fibrillation. Other divergences in priorities for health research between professionals and the public were found in the areas of arthritis, Alzheimer disease, and mental health.\textsuperscript{22} Based on experience with priority setting for Health Technology Assessment, Bastian et al. suggested that the interests of patients and other end users of information at the societal level may even be the opposite of the concerns of policy makers.\textsuperscript{23} Public involvement in decision making processes could be a route to enhance the relevance and quality of health research.

Another argument relevant to health related motivation focuses on the actual contribution patients can make to the decision making process and thus to the rationality of the process and the quality of its direct or long-term outcome. Beresford, for example, argues that, “the shorter the distance between direct experience and interpretation (for example as can be offered by user involvement in research), then the less distorted, inaccurate and damaging resulting knowledge can be.”\textsuperscript{24} Patients not only have a right to engage in discussions on decision making about priorities (the political stance), their input is also needed, because they have a specific, relevant type of knowledge: their ‘experiential knowledge’.\textsuperscript{25, 26, 27}

In a third and final category of motivations, patient and citizen involvement can be promoted by arguments of transparency and trust. For example, an analysis of the benefits of patient involvement by the EMA lead the investigators to conclude: “participation of patients in the scientific committees leads to increased transparency and trust in regulatory processes and develops mutual respect between regulators and the community of patients. It is also acknowledged that their contribution enriches the quality of the opinion given by the scientific committees.”\textsuperscript{28}

These motivations provide a strong justification for efforts to further develop patient and citizen involvement in priority setting. A next step is to create an evidence base for meaningful models of involvement. At present, there is a lack of an overview of various initiatives undertaken and several knowledge gaps exist; together these are hampering efforts to evaluate and further develop patient and citizen involvement in priority setting.

4. Terminology: ‘patients and citizens’? or ‘the public’?

In this background paper, we discuss the available knowledge and experience on patient and citizen involvement. A preliminary question is whether patients and citizens need to be viewed as separate groups. In general, patient involvement is distinguished from public (or citizen) involvement.

However in, for example, the United Kingdom, policy guidelines on involvement usually refer to ‘the public’, including: “Patients and potential patients; people who use health and social services; informal carers; parents/guardians; disabled people; members of the public who are potential recipients of health promotion programmes, public health programmes and social service
interventions; and organizations that represent people who use services.” In some cases, the term ‘stakeholder’ is used. Stakeholders are those who have a legitimate stake in an issue, independently of whether these actors have decisional power. Since the group of stakeholders in priority setting for pharmaceutical innovation is much broader than patients and citizens, this term seems less adequate for our purpose.

While there is widespread belief that values for health states differ between patients and the general public, there is a longstanding debate among health economists about the evidence to support this belief. A systematic review of 33 studies found that preferences for hypothetical health states did not differ between patients and the public. Another meta-analysis demonstrated that patient preferences for an actual health state do not differ significantly from population preferences for a hypothetical health state. These findings suggest that patient and population preferences can both be used to set priorities for pharmaceutical innovation. However, Peeters and Stiggelbout claim that patients give higher valuations to health states compared to members of the general public, based on a meta-analysis of studies reporting valuations given by patients and non-patients. Hence, there is evidence for treating patients and citizens as different populations, as well as for the use of one term (‘the public’) for both patients and citizens.

In general it seems that that patients and citizens involvement can be captured by the term ‘involvement of the public’ or ‘public involvement’ in many cases but not all. First, patients and citizens may have competing or contrasting interests in priority setting for pharmaceutical innovation. For example, it is in the general interest of citizens and society to have medicines with a favourable cost-benefit profile. For patients, access to the best available treatment is the primary interest. In setting priorities, these interests will not always converge. Second, there are circumstances that ask for a more specific use of experience. This is the case when involvement is sought with the explicit aim to grasp the experiential knowledge of a patient, or a well-described group of patients or carers. For our recommendations we will use ‘patients’ and ‘citizens’ where appropriate and distinguish between the two terms only when necessary.

In this paper, we give an overview of the literature on patient and citizen involvement. We will follow the terminology used in a study, report or article when we refer to it in this paper.

5. Levels of involvement

Patient and citizen involvement is organized in numerous ways, though these approaches are considered to be not equally meaningful. The ‘participation ladder’ is a conceptual model that can be used to assess different types of patients and citizen involvement in decision-making. Arnstein published the participation ladder to distinguish degrees of citizen control over decisions in 1969. The ‘ladder’ aims to categorise different sorts of participation, ranging from an illusory form of powerless participation to citizen control. Abma et al. adapted the model in 2010 to analyse patient participation initiatives.
Discussing of the model proposed by Arnstein in some more detail could give an overview of the general types of involvement in decision-making. The participation ladder has eight rungs: Manipulation, Therapy, Informing, Consultation, Placation, Partnership, Delegated Power and Citizen Control (see Figure 8.5.1).

The first two levels of involvement are actually forms of non-participation: they are ‘Manipulation’ and ‘Therapy’. Examples of such ‘illusory forms’ of participation are placing people on advisory committees for the express purpose of ‘educating’ them: patients and citizens are subjected to advice and persuasion from the experts who retain their full decision making power.

Tokenism is the label for the next three levels: ‘Informing’, ‘Consultation’ and ‘Placation’. These forms of involvement can be a first step towards genuine participation: they allow patients and citizens to have a voice. However, at the tokenism-level their influence on policy-making can be restricted by practical and financial structures, differing knowledge bases, cultural barriers and personal attitudes. Examples of tokenism-level involvement are providing information to patients, the collection of patients’ and citizens’ views (e.g. through surveys, opinion polls, interviews or focus groups) and inviting patients and citizens in advisory bodies without giving them any decision making power.

Actual patient and citizen decision-making power is achieved in the highest three rungs of the participation ladder: through Partnership, Delegated Power and Patient/Citizen Control.
Partnership enables patients and citizens to negotiate and engage in trade-offs with traditional power holders. Delegated Power implies that citizens and patients obtain the majority of decision making seats. At the top of the ladder is Patient and Citizen Control, which refers to a situation where decision making power is transferred from experts to patients and citizens.

Arnstein described the participation ladder with the aim of separating meaningful involvement of citizens in political decision making from illusory forms of participation. The model has been criticized for its sole emphasis on power. This may close off other relevant considerations such as the existence of different relevant forms of knowledge and expertise. Also, the focus on gaining decision-making power may limit effective responses to the challenge of involving patients and citizens. For example, expecting people to participate in formal election processes to attain a position on a board or committee may exclude members of populations that are both more likely to require health services and, historically, have been less well served. Another limitation is the fact that the question of how effective patient and citizen involvement should be organized, cannot be answered by referring to the participation ladder. Moreover, it should not be concluded that ‘higher’ rungs on the ladder are the best for each situation.

The participation ladder is also used by authors in the field of health policy and research. For example INVOLVE, the national advisory group on public involvement in health research of the United Kingdom’s National Institute for Health Research have presented a condensed ladder of participation with three steps: consultation, collaboration and lay control. Consultation is defined as asking lay people for their views and using those views to inform decision-making. For example, funders of research have held one-off meetings with people to ask them about their priorities for research. Collaboration involves an on-going partnership between researchers and the members of the public, where decisions about the research are shared. For example, members of the public might collaborate with the researchers on developing the research grant application, be members of the study advisory group and collaborate with researchers to disseminate the results of a research project. Lay-controlled or user-controlled research is research that is actively controlled, directed and managed by service users and their service user organizations. Professionals are only involved by invitation. INVOLVE reported several examples of user-controlled research. One example is a project carried out by Thyroid UK, a small registered charity run by people with direct experience of thyroid and related problems with the aim of providing information and resources to promote effective diagnosis and appropriate treatment for people with thyroid disorders in the United Kingdom. The personal experience of some of their members (people with continuing problems despite blood test results that fall within the normal range) prompted this research, a clinical trial to examine and compare the accuracy of two different tests (blood and urine) in relation to people’s symptoms.

Similar categorizations of levels of public involvement have been identified by for example Boote et al. and Steyaert. They were also used in the Value+Toolkit, which was developed in 2010 by patients organisations and the European Commission (EC) to support the exchange of information and experience on good practice relating to patient involvement in EC projects.
6. **Structures for involvement**

Patient and citizen involvement is a broad subject, covering a wide range of activities, policies, and research. Reviews of ‘service user involvement’ in health and social care research have highlighted a wide range of theoretical approaches and conceptual models, indicating how widespread it has now become. The literature on structures for involvement is comprised of mainly qualitative or case study reflections of patient and citizen involvement, or cross-sectional studies reporting individual or organizational views of involvement, with relatively little critical evaluation. The available information is very often descriptive and very seldom conceptual or evaluative. While involvement of patients and citizens is becoming more frequent as partners in research projects, for example as members of supervision committees, consultation of patients and citizens at the beginning to identify and prioritize areas for research is still rare.

The various approaches that are available to guide priority setting for health research differ on important aspects (role, position in process, responsibility). Consensus on a gold standard or best practice for health research prioritization thus seems difficult to achieve and is, more importantly, disqualified by some as ‘not an appropriate response’.

To grasp the experiences with patient and citizen involvement, we analyzed publications on models and strategies for involvement. Our aim was to provide a structure for the knowledge available. Literature on priority setting for pharmaceutical innovation is limited, and often involves case description. Therefore, our scope was broadened to health policy decision-making and medical research. In this section we describe several approaches: surveys and citizens juries; institutionalized involvement; toolkits; dialogue model and priority setting partnerships; and checklists and criteria. Finally, a list of published examples is added to illustrate the variety and nature of employed strategies.

6.1 **Initial approaches: Surveys and citizens juries**

In brief, citizens juries bring together members of the public (jurors), and provide a structured discussion of relevant information provided by expert witnesses. Facilitators or moderators are present to guide the process. The end result is a written report authored by the jurors. An example of a health priority setting process with jury input is in Box 8.5.1. In a comprehensive overview of the literature on public participation processes, Abelson et al. report that the National Health Service (NHS) in the United Kingdom started exploring possibilities for a greater role for public views in setting health care priorities in the early 1990s. Mail surveys and interviewer-administered surveys were the initial method of choice, but their ability to generate meaningful insight was limited. This lead to a search for new (deliberative) public involvement methods. In the mid-1990s citizens juries became popular for priority setting processes in the United Kingdom and New Zealand. Several juries have dealt with questions of whom should set priorities and how; others were asked to allocate resources within or between programme areas. There are considerable challenges to the process, and it has become clear that juries are imperfect means of ensuring democracy, representation and influence.
6.2 Institutionalized involvement: NICE, EMA and FDA

The task of giving guidance on which treatments or services should be provided out of public funding by the NHS and which should not, was given to the National Institute for Health and Clinical Excellence (NICE) on its creation by the United Kingdom government in 1998. As such, it has a fair degree of independence. NICE produces guidance in three areas of health: public health, health technologies and clinical practice. NICE has adopted a very comprehensive approach to involving patients and consumers. The activities can be categorized into four broad areas:

(i) Stakeholder consultation

Organizations can register and comment at any stage during the clinical guideline development process from the suggestion of guideline topics, drafting of scopes, development and initial drafting of guidelines, to the second consultation draft.

(ii) Direct input

All NICE committees and working groups are expected to include at least two members who play a crucial role by providing a patient/carer perspective to their discussions and decisions. They may be patients, carers or patient advocates.

(iii) Indirect input

Examples include focus groups with patients, patient written testimonials and video-taped interviews with patients that are presented to a technology appraisal committee.

(iv) Dissemination of NICE guidance to and by patients

All NICE guidance is produced in versions written for patients, carers and the public.

The European Medicines Agency is an agency of the European Union, located in London. The Agency is responsible for the scientific evaluation of medicines developed by pharmaceutical companies for use in the European Union. It has a Working Party with
Patients’ and Consumers’ Organizations (PCWP). It implemented the ‘Framework on the interaction between the Agency and the patients’ and consumers’ organizations’, which has established formal interaction between patients'/consumers’ organizations and the EMA. PCWP members monitor patient participation in the varied activities within the Agency, i.e. the review of information for the general public, participation in scientific advisory group meetings, Committee/Working party consultations, participants in conferences and workshops. One method to involve patients in decision-making is their inclusion on committees. For example, at EMA patients are included as formal members in the Agency’s Scientific Committees, such as the Committee for Orphan Medicinal Products (COMP), the Paediatric Committee (PDCO) and the Committee for Advanced Therapies (CAT). The tasks of patient members include:

(i) *Participation* in the assessment of applications and in peer reviews.
(ii) *Providing advice* on the identification of external experts.
(iii) *Collaboration* in the preparation of public summaries of opinion.  

The U.S. Food and Drug Administration (FDA) has organized patient participation in advisory committees, a consultation program and public hearings. These are organized under three headings:

(i) Patient Representative Program: responsible for providing the FDA and the Advisory Committees the unique perspective of patients and family members directly affected by a serious or life-threatening disease. Patient Representatives are invited to participate in FDA Advisory Committee meetings to discuss medical products for the treatment of serious or life-threatening diseases such as cancer or AIDS. These FDA advisory committees provide independent expert advice and recommendations to the Agency on scientific, technical, and policy matters related to FDA-regulated products.

(ii) Drug Development Patient Consultant Programme: incorporates the perspective of patient advocates into the drug development process allowing them an opportunity to participate in the FDA drug review regulatory process.

(iii) Open Public Hearings: Every Advisory Committee meeting includes an open public hearing (OPH) session, during which interested persons may present relevant information or views orally or in writing (21 CFR 14.25(a)). The FDA may also solicitate the opinion of stakeholders by organizing a public hearing.

**6.3 Toolkits: G-I-N, INVOLVE, The Participatory Methods Toolkit and Value+ Toolkit**

The Guidelines International Network, G-I-N, is a global network, founded in 2002. By 2013 it has grown to comprise 92 guideline organizations, and 127 individual members (implementers, end-users, researchers, students and other stakeholders) representing 48 countries from all continents. G-I-N aims to promote evidence-based guideline development, adaptation, dissemination and implementation. Guideline organizations use a number of different methods to involve patients and the public. In the ‘G-I-N PUBLIC toolkit’ three general involvement strategies are presented. The toolkit provides practical advice on how to implement these methods successfully.
(i) **Consultation strategies**: involve the collection of information from patients and the public. This can include methods such as surveys, focus groups, individual interviews, online consultation, the use of primary research on patients’ needs and expectations, or the use of a systematic review of studies on patients’ and the public’s perspective.

(ii) **Participation**: involves the exchange of information between guideline developers and the public. This can be done through participation of patient and public representatives on guideline development groups and other methods.

(iii) **Communication strategies**: involve the communication of information to patients and the public to support their individual health care decisions and choices. This can include the production of plain language versions of clinical practice guidelines or the development of patient decision aids or education material.

The United Kingdom based intermediary organization INVOLVE aims to stimulate and support active participation of the public in NHS, public health and social care research. INVOLVE is part of the National Institute of Health Research (NIHR). Recently, a collection of briefing notes for researchers was published by INVOLVE. It refers to numerous examples of public involvement in a range of health research activities. These include helping to develop the research question, applying for funding and ethical approval, sitting on advisory groups, carrying out the research and disseminating the research findings. Briefing Note Eight considers the different ways members of the public can get involved in the stages of the research cycle.

The Participatory Methods Toolkit from the King Baudouin Foundation offers an overview of techniques to actively involve ‘the public’ in decision-making processes. The public can be regular citizens, the stakeholders of a particular project or policy, experts and even members of government and private industry. The approach can be applied by various organizations that wish to engage a broad range of perspectives in decision making processes. It is the aim of participatory methods to emphasize the processes of democracy by giving structure and organization to various forms of dialogue. It invites pluralism, diversity, and dissent with the ambition to examine issues from as many angles as possible in order to find the best common solution. The contents of the toolkit comprise a description of thirteen techniques and methods, for example consensus conference, deliberative polling, Delphi methods and expert panels. Moreover, a comparative chart for the 13 participatory methods is given. Finally, in-depth general guidelines and tips for conducting participatory methods are described.

The Value+Toolkit is the result of a project to support the exchange of information and experience on good practice relating to patient involvement in projects co-financed by the EC. The Value+Toolkit was developed by patient organisations and is divided into chapters covering several topics. Among these are:

(i) **Meaningful Patient Involvement.** This chapter includes the Value+research findings on the barriers and challenges to patient involvement and good practice in patient involvement.

(ii) **Your Own Organisation and Meaningful Patient Involvement.** In this chapter, basic information which may help patient organisations prepare themselves for taking on an EC-funded project is provided.

(iii) **Resources** contains tools and examples from Value+ and other sources, examples of good practice, a list of websites, the Value+ Literature Review, a list of patient
organisations that operate at European and national level, information on patient rights specific to individual countries, and national contacts for the European Commission.

The Value+Toolkit has been published with the support of the European Commission, Directorate General for Health and Consumers under the Public Health Programme 2008-2013.

6.4 Process models: Dialogue Model and Priority Setting Partnerships

The Dialogue Model is based on the idea that in order to give patients a real voice in decisions on health research, they need to be involved as partners. In this model, research is not framed by the relevant interests of the scientists but developed in interaction with various stakeholders.

The overall process is a dynamic and cyclic process of activities, in which tentative results are tested and refined in practice in an iterative way. The activities are structured in roughly six phases:

1. **Initiation and preparation**, in which the project team is established, an initial assessment is made of the problems, ideas and wishes of patients and other stakeholders, and a start is made with creating conducive social conditions.

2. **Consultation**: in which various stakeholder groups are consulted separately to develop different lists of issues that are relevant from the perspectives of the different stakeholder groups.

3. **Prioritization**: in which stakeholder groups value the different research topics from their lists and rank them in order of importance, resulting in different tentative research agendas.

4. **Integration**: in which stakeholders exchange information, address conflicts and integrate research agendas through dialogue, resulting in one integral research agenda.

5. **Programming**: in which the integral research agenda is translated into a coherent program or action plan.

6. **Implementation**: in which participants determine and take action, monitor progress and evaluate results.

Priority Setting Partnerships (PSPs) unite patients or carers, or both, with clinicians (or representative groups of patients, carers, or clinicians) to prioritise treatment uncertainties for research using consensus development methods. The James Lind Alliance (JLA) has published a Guidebook to establishing PSPs. The activities of PSPs take place in five phases:

1. **Identification and invitation of potential partners**;
2. **Initial stakeholder meeting**;
3. **Identifying treatment uncertainties**;
4. **Refining questions and uncertainties**; and
5. **Prioritisation**.

6.5 Quality criteria checklists: two examples by Viergever and Saunders

Some authors have described a health research priority setting process to assist researchers and policymakers in effectively targeting research. Taking the heterogeneous nature of research priority setting exercises into account, we present two proposals for a priority setting process that explicitly address involvement of stakeholders.
Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement

First, a checklist for health research priority setting developed by Viergever et al. explicitly addresses stakeholder involvement (see Annex 8.5.4). Under the heading of inclusiveness, the checklist demands that it should be decided which stakeholders need to be involved in the research priority setting exercise, why their opinions need to be sought and what role they should play in the process. Among the potential roles that stakeholders can play in the process are: providing opinion, providing evidence and being a part of the group that decides on priorities.

Second, a strategy of using a set of criteria to evaluate research that reflects consumer values is suggested by Saunders et al (Annex 8.5.5). The values held by cancer consumers and the wider community with regard to research were identified and combined in optimal rating scales to evaluate research. The relevance to priority setting for pharmaceutical innovation is that is offers a framework to incorporate consumer needs into the process of judging and allocating research grants.

6.6 Other examples

Besides the more general approaches presented above, there is a rich body of publications on examples of patient and citizen involvement in priority setting, health research, systematic reviews, clinical guideline development and health policy decision making.

IAPO Policy Framework

The International Alliance of Patient Organizations has a Policy Framework in which they state a number of priorities. Moreover, they put in place three methods to identify and prioritise policy issues and activities. These methods are: (i) input of members and stakeholders, (ii) identification of issues by IAPO staff and Governing Board facilitated by on-going research activities and (iii) annual online consultation of members.

Patient-Centered Outcomes Research Institute (PCORI)

PCORI helps people and their caregivers communicate and make informed health care decisions, allowing their voices to be heard in assessing the value of health care options. In 2010, the United States Patient Protection and Affordable Care Act created the PCORI. This was established to emphasize the critical importance of a patient-centred perspective in conducting research on comparative effectiveness of clinical interventions. Its mission statement commits to producing and promoting high-integrity research that is guided by patients, caregivers and the broader health care community. PCORI has proposed national priorities for research, following an extensive procedure of stakeholder consultations.

The Cochrane Agenda and Priority Setting Methods Group (CAPSMG)

In 2011 the CAPSMG was established. It is one of 16 Cochrane Methods Groups established to develop methodology and advise The Cochrane Collaboration on how the validity and precision of systematic reviews can be improved. The CAPSMG aims to inform the Cochrane entities about the empirical evidence available on methods to set a research agenda or establish top research priorities. In addition, it will endeavour to serve as a discussion forum.
Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement

connecting people interested in methods to set research agendas or priorities inside and out of the Cochrane Collaboration.69

**Short bibliography of other examples**

The following short bibliography aims to illustrate the variety of patient and citizen involvement activities published in scientific journals. These articles mainly describe isolated instances of involvement across a variety of health research and policy making activities.

*(i) Priority setting*


Bruni RA, Laupacis A, Levinson W, Martin DK. Public involvement in the priority setting activities of a wait time management initiative: a qualitative case study. BMC Health Serv Res. 2007 Nov 16;7:186.

Buckley BS, Grant AM, Tincello DG, Wagg AS, Firkins L. Prioritizing research: patients, carers, and clinicians working together to identify and prioritize important clinical uncertainties in urinary incontinence. Neurourology and Urodynamics, 2010; 29: 708–714.


Wright D, Corner J, Hopkinson J, Foster C. Listening to the views of people affected by cancer about cancer research: an example of participatory research in setting the cancer research agenda. Health Expectations, 2006, 9(1), 3-12
(ii) Involvement in Health Research


Patient Partner Project to promote the role of patient organizations in the clinical trials: context www.patientpartner-europe.eu (finalized in 2011)


(iii) Systematic reviews


(iv) Clinical guideline development


(v) Health policy

7. Roles and expertise of patients and citizens in health research & policy

The literature on roles of patients and citizens in health policy and research seems to reflect broad agreement on the idea that meaningful involvement requires more than a ‘tokenistic’ approach. Arnstein’s ladder of involvement and its variations illustrate a different levels of involvement, based on a measure of control over the decision making process. The rich variety of structures for involvement that have been employed in the field of health policy and research is a sign of a developing field of expertise and experience. One aspect of patient and citizen involvement however seems to lag behind in this process: developing good understanding of the expertise and the contribution of patients and citizens at different levels of involvement (i.e. consultation, collaboration and control) and in a variety of models.

There is a major lack of reflection of the very concept of patient and citizen involvement. What is it exactly that a patient or a citizen brings to the table in decision making processes? How can this be defined and measured? Concepts have been used interchangeably, with patient and citizen involvement variably defined and often poorly described. One of the few descriptions of different roles patients can play comes from EMA. In a report on the role of patients as members of scientific committees, EMA states: “The added value of having patients and consumers in the scientific committees is to bring a unique and critical input based on their real-life experience of being affected by a disease and its current therapeutic environment. This element fills a gap that other committee members (so-called scientific experts) cannot fill”. More in detail, EMA described the following roles:

1. **Expertise**
   - Convey a combination of specific education, training or professional experience

2. **Experience**
   - Convey practical disease knowledge obtained from direct contact with the disease (affected person or close contact with affected person, e.g. family, carer)

3. **Advocacy**
   - Act on behalf of the affected patients in defence of their rights; provide patient-oriented public health/health care policy perspective

4. **Empowerment**
   - Participate in decision making process within the committee; having access to information and process on behalf of patients.

Despite the value of this description, a deeper understanding of the nature and value of patient and citizen involvement is needed. Currently, the conceptual basis of patient and citizen involvement is limited to reference to experiential knowledge of patients. For citizens, being a ‘lay person’ is their task. For both, it remains an open question as to how their knowledge and perspective should be organized. Are patients asked to bring forward only their personal experience? Should patients and citizens involved collect the experiences and preferences from the groups they represent? Descriptions of levels and structures for involvement are not sufficient to answer these questions, and this is problematic since the concepts of patient and citizen knowledge and perspective form the building blocks of frameworks for involvement.

This point is well illustrated by a recent discussion of patient and public involvement by Ives et al. in their paper addressing a possible paradox in patient and public involvement (PPI); the “PPI paradox”. This paradox is constituted as follows; the value of patient and public
involvement is in the ‘lay’ perspective on research, it is the experience of being a service user or member of the public that justifies their involvement. Efforts to access this expertise can however compromise the ‘lay status’ of the patient or citizen involved. This risk of the lay perspective being tainted is absent at the lower levels of involvement, for example, if lay expertise is accessed through a simple consultative model. However, patient and public involvement in this form is not usually considered sufficient. Patients and citizens who are involved at a level that is higher on Arnstein’s ladder, need adequate training to be able contribute substantially to the scientific process. But once a ‘lay person’ undergoes training, and becomes familiar enough with research to be substantially involved, their ‘lay’ status is compromised and they become ‘more expert’. And even though they can still contribute to the process in a way that is informed by their own experience of illness or disability, their lay perspective is at risk of being ‘tamed’ to make it more congruous with that of the professional researcher. A critique on this idea came from Staley, who argued that the views of the lay person are complementary to those of the technical experts. Lay people know what research would help them, how to make participation a positive experience and how best to communicate the findings to a lay audience.  

Progress on developing a conceptual underpinning for patient and public involvement can resolve these uncertainties. A critical appraisal of different structures for involvement can identify different meaningful combinations of content, level and structure of patient and citizen contributions to the scientific process.  

8. Evaluating the impact of patient and citizen involvement

The wide range of policies and other initiatives that have now been used to shape a practice of patient and citizen involvement, raise the question of their impact. A review of examples of public involvement at the design stage of primary health research by Boote et al. in 2010 found only one study that reported an explicit attempt to measure the impact this involvement. Other authors concluded that the evidence base on the impact and benefits of patient and citizen involvement in research is still small and that much of the evidence consists of descriptive, often retrospective, accounts of involvement. Stewart et al., analyzed literature on identifying research priorities and they found that only nine out of 258 papers which addressed this topic reported patient involvement. Oliver et al. systematically reviewed different methods of consumer involvement in research priority setting. They concluded that "what we know about the advantages and disadvantages of methods for involving consumers in agenda setting rests on weak short-term evidence and almost entirely speculative long-term evidence". Currently, calls are being made for more rigorous evaluation of the process and the impact of involvement.

8.1 Available evidence: what does it say?

Research into the impact of patient and citizen involvement in health research and policy can be categorized into knowledge of the impact of involvement on research processes and outcomes and impact on the patients and citizens who were involved. Finally, there is some knowledge on the impact of patient and citizen involvement on professionals.
Impact on research processes and outcomes

Recently, Barber et al. analyzed literature on evaluation of service involvement in identifying and prioritizing research. They conclude that a variety of impacts have been reported:

(i) Involving service users increases the range of research topics, highlighting issues of importance to service users and identifying new themes.
(ii) Service user involvement ‘pushed the science forward more quickly’.
(iii) Service user involvement at the research design stage lead to a more ethically acceptable research design, has contributed to improving trial consent procedures and enhanced recruitment and accrual rates.
(iv) Where studies have used service users as co-researchers and interviewers, the quality of the data was influenced in a positive way.
(v) Service user involvement in analyzing data includes questioning the interpretations of researchers and modifying researchers’ misinterpretations, and making adjustments to how findings have been reported.
(vi) Service user involvement in disseminating research findings was said to enhance the power and credibility of the findings, leading to wider and more accessible dissemination.

A few years earlier, Staley assessed where there appears to be the most evidence for the impact of public involvement in research and identified four themes:

(i) Public involvement was reported to increase recruitment to all types of research;
(ii) Public involvement was reported to be of particular value in qualitative research where participants are asked to share their views and experiences;
(iii) Public involvement was reported to be of particular value in clinical trials where it helped to improve trial design and ensured the use of relevant outcome measures;
(iv) Public involvement was most frequently reported to benefit the people involved as well as the research participants.

Nilsen et al. reviewed evidence from randomized controlled trials on methods of consumer involvement in developing healthcare policy and research, clinical practice guidelines and patient information material. They concluded that evidence of the effects of consumer involvement in healthcare decisions at the population level is weak, but also report some evidence for positive effects:

(i) Moderate quality evidence shows that involving consumers in the development of patient information material results in material that is more relevant, readable and understandable to patients, without affecting their anxiety. This ‘consumer-informed’ material can also improve patients’ knowledge;
(ii) Low quality evidence shows that using consumer interviewers instead of staff interviewers in satisfaction surveys can have a small influence on the survey results;
(iii) Low quality evidence that an informed consent document developed with consumer input (potential trial participants) may have little if any impact on understanding compared to a consent document developed by trial investigators only;
(iv) There is very low quality evidence that telephone discussions and face-to-face group meetings engage consumers better than mailed surveys in order to set priorities for community health goals. They also result in different priorities being set for these goals.

Despite these reports of limited evidence for the effects of patient and citizen involvement, there are also studies with opposing conclusions. Domecq Garces JP, et al. reported that they found ample evidence suggesting that engagement of patients in patient centered outcome
research improved study design (by choosing outcomes more meaningful to patients or designs that are more culturally sensitive or consistent with patients’ context), execution (improving subject recruitment and retention) and translation (better implementation, dissemination and uptake). Others identified tensions and barriers between different stakeholders at the research design stage concerning variable levels of understanding of service users about health research methods, time and costs, and difficulties raised when researchers used jargon and complex language.

Assessment of the impact of patient and citizen involvement is complicated by the way experiences are reported in the literature. In general, these descriptions are often brief and provide limited evidence of impact. Concepts like consultation, representation, and expertise have been used interchangeably, with patient and citizen involvement variably defined and often poorly described. Longer qualitative descriptions often provide a better insight into impact. However, while such descriptions can be very valuable, they provide no indication of the extent of impact, its magnitude or how it compares across different areas of impact.

Impact on patients and citizens involved

In a recent review of the literature on evaluation of service involvement in identifying and prioritizing research Barber et al., stated that service user involvement is associated with empowerment and strengthening of the service user voice. In addition, service users describe increased knowledge, skills and confidence, and support from others in user groups. A study of experiences of cancer patients with involvement activities in cancer services, palliative care and research, revealed that many participants described their involvement activity as a positive way to keep active, to combat depression and loneliness, and as a way to deal with their (cancer) diagnosis and treatment.

Fudge et al. assessed user involvement in health service development in an ethnographic study. They found that service users discussed the impact of involvement primarily in terms of personal gains. For example, they reported satisfaction in feeling that professionals were listening to them, that their ideas were acted on, and that their experience was being harnessed to help others. Service users were observed engaging with the programme for the social opportunities it provided. Finally, service users described their involvement as helping to increase their knowledge and understanding of their condition. When asked about how their involvement had improved services, few service users could directly answer the question.

In other studies, some negative consequences, such as feeling overburdened, reliving distressing memories, hearing stark medical details or being referred to as ‘professional users’, have also been reported.

Impact on professionals

We know little about the effects of service user involvement in research on researchers, but there are suggestions that such involvement has led to researchers developing a deeper understanding of service user issues, and prompted them to challenge their own beliefs and assumptions. However, Staley reported that researchers who have reflected on involvement of the public in research, conclude that it is difficult to assess the impact of involvement or to predict where involvement would have the greatest impact. Other patients have voiced perceived threats to professional skills and knowledge: “They are coming in and suddenly they
are the experts and they have done no studying, no qualifications and I think she feels a bit kind of like that’s not right, their experience cannot outweigh my academic qualifications and knowledge.”

Also, researchers generally seem to find it difficult to give up control in order to share knowledge and power and have learnt to espouse scientific methodologies that typically exclude “lay people”. Within professional groupings, Fudge et al. identified two categories. Firstly, professionals who viewed user involvement as an exercise in democracy and promoted patients’ expertise as valid as that of professionals were identified. Secondly, there were those who unquestionably enacted out the policy of involvement as a directive to be implemented as part of a patient centred NHS.

8.2 A framework for evaluating patient and citizen involvement

Ultimately, the effectiveness of any public participation or consultation process should be judged by some measure of the outcomes achieved. There is no agreement on desirable or appropriate outcomes. For example, a change in research priorities resulting from patient involvement may not necessarily be a change for the better for all patients. The reported effect that patient involvement ‘pushed the science forward more quickly’ may or may not have had a positive effect on the quality of the scientific result and a single patient may be a good representative for some patients from a certain country or (sub-)culture, but not for others. Debate within the public participation literature divides between those concerned more with process measures against those more interested in what the influence of involvement is on the final decisions taken. Abelson et al. argue for a stronger evidence base to evaluate patient and citizen involvement. To facilitate evaluation of deliberative processes they propose four elements that need to be assessed:

I Representation: To what extent are different types of representation achieved (e.g. geographic, demographic or political)? Consultation processes may also be assessed against criteria that emphasize both access to a consultation (by providing equal opportunities) as well as clarity and legitimacy in the selection process.

II Structure of the process or procedures: Are the procedural aspects of a consultation process legitimate, reasonable, responsive and fair? These are considered fundamental aspects of the evaluation process. Legitimacy and responsiveness principles are assessed by considering questions such as: What point in the decision making process is public input being sought? (i.e., is the public involved in significant aspects of decision making, such as agenda setting, or in minor decisions only?); At what level of the organization does the participation occur? Evaluations of deliberative processes in particular would also assess elements of the process such as: (1) Was ample time provided for discussion? (2) Did participants have the opportunity to challenge the information presented? (3) Was mutual respect and concern for others emphasized throughout deliberations?

III Information: What and how is information selected, presented and interpreted? These are crucial elements of any consultation process and are therefore important evaluation principles to consider.

IV Outcomes and decisions arising from the process: The final set of evaluation principles considers the various sets of potential outcomes of the public participation process. Elements to consider include an assessment of the extent to which public input was incorporated into the final decisions, how decisions and the public’s input into these decisions were
communicated to the public, and the degree to which the decision making authority was found to respond to the public’s input (i.e., what aspects of the input did they incorporate or not incorporate and why?). Secondly, participants must be satisfied with the process which must lead to a more informed citizenry with a better understanding of the issue. Thirdly, an important outcome is the extent to which consensus was achieved and finally, it must be asked if better decisions were taken and the participation process improved policy making (i.e., did the process make a difference to the final decision).

9. Conclusion and future strategies

The number of initiatives that have been employed to organize patient and citizen involvement in health research and policy reflect broad acknowledgement of its value and importance. The variety of efforts is, however, also a symptom of ambiguity and an unfinished search for best practices. In this section, we propose future (research) strategies to maximize the potential benefits of patient and citizen involvement in priority setting for pharmaceutical innovation. We refer to knowledge on patient and citizen involvement in the literature, combined with the results of a survey and the outcomes of two meetings with representatives from stakeholders (i.e. patient organizations, regulators, pharmaceutical industry and scientists).

9.1 Validity and representativeness

One of the main arguments for patient involvement concerns the contributions that patients could make to the relevance and quality of biomedical research based on their ‘experiential knowledge’. However, the validity of patients’ experiential knowledge in the context of biomedical research processes raises a number of questions: To what extent is the experienced perception of a patient representative credible? And, how can this specific knowledge be absorbed into the scientific process? What methods can be used to enhance the credibility of the contribution of patients and citizens to the decision making? Questions of validity need to be addressed since they limit acceptance of non-expert involvement. While patient and citizen organizations struggle to demonstrate credibility, their position may be undermined by ambiguity in their roles and the goals of their involvement in priority-setting and decision making.

The debate on representativeness of patients and citizens is long standing. Many researchers question whether or not anyone involved could be ‘representative’ of patients or the public in a broader sense – even when a study involved people who had experienced the condition being explored. On the contrary, supporters of involvement have insisted that we focus on inclusion and diversity of participants rather than representativeness. More work is needed to ascertain whether the views of those involved are the same as those not involved and whether user involvement is leading to inequalities—providing benefits to those involved over those who are not. The potential PPI paradox—the lay perspective being ‘tamed’ to fit the scientific process by training of patient and citizen representatives—needs to be addressed for its potential threat to validity and representativeness.
9.2 Framework development

Organizing meaningful patient and citizen involvement is highly complex. As stated above, the wide variety of approaches for patient and citizen involvement has added to experience, but this has not yet resulted in a widely accepted model or a framework for meaningful involvement. Such a framework is needed to ground patient and citizen involvement in an evidence base and to optimize its practice. In the literature, a call is made for consistent and explicit terminology to describe stakeholders and engagement methods. Taking the task of developing a framework, implies the opportunity to start clarifying some of the ambiguity that currently exists, for example by identifying the areas of impact that should be included in an instrument to ensure its content validity. It would also offer the opportunity of explaining how important elements such as the context and process of patient and citizen involvement are considered. Currently, several knowledge gaps hamper the development of a framework for patient and citizen involvement in priority setting for pharmaceutical innovation.

(i) Lack of oversight of initiatives for involvement. Patients and citizens are involved in health research and policy, but there is a lack of clarity on their role and expertise. The experiences reported in the literature stem from a broad field: from patient and citizen involvement in medical research (i.e. supporting the recruitment of research participants) to involvement in committees responsible for marketing authorization of new medicines (i.e. at the EMA). Among and within patient and citizen organizations, there is no consensus on the goals of involvement in priority setting. Experts involved in the meetings to support this background paper observed a lack of knowledge and understanding of patient and citizen involvement, both on the part of patient/citizens and experts (See Annex 8.5.2).

(ii) Models and frameworks. The general consensus is that a one-size-fits-all model for patient and citizen involvement seems unrealistic and unwanted. Based on the research in this background paper, we suggest that further research on different models for patient and citizen involvement should work from a combination of five variables. These are related to the following questions: (1) what is the goal of involvement; (2) who should be involved; (3) what is the role or expertise of the patient or representative involved; (4) what level of involvement is pursued and (5) what structure for involvement is suitable. The five variables all have different subcategories. For example, the goal of patient involvement may be to enhance transparency in the process of priority setting. Another goal of involvement is to give patients and citizens a say in the decision making process. Finally, it may be argued that control of end users over the priority setting process is a goal of patient and citizen involvement. The variable ‘level of involvement’ refers to the levels of citizen participation. For the purpose of patient and citizen involvement in priority setting for pharmaceutical innovation, it may be preferable to use the categories consultation, observation of the decision making process, partnership and lay control. As regards the patients or citizens that are involved and their role we distinguish four possibilities:

* The experienced individual (a patient or a carer): this person can inform the decision makers with a (non-representative) account of experiential knowledge acquired as a patient or a carer.

* A patient representative: is a person (not necessarily a patient) who gives input and feedback to decision makers about priorities and needs of groups of patients. This information should be representative for all relevant groups of patients. A patient representative also has a role in informing the public about priority setting.
Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement

* A lay person: this person is not a member of the scientific community. He or she can give a non-expert input and feedback on the process and/or the outcome of priority setting
* A public representative: a person who has been chosen to provide representative input and feedback from the public’s perspective on research priorities. They can also help increase public understanding of priority setting processes.

In Figure 8.5.2 we present a schematic overview of these variables

**Figure 8.5.2: Initial framework for patient and citizen involvement (for further development)**

<table>
<thead>
<tr>
<th>Who</th>
<th>Experienced patient or carer</th>
<th>Patient representative</th>
<th>Lay person</th>
<th>Public representative</th>
<th>Level of involvement</th>
</tr>
</thead>
<tbody>
<tr>
<td>Role</td>
<td>Observation of decision making</td>
<td>Observation of decision making</td>
<td>Observation of decision making</td>
<td>Observation of decision making</td>
<td>Transparency</td>
</tr>
<tr>
<td>Consultation</td>
<td>Partnership</td>
<td>Consultation</td>
<td>Partnership</td>
<td>Collaboration</td>
<td></td>
</tr>
<tr>
<td>Lay control</td>
<td>Lay control</td>
<td>User control</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

We stress the point that Figure 8.5.2 is no more than a proposal for a starting point for further development: in each cell of the figure, research needs to be done to elaborate and substantiate the different categories. This should result in a more in-depth understanding of the roles: what expertise is a patient or citizen asked to bring to the decision making process? It may also lead to an alternative characterization of levels of involvement. We are currently not aware of examples of lay control in health research and policy making. This option may be removed or retained for reasons that are yet unrevealed.

The figure illustrates on the one hand, that forms of involvement that some may call ‘tokenistic’ are considered adequate to achieve a certain goal. For example, for the sake of achieving transparency in the process of priority setting, involving patients and citizens as observants of the decision making may be just as effective as and more cost-efficient than setting up a collaborative partnership. On the other hand, it becomes clear that:

(iii) **Priority setting within patient and citizen organizations.** Organizations of patients and citizens which may be involved in priority setting, have to clarify their approach towards priority setting for pharmaceutical innovation. Literature on this topic is lacking and patient organizations in our survey report no formal internal process for setting priorities. This lack of procedure and preparation is a threat to further development of patient and citizen involvement.
9.3 Evaluate and learn

There is a knowledge gap with regard to the effects of patient and citizen involvement. Few researchers have tried to assess benefits, costs and adverse effects of different types of involvement activities. The evaluation of benefits in relation to costs is virtually absent. A strategy to enhance patient and citizen involvement in priority setting for pharmaceutical innovation would be to assure structural outcome assessment of initiatives to involve patients and citizens. This will not only strengthen the evidence for patient and citizen involvement, it is also needed to justify policy making and the expenditures required to facilitate this involvement. Critical scrutiny of initiatives would not only involve description and effect measurement, but also a cost-benefit assessment.

At present the evidence base does not provide impact data in enough qualitative detail to be the only source in the development of an instrument to measure impact and there is a need for further (qualitative) exploration. Abelson et al. propose four elements that need to be addressed to facilitate evaluation of deliberative processes:

(i) **Representation:** To what extent are different types of representation achieved (e.g. geographic, demographic or political)?

(ii) **Structure of the process or procedures:** Are the procedural aspects of a consultation process legitimate, reasonable, responsive and fair?

(iii) **Information:** What and how is information selected, presented and interpreted?

(iv) **Outcomes and decisions arising from the process:** The final set of evaluation principles considers the various sets of potential outcomes of the public participation process. In addition, participants must be satisfied with the process which must lead to a more informed citizenry with a better understanding of the issue. Another important outcome is the extent to which consensus was achieved and finally, it must be asked if better decisions were taken and the participation process improved policy making (i.e., did the process make a difference to the final decision).

These could be a starting point for the development of impact measurement instrument for patient and citizen involvement in priority setting. In addition, economic analysis of patient and citizen involvement is not yet reported in the literature, which suggests that future collaborations with health economists could advance our understanding of how to develop economic appraisal of the impact of patient and citizen involvement.

9.4 Empowerment and capacity building

A model is essential but in the absence of people who are willing and able to realize its potential, it will remain a paper tiger: weak and indecisive. Hence, capacity building is needed to realize meaningful involvement of patients and citizens in priority setting for pharmaceutical innovation. In this background paper, several aspects are identified for which capacity building efforts are needed. Future strategies for involvement should be based on strong values and frameworks to ensure accountability, independence and transparency. In addition to the framework development discussed above, other efforts are needed to build a good practice, mainly in education and training. All stakeholders need to be prepared for decision making on priorities that involve patients and citizens. This requires the empowerment of patients and citizens and education and training for all the parties involved. Initiatives such as the IMI European Patients’ Academy on Therapeutic Innovation (EUPATI) will play an important role in this.
(i) **Empowerment and education.** All stakeholders need to be prepared to for decision making on priorities that involves patients and citizens. In the literature, examples are given of how meaningful involvement of patients and citizens is complicated by the inequality of power in the decision making process between the expert community of scientists on the one hand and the patient and citizen representatives on the other. Feedback from people who have become actively involved in research, is that researchers should not underestimate the confidence it takes to get involved. Without emotional and psychological strength involvement is much more unlikely. A case study described how exclusion mechanisms (such as leaving certain people out or allowing less time for particular people to speak) and inclusion strategies (e.g. the lack of titles on name badges and the use of clear and informal language) can influence the process and outcomes of a dialogue meeting between researchers and service users. To resolve these issues, empowerment of patients and citizens is required as well as education of the other parties involved.

(ii) **Equal information access.** In the literature, a power imbalance is observed between experts/scientists who possess what seems to be the desired information, who control its dissemination and the forum within which it is debated on the one hand, and patients and citizens who do not have this control over information on the other. The asymmetry of information access was also identified as an important barrier at the first stakeholder meeting to support this background paper (see Annex 8.5.2). Priority setting for pharmaceutical innovation is currently not a transparent process. Patients and citizens often do not have access to information about decision making from pharmaceutical companies, regulators and governmental agencies. Asymmetry of information access creates asymmetry in power relations and hampers empowerment.

(iii) **Ignored knowledge.** Professionals generally control the interpretation of involvement and the ways that patients and citizens are involved. Patient organizations at our meetings reported the perceived lack of recognition of patients’ expertise as a major concern. The literature reports that members of drug reimbursement committees often believed that a patient’s ability to access information on the internet, and the presence of members of the public on the committee allowed sufficient access to patients. Some even speak of “intractable difficulties” including many scientists’ lack of conviction that service user involvement has the potential to contribute scientifically to such research; the dominance of positivist scientific paradigms that preclude engagement with experiential knowledge and anxiety that service users lack the requisite objectivity and familiarity with high-level abstraction adequately to participate. Including experiential knowledge of patients into the process of ‘doing science’ requires that the set of considerations allowed in decision making is broadened. Scientists and technical experts have a common knowledge base and a common language and patient experience and citizen preference are not sufficiently incorporated in the scientific discourse, it is ‘ignored knowledge’. This may have implications for the ability of patient and citizen involvement to bring about fundamental change.

There are several initiatives aimed at educating participants in decision making in health policy and research, for example NICE, IAPO, and INVOLVE. The European Union has played an active role by funding the PatientPartner Project, three-year EU Seventh Framework Programme (FP7) project investigating, enforcing and advising on the role of patient organizations in clinical trials. This project demonstrated the need for and willingness of patients to contribute to medical research. Subsequently, EU participated in
the Innovative Medicine Initiative project of the European Patients’ Academy on Therapeutic Innovation (EUPATI), which, started in 2012. Through these and other projects, knowledge on processes and mechanisms that may inhibit meaningful contribution of patients and citizens also has increased. Additional research and support is needed to identify barriers to meaningful involvement and to design measures to overcome them. It may also be important for patients to gather in patients’ organizations as this critical mass may better represent the needs and priorities of patients.

9.5 Dealing with conflict of interest

With the rise of patient and citizen involvement, the attention on conflicts of interest has also grown. Many patient and consumer groups accept pharmaceutical industry funding to support their activities. Some of them see this as a necessity to achieve their aims and they argue that patient groups are able to defend their independence from the influence of any sponsor. Other patient organizations refuse drug industry funding to maintain their autonomy. Another argument against financial relationships between commercial and civil society groups is voiced by Health Action International (HAI): “It is imperative to maintain the distinct view of each stakeholder in order to make balanced decisions about pharmaceutical regulation and health policy.” Accepting funding from the pharmaceutical industry clearly puts patient organizations in a condition of potential conflict of interest. Undue pressure can also come from other sponsors, such as public agencies or research institutes. The same holds for citizens’ organizations, who are also relevant parties from the perspective of private and public interests. Problems may arise when publication of conflict of interest information leads to diminished functioning of patient organizations and additional problems of validity and representativeness. Adequate strategies for dealing with conflicts of interest have to be designed. Potential adverse effects of conflicts of interest are a problem for many stakeholders in the pharmaceutical sector. Transparency is often advised as a way to resolve problems of conflicting interests. Regardless of the necessity of transparency, additional strategies are needed to ensure the primacy of the interests of patients and society. These ‘firewalls’ are still underdeveloped.

The EMA, for example, formulated criteria to be met by patients’ and consumers’ organizations involved in EMA activities. Problems may arise when information about funding sources is not disclosed, or if the relationship between the funding sources and activities of patient organizations is not appropriately addressed. This may lead to diminished trust in patient organizations and additional problems of validity and representativeness. Therefore, relationships with sponsors and common policies to maintain independence should be discussed transparently in order to avoid these problems.

Patient and citizen involvement can strengthen the quality and legitimacy of the decision-making process. Its potential is currently widely acknowledged and much experience is gained in the past decade. Thus, patient and citizen involvement is here to stay. However, to fully capture the value offered by such involvement, there is a need to invest in research in this area to identify appropriate groups, design frameworks for analysis, build sufficient capacity and address conflicts of interest. The European Commission (DG Research and Innovation) have a long history of establishing and supporting “Networks of Excellence”. Such an approach may be useful to support such needed research. The EUPATI project, supported by the EU through IMI, may be another model that could be adapted to focus on evaluation and learning from experience.
References


3 Council of Europe. The development of structures for citizen and patient participation in the decision making process affecting health care. In: Recommendation adopted by the Committee of Ministers of the Council of Europe; February 24, 2000; Strasbourg, France.


13 INVOLVE 2012. Diversity and inclusion: What’s it about and why is it important for public involvement in research?

14 European Medicines Agency. Fifth report on the interaction with patients’ and consumers’ organizations. 2011


INOLVE. Briefing notes for researchers: involving the public in NHS, public health and social care research. INVOLVE, Eastleigh 2012.


Boote J, Telford R, Cooper C. Consumer involvement in health research: a review and research agenda. Health Policy, 2002, 61(2), 213-236


Staley K. There is no paradox with PPI in research. J Med Ethics Published Online First: 2 January 2013 doi:10.1136/medethics-2012-100512


Keizer B. Exchanging knowledge on participation of health consumers and patients in research, quality and policy, Den Haag:ZonMw 2012


Wilmot S. Evidence, ethics and inclusion: a broader base for NICE. Med Health Care Philos 2011;14:111-121
8.5-33

Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement


54 European Medicines Agency. Fifth report on the interaction with patients’ and consumers’ organizations. 2011


56 Food and Drug Administration. Guidance for the public, FDA Advisory Committee Members, and FDA Staff: The Open Public Hearing at FDA Advisory Committee Meetings. FDA 2010.


59 INVOLVE. Briefing notes for researchers: involving the public in NHS, public health and social care research. INVOLVE, Eastleigh 2012

60 Steyaert S, Lisoir H (eds.). Participatory methods toolkit: A practitioner’s manual: King Baudouin Foundation/viWTA 2005


63 Stewart RJ, Caird J, Oliver K, Oliver S. Patients’ and clinicians’ research priorities. Health Expectations, 2011;14(4):439-48


65 http://www.jlaguidebook.org/


69 http://capsmg.cochrane.org/welcome


71 European Medicines Agency. The role of patients as members of the EMA Human Scientific Committees. EMA 2011


Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement

74 Staley K. There is no paradox with PPI in research. J Med Ethics Published Online First: 2 January 2013 doi:10.1136/medethics-2013-100512


78 Callard F, Rose D, Wykes T. Close to the bench as well as at the bedside: involving service users in all phases of translational research. Health Expectations, 2011


83 Stewart RJ, Caird J, Oliver K, Oliver S. Patients’ and clinicians’ research priorities. Health Expectations, 2011;14(4):439-48


Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement


INVOLVE 2012. Diversity and inclusion: What’s it about and why is it important for public involvement in research?


Callard F, Rose D, Wykes T. Close to the bench as well as at the bedside: involving service users in all phases of translational research. Health Expectations, 2011;15:389-400


INVOLVE 2012. Diversity and inclusion: What’s it about and why is it important for public involvement in research?


Keizer B. Exchanging knowledge on participation of health consumers and patients in research, quality and policy, Den Haag:ZonMw 2012.


European Medicines Agency. Criteria to be fulfilled by patients’ and consumers’ organizations involved in European Medicines Agency (EMA) activities. EMA/MB/24913/2005; revised 2011.
Annexes

Annex 8.5.1: Bibliography patient and citizen involvement in health research and policy


Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement


Bennani YL. Drug discovery in the next decade: innovation needed ASAP. Drug Discov Today 2012 17; Suppl:S31-44.


Bruni RA, Laupacis A, Levinson W, Martin DK. Public involvement in the priority setting activities of a wait time management initiative: a qualitative case study. BMC Health Serv Res. 2007 Nov 16;7:186.
Buckley BS, Grant AM, Tincello DG, Wagg AS, Firkins L. Prioritizing research: patients, carers, and clinicians working together to identify and prioritize important clinical uncertainties in urinary incontinence. Neurourology and Urodynamics, 2010; 29: 708–714.


Callard F, Rose D, Wykes T. Close to the bench as well as at the bedside: involving service users in all phases of translational research. Health Expectations, 2011;15:389-400


Council of Europe. The development of structures for citizen and patient participation in the decision-making process affecting health care. In: Recommendation adopted by the Committee of Ministers of the Council of Europe; February 24, 2000; Strasbourg, France.


European Medicines Agency. Reflection Paper on the Further Involvement of Patients and Consumers in the Agency’s Activities 2009.

European Medicines Agency. Criteria to be fulfilled by patients` and consumers’ organizations involved in European Medicines Agency (EMA) activities. EMA/MB/24913/2005; revised 2011


European Medicines Agency. The role of patients as members of EMA Human Scientific Committees. 2011


Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement


Food and Drug Administration. Guidance for the public, FDA Advisory Committee Members, and FDA Staff: The Open Public Hearing at FDA Advisory Committee Meetings. FDA 2010.


Fransen, C. More than research subjects? The perspective of pharmaceutical companies on the active involvement of patient organizations in clinical trials in Europe. Internship report. Amsterdam, Athena Institute, VU University 2009.


Gauvin F-P, Abelson J, Giacomini M, Eyles J, Lavis JN. “It all depends”: Conceptualizing public involvement in the context of health technology assessment agencies. Social Science and Medicine, 2010, 70, 1518-1526


Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement


Kapiriri L, Norheim OF, Martin DK. Fairness and accountability for reasonableness. Do the views of priority setting decision makers differ across health systems and levels of decision making? Social Science & Medicine 2009;68(4):766–773

Keizer B. Exchanging knowledge on participation of health consumers and patients in research, quality and policy, Den Haag:ZonMw 2012.


Kernick D, Mitchell A Working with lay people in health service research: A model of co-evolution based on complexity theory. Journal of Interprofessional Care, 2010, Available online at: DOI: 10.1080/13561820903012073

Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement


Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement


Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement


O’Donnell M, Entwistle V. Consumer involvement in decisions about what health-related research is funded. Health Policy, 2004, 70/3(281-290).


Quennell P. Getting their say, or getting their way? Has participation strengthened the patient "voice" in the National Institute for Clinical Excellence? Journal of Management in Medicine, 2001, 15/3(202-19).

Rabeharisoa V. The struggle against neuromuscular diseases in France and the emergence of the “partnership model” of patient organization. Social Science & Medicine 2003; 57(11): 2127-2136.


Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement


Rose, D., Fleischman, P., & Wykes, T. 2008. What are mental health service users’ priorities for research in the UK? Journal of Mental Health, 17, (5) 520-530


Staley K, Hanley B, for the James Lind Alliance. Scoping research priority setting (and the presence of PPI in priority setting) with UK clinical research organizations and funders. December, 2008. 
http://www.lindalliance.org/pdfs/ILA%20Internal%20Reports/TwoCan%20ILA%20report%20March%202009_with%20appendices.pdf


Staley K, Kabir T and Szmukler G. Service users as collaborators in mental health research: less stick, more carrot Psychological Medicine 2012, null:1-5


Staley K. There is no paradox with PPI in research. J Med Ethics Published Online First: 2 January 2013 doi:10.1136/medethics-2012-100512


Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement

Szmukler G. Service users in research and a ‘well ordered science’. Journal of Mental Health, 2009, 18(2), 87-90


Teram E, Schachter CL, Stalker CA. The case for integrating grounded theory and participatory action research: empowering clients to inform professional practice. Qualitative Health Research, 2005, 15/8(1129-1140)


200

210
Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement


Whitstock, M. T. Seeking evidence from medical research consumers as part of the medical research process could improve the uptake of research evidence. Journal of Evaluation in Clinical Practice 2003; 9(2): 213-224.


Wilmot S. Evidence, ethics and inclusion: a broader base for NICE. Med Health Care Philos 2011;14:111-121


Annex 8.3.2: Meeting report

IN Volvement of Patients and Citizens in Priority Setting for Pharmaceutical Innovation

Brussels 27 September 2012

Traditionally, the role of patients and citizens in pharmaceutical innovation is restricted to being research subjects and beneficiaries of research results. At the time of the development of the WHO Priority Medicines for Europe and the World 2004, patient participation in priority setting was uncommon and knowledge on the effects of such participation was limited. Over the past decade, the claim has been voiced by many that pharmaceutical innovation is misguided if patients and the public are not involved enough. Awareness of the need for a fully-fledged position for patients and citizens in the pharmaceutical innovation process is growing.

Currently, work is underway to update the 2004 WHO Priority Medicines Report. The Belgian Government offered to contribute to this update project by funding the development of a background paper on the role of patients and citizens in priority setting for pharmaceutical innovation. Belgium’s National Institute for Health and Disability Insurance (RIZIV/INAMI) agreed to organize two meetings to facilitate the production of a background paper. A group of experts on the topic joined a first meeting on September 27th 2012 (for a list of participants see Appendix) in Brussels. The goal of this meeting was to develop understanding of the role of patients and citizens in priority setting for pharmaceutical innovation.

A second larger meeting is planned for 22 February 2013, at which the draft background paper will be discussed in detail. In this report we summarize the ideas and conclusions of the first meeting.

The role of patients and citizens in priority setting for pharmaceutical innovation

WHO Priority Medicines for Europe and the World is concerned with priority setting for pharmaceutical innovation. Considering the limited literature and diverse experience with patient involvement in priority setting for pharmaceutical innovation, participants in this first meeting were invited to share as many experiences and viewpoints as possible, even if they came from patient involvement in for example regulatory bodies or reimbursement decision making.

It is important to note that patient involvement and citizen involvement in priority setting for pharmaceutical innovation are not the same. The societal perspective comprises the perspective of citizens as taxpayers, health care users and potential patients. The individual perspective is the perspective of the patient as expert by experience. These perspectives are complementary, but different. The contributions to this first meeting were generally focused on the role of patients. In this report, we discuss patient and citizen involvement together. However, the citizen’s perspective may have been under-represented. This raised the question of how to manage perspectives and biases from all groups involved in priority setting. Also, not all relevant patient groups have adequate representatives (for example children, prisoners).
Participants identified critical issues and suggestions on how to move forward. The issues presented and discussed are in this report summarized under four headings: (1) alignment of pharmaceutical innovation with medical need; (2) barriers to patient and citizen involvement; (3) moving forward and how to overcome barriers, and (4) what was agreed? This report aims to give a comprehensive and structured overview of the issues discussed and therefore, contributions from participants were grouped and merged.

1 **Alignment of pharmaceutical innovation with medical need**

Currently, priority setting mainly driven by scientists, research funding organizations and pharmaceutical companies. Priorities are thus set by the suppliers of pharmaceutical products. Patients introduce and emphasize issues that researchers, companies or regulators may not identify as important. In addition, implementation of innovations can be hampered if patients are not involved in the process of development. By working with patients, patient organizations and the public, innovation can be focused at pharmacotherapeutic interventions that reflect the views of patients and citizens, and meets their healthcare needs.

2 **Barriers to patient and citizen involvement**

The participants of the meeting shared their views and experiences on the existing barriers that hamper patient and citizen involvement in pharmaceutical innovation.

A **‘Ignored knowledge’**

The perceived lack of recognition of patients’ expertise is a major concern. Patient organizations report that participation sometimes amounts to no more than ‘tokenism’. Tokenism is involvement in the form of information or consultation that aims to legitimate decisions and gain support from patients without giving them any actual influence on the decision making.

The goal to include experiential knowledge of patients in the process of ‘doing science’ and in decision making, is challenging for both patients and scientific experts. It requires a broadening of the set of considerations that are allowed in the decision making. Scientists and technical experts have a common knowledge base and a common language and patient experience and citizen preference are not sufficiently incorporated in the scientific discourse, it is ‘ignored knowledge’. Bringing this **ignored knowledge** to the process of priority setting is complicated by unequal power relations. Patients and citizens are challenged to legitimize their input. They struggle to meet this challenge and to find ways to demonstrate credibility. Experts who recognize the value of patient involvement, may experience problems when they have to translate experiential knowledge to evidence based language.

B **No agreement on goals and process**

Patient and citizen involvement can serve several goals. For example, improving patient (health related) outcomes, increasing efficiency, achieving a more equal status of participants and gaining control over the process of priority setting are all mentioned in the literature. Among and within patient organizations and civil societal groups there is no clear consensus on the goals of their involvement in priority setting. With regard to process, there are also many unanswered questions. There is no oversight, guidance or best practice for patient and citizen involvement in pharmaceutical innovation processes. There is a need for case descriptions and examples of successes and failures. In the light of these omissions it is also very difficult to evaluate success or failure of involvement.
Problems with legitimacy and representativeness
Organizing patient and citizen involvement is highly complex. Patients can sit in on meetings with researchers and regulators, but their presence has to be backed-up by a justified view on legitimacy and representativeness. This raises questions such as:
- Who is represented by the patient involved in priority setting?
- What is the legitimacy of involving (non-elected) citizen panels?
- How can we define the knowledge from the patient and the societal perspective that is relevant for priority setting?
These questions are pressing, since a possible lack of legitimacy and representativeness is an important barrier for empowerment of patients and citizens who are involved in priority setting.

Absence of legal framework, knowledge and resources
There is a lack of knowledge and understanding of patient involvement both on the part of patients/citizens and experts. Capacity building is needed to learn to work together and to account for different perspectives in priority setting for pharmaceutical information. The general absence of a legal framework to organize and fund patient organizations is a barrier for organization of patient and citizen involvement and capacity building. In addition, patient organizations often have limited resources. This is a reason for patients not to get involved. And if they do, lack of funding may be a barrier to facilitate on-going improvement by evaluation and learning.

Asymmetry of information access
The informational barrier is twofold. First, priority setting for pharmaceutical innovation is not a transparent process. Patients and citizens often do not have access to information about decision making from pharmaceutical companies, regulators and governmental agencies. Asymmetry of information access creates asymmetry in power relations and hampers empowerment. Second, there is no oversight on patient and citizen involvement. Patient and societal organizations are usually unaware of the activities and experiences of colleagues.

Strict Conflicts of Interest (COI) policies
Strict policies on COI are the result of heightened attention for the adverse effects of conflicts of interest in the scientific community. Some patient organizations have experienced problems with these policies for their involvement in decision making (e.g. due to the funding of their organization). For example, new COI regulations at the EMA have made some patient representatives no longer eligible.

Moving forward: how to overcome barriers?
Participants identified barriers, but also shared ideas and examples of how patients organizations and civil society organizations can overcome these barriers and get involved in decision making on pharmaceutical innovation.

Promote involvement
The participants of the meeting see the need to promote a much greater role for patient representatives in all aspects of the drug development process including priority setting for pharmaceutical innovation. To prepare all stakeholders for decision making that involves patients and the public, substantial efforts are needed. Partnerships should be based on
strong values and frameworks to ensure accountability, independence and transparency. In addition, the possibility of active measures should be considered. Some suggestions are:

- Patient involvement as a condition for funding (e.g. in EU projects);
- Horizon 2020 and IMI should provide grants to support patient involvement in priority setting and all aspects of drug development, and investigate the most impactful and appropriate models for patient involvement in these processes;
- Promote governments to set conditions to health R&D funding to ensure that this funding meets societal needs;
- Advocate conditions to public funding that ensure affordability and availability of the end product from the start of the innovation process.

B  Describe models for involvement

Identifying the different roles of patients/citizens and the different contexts (or levels) on which involvement can be realized is seen as key element to improve involvement. The participants agree that it is unlikely that a one-size-fits-all model for patient and citizen involvement will be successful. Different models for different group-task combinations are needed.

Specification is needed of at least the following aspects of patient and citizen involvement to achieve a solid framework for different models:

- Expectations of all stakeholders;
- Goals of patient and citizen involvement;
- Role of patients and citizens in participation (experts working together with lay people);
- Added value of patient and citizen involvement;
- Degrees of involvement (depending on goal and process of setting priorities);
- Selection process of representatives;
- Outcome measures to evaluate patient and citizen involvement;

C  Capacity-building

Identifying, collecting and sharing good practices on patient and citizen involvement is a good starting point to facilitate capacity building. With well-designed models for involvement – and supported by resources – patients, citizens and experts can be educated to enable meaningful involvement. Capacity building can also help empower patient and citizen representatives. The view that the debate on the added value of involvement needs evidence that can come from, for example, case studies and best practices is shared by the participants.

D  Create transparency and look into COI management

Transparent decision making and information is important to make patient and citizen involvement possible. Regulators, companies, research funding organizations and governments should provide the necessary information for patients and citizens to facilitate involvement.

Conflicts of interest surrounding the development of pharmaceuticals is an issue that attracts a lot of societal attention. Mechanism to deal with conflict of interests are put in place. Unequal treatment of patient/citizen experts compared to scientific experts regarding conflict of interest should be avoided. Principles of transparency, adherence to codes of ethics and integrity, good governance and sound financial systems can guide the management of conflicting interests.
E Advocate alternative innovation models and make use of the internet

The participants felt that opportunities for better patient and citizen involvement in priority setting could be found by looking at new ways of innovation and how to get patients and citizens involved there. One way to create space for priority-setting based on health needs to be determined by multi-stakeholder engagement, is by advocating for alternative innovation models that stimulate needs-driven innovation. In addition, in developing models for involvement, the internet should especially be investigated for its opportunities. There are several internet-communities of patients that are potentially useful for this task. Examples are www.patientslikeme.com and www.patientvoices.org.uk

4 What was agreed?

● The common objective of patient and citizen involvement is to achieve better alignment of pharmaceutical innovation with medical need.

● Patient and citizen involvement is necessary for reasons of legitimacy and efficiency.

● Legitimization of patient and citizen involvement needs to be explicated.

● Goals & motivations of patients and citizens to be involved should be researched.

● Models for involvement for different contexts need to be developed.

● There is a need for clarification of roles, settings and purpose of involvement.

● Transparency is necessary to enable meaningful involvement.

● An annotated bibliography will be made to gain insight in the work on patient involvement in the literature.

● A survey will be held among patient and reimbursement organizations with the aim to document case examples & best practices.

● We need to systematically describe several characteristics of patient and citizen involvement (who/which input/which role etc.).

● We need to look at methods to measure effects of patient involvement.

● There is a need for capacity building and education of all stakeholders.

● COI policies should be reviewed to identify unnecessary barriers for patient involvement.

● A second meeting will be held on February 22nd 2013. On the agenda will be the results of the survey, a draft of the annotated bibliography and a draft background paper on patient and citizen involvement.
Annex 6.8.3: Survey results

RESPONDENTS

A survey was held under members of the International Alliance of Patients’ Organizations (IAPO) and the European Patients Forum (EPF)

IAPO is a global alliance representing patients of all nationalities across all disease areas.
EPF represents 55 chronic disease specific patient organizations operating at EU level and national coalitions of patients organizations.

In addition a slightly adapted survey was sent out to the Pharmaceutical Pricing and Reimbursement Information Network.

PPRI is a networking and information-sharing initiative on burning issues of pharmaceutical policies from a public health perspective. It involves PPRI members of almost 70 institutions (mainly competent authorities and third party payers) from the whole European Union, plus Albania, Canada, Iceland, Norway, South Africa, Switzerland and Turkey.

Seventeen representatives of Patient Organizations, through the network of IAPO and EPF completed the survey;
Nine representatives of members the PPRI Network completed the survey.

Below we present the results of the survey. In view of the number of respondents it is questionable if the information is representative. The results may not fully reflect national differences in involvement of patient’s perspective. Nonetheless, the reactions we received to the presentation of these results at the second stakeholder meeting (see Annex 8.5.2) seemed to reflect endorsement rather than criticism.

RESULTS PATIENT ORGANIZATIONS

Involvement of patient organizations in Priority Setting
Patient organizations were asked : Are you/your organization/your members currently involved (as representatives, experts or lay people) in discussions around priority setting for biomedical innovation (pharmaceuticals, medical devices)?
Role or capacity
Patient organizations were asked: In what capacity do/did they act?

Table 2. Roles of patient representatives involved in health care policy and research

<table>
<thead>
<tr>
<th>Capacity (N = 17)</th>
<th>N (%)*</th>
</tr>
</thead>
<tbody>
<tr>
<td>Representative of patient organization</td>
<td>6 (35%)</td>
</tr>
<tr>
<td>Expert</td>
<td>0</td>
</tr>
<tr>
<td>Lay person</td>
<td>1 (6%)</td>
</tr>
<tr>
<td>Unknown</td>
<td>2 (12%)</td>
</tr>
<tr>
<td>Not applicable</td>
<td>9 (53%)</td>
</tr>
</tbody>
</table>

*Total >100% because more than one answer was allowed

Experiences of patient organizations with involvement

Table 3. Experiences of patient organizations with involvement

<table>
<thead>
<tr>
<th>Experience (N = 17)</th>
<th>N (%)*</th>
</tr>
</thead>
<tbody>
<tr>
<td>Priority setting</td>
<td>1 (6%)</td>
</tr>
<tr>
<td>Technology appraisal at NICE</td>
<td>2 (12%)</td>
</tr>
<tr>
<td>Reimbursement</td>
<td>3 (18%)</td>
</tr>
<tr>
<td>PCWP at EMA</td>
<td>1(6%)</td>
</tr>
<tr>
<td>Other</td>
<td>6 (35%)</td>
</tr>
<tr>
<td>None</td>
<td>9 (59%)</td>
</tr>
</tbody>
</table>

*Total >100% because more than one answer was allowed

Internal process of priority setting
Does your organization have a process for setting priorities in research or have activities involving patients within your own organization? If so, who is responsible for priority setting?

Table 4. Process for priority setting

<table>
<thead>
<tr>
<th>Priority setting by (N = 17)</th>
<th>N (%)*</th>
</tr>
</thead>
<tbody>
<tr>
<td>Individual member of organization</td>
<td>1 (6%)</td>
</tr>
<tr>
<td>Organization as a group</td>
<td>4 (23%)</td>
</tr>
<tr>
<td>No formal process for priority setting</td>
<td>4 (23%)</td>
</tr>
<tr>
<td>Unknown</td>
<td>8 (47%)</td>
</tr>
</tbody>
</table>
Neglected groups
The question was asked: Are you aware of any ‘neglected patient groups’ with regard to patient representation and patient involvement? Could you provide us with examples?
YES 2 (12%) vestibular diseases, all long term conditions
NO 15 (88%)
UNKNOWN 1 (6%)

Capacity building
Are you aware of any efforts for capacity building for patients (e.g., courses for patient representatives)?
YES 3 (18%) via IAPO, EPF, EUPATI, EMA, etc.
NO 14 (82%)

Are you aware of efforts to educate professionals on how to incorporate patient experience/expertise in decision making or priority setting on pharmaceutical research?
YES 3 (18%) via Eduvital, EPF Value+ kit
NO 14 (82%)

Success factors and barriers
The patient organizations were asked: What are the factors that contribute to successful patient involvement in decision making or priority setting on pharmaceutical research? (e.g., education of patients/professionals, obligatory patient involvement)

Factors mentioned:
1 Patient support groups
2 Education of patients/professionals.
3 Obligatory patient involvement
4 Funding
5 Participating in congresses and raise our voice
6 Partnership and dialog with the appropriate stakeholders
7 Understand that involvement is a process, not a one-time event

Subsequently, the question was: What are the factors that hamper involvement of patients in decision making or priority setting on pharmaceutical research?

Factors mentioned:
1 Use of technical language
2 Heavy bureaucratic system
3 Lack of any ideas on what would be suitable drugs partly because the diseases are not understood
4 No close contribution between patients organizations and government institutions.
5 Lack of resources (always assuming that patients should do everything on volunteer basis).
6 Lack of education
Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement

7 Lack of knowledge of where to find expert patients
8 The decision making stakeholders usually do not think that patients are part of the system or qualified to participate
9 Experts of pharmaceutical research are unaware of the needs of patients
10 Pharmacoeconomical aspects
11 Ethical issues
12 Genetic aspects
13 The whole process and language is designed for professionals and it is perceived as too much work and too complicated to involve patients.
14 There is confusion on the difference between a (individual) patient and a patient representative, and which one would be the right one
15 The perceived bias by patient representatives because of industry funding to their organizations. Sometimes it does not matter how responsibly and in which transparent way a patient group is doing this, it is just bad in itself and patients are assumed to be more naïve than healthcare professionals.

Finally, the respondents were invited to bring to our attention anything they considered relevant to the topic.
Response came from two organization representatives:
1 Strengthen relationship among patient organizations in the world in order to increase their voice.
2 Organize necessary educational programmes from WHO to involve patients in decision making in different fields of health system.
3 Patients leaders should be members in the WHO since they are representing the patients the best.
4 WHO should assist us to reach every relevant workshop or congress to bring the patients voice.

RESULTS PPRI NETWORK MEMBERS

Involvement of PPRI representatives in Health Care Policy and Research
PPRI members were asked: In your country are patients or citizens involved in decision making for pricing or reimbursement decisions. In addition, we asked if they knew of any examples of patients or citizens involvement in pharmaceutical innovation, market authorization, guideline development and biomedical research in their country. The results are summarized below.

PPRI network survey launched end October 2012
Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement

Involvement of patients or citizens in pricing or reimbursement decisions

<table>
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<tr>
<th>Yes</th>
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<td>MT</td>
<td>AT</td>
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<tr>
<td>NO</td>
<td>BE</td>
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<td>SE</td>
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Who is involved in these discussions?
- Patient representatives
- Relevant patient organizations

What’s their role?
- Directly involved in the decision process
- Consultation

Involvement of patients or citizens in pharmaceutical innovation, market authorization, guideline development and biomedical research programs

<table>
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<th>Yes</th>
<th>No</th>
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<tbody>
<tr>
<td>AT (market authorization, innovation)</td>
<td>CZ</td>
</tr>
<tr>
<td>BE (guideline development)</td>
<td>HU</td>
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<tr>
<td>ES (market authorization)</td>
<td>MT</td>
</tr>
<tr>
<td>NO (innovation, guideline development)</td>
<td>SE</td>
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<tr>
<td>UK (market authorization, guideline development)</td>
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Awareness of neglected patient groups

<table>
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<tr>
<th>Yes</th>
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<tr>
<td>NO (elderly people)</td>
<td>AT</td>
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Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement

Capacity building

Efforts for capacity building for patients and citizens (e.g. courses for patient or citizen representatives)

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Efforts to educate professionals on how to incorporate patient and citizen experience/expertise in decision making on priority setting on pharmaceutical research, pricing or reimbursement

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<th>Yes</th>
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<td>AT</td>
<td>BE</td>
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<tr>
<td>(“economic prescription” courses)</td>
<td></td>
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<td>CZ</td>
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Success factors and barriers

What are the factors that contribute to successful patient or citizen involvement in decision making on price or reimbursement setting?

- Adequate education (e.g. knowledge/understanding decision making criteria) is important
- No “conflict of interest”
- An obligatory “conflict of interest declaration” should be introduced
- Other stakeholders’ understanding of the importance of the participation of patients/citizens
- Timely information to the patient organizations
- Obligatory patient representative

What are the factors that hamper involvement of patients in decision making or priority setting on price or reimbursement setting?

- Inadequate education
- “Conflict of interest”
- Absence of a patient representative
- Unstructured patient support groups
- Unclear role of patient support groups
Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement

- Absence of umbrella organizations
- Potential influence of marketing by pharmaceutical companies
- Low price sensitivity for medicines
- Too short deadlines in the decision making process

DISCLAIMER by PPRI:
The data contained in this summary have been provided by the members of the PPRI network and represent the current situation. The data do not have any legally binding value and are meant exclusively for the information of PPRI network members who are committed to sharing information on pharmaceutical pricing and reimbursement.
Annex 8.5.4: Viergever’s Checklist for health research priority setting

Table 1 Checklist for health research priority setting

<table>
<thead>
<tr>
<th>Preparatory work</th>
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<tbody>
<tr>
<td>1. Context</td>
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<td>Decide which contextual factors underpin the process: What resources are available for the exercise? What is the focus of the exercise (i.e. what is the exercise about and who is it for)? What are the underlying values or principles? What is the health, research and political environment in which the process will take place?</td>
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| 2. Use of a comprehensive approach |
| Decide if use of a comprehensive approach is appropriate, or if development of own methods is the preferred choice. These approaches provide structured, detailed, step-by-step guidance for health research priority setting processes from beginning to end. |

| 3. Inclusiveness |
| Decide who should be involved in setting the health research priorities and why. Is there appropriate representation of expertises and balanced gender and regional participation? Have important health sectors and other constituencies been included? |

| 4. Information gathering |
| Choose what information should be gathered to inform the exercise, such as literature reviews, collection of technical data (e.g. burden of disease or cost-effectiveness data), assessment of broader stakeholder views, reviews or impact analyses of previous priority setting exercises from other geographical levels. |

| 5. Planning for implementation |
| Establish plans for translation of the priorities to actual research (via policies and finding) as a priority at the beginning of the process. Who will implement the research priorities? And how? |

| Deciding on priorities |
| 6. Criteria |
| Select relevant criteria to focus discussion around setting priorities. |

| 7. Methods for deciding on priorities |
| Choose a method for deciding on priorities. Decide whether to use a consensus based approach or a metrics based approach (pooling individual rankings), or a combination |

| After priorities have been set |
| 8. Evaluation |
| Define when and how evaluation of the established priorities and the priority setting process will take place. Health research priority setting should not be a one-time exercise! |
9. Transparency
Write a clear report that discusses the approach used: Who sets the priorities? How exactly were priorities set?
Annex 8.5.5: Saunders’ criteria and rating scales


Table 3 Final consumer review criteria and rating scales

<table>
<thead>
<tr>
<th>Criteria</th>
<th>Range of scores</th>
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| **Extent of benefit:** Will the findings potentially have an important positive impact on human lives, including any of the following aspects: disease causation (identifying the biology of cancer and the fundamental mechanisms by which cancers arise), prevention, diagnosis, treatment, physical and/or mental and/or social wellbeing, quality of life, dignity, survival? When assessing this criterion, trained consumer reviewers may want to consider some or all of the following: | Nil (no information provided) = 0  
Minor = 1  
Moderate = 2  
Substantial = 3 |
| - Has the researcher explained how the research will generate tangible benefit(s) to human life?  
- Has the researcher indicated the probability, magnitude, and/or duration of these potential benefits?  
- Does the research provide a number of benefits? | |
| **Pathway for realizing the benefit:** Is there a clear description of the steps required to reach the stated benefits of the research? When assessing this criterion, trained consumer reviewers may want to consider some or all of the following: | Nil = 0  
Moderate = 1  
Substantial = 2 |
| - Has the researcher provided a brief description of the broad steps or stages required to reach the stated benefits of the research?  
- Do the steps or stages appear reasonable?  
- Are the steps or stages achievable?  
- Do the steps or stages represent significant constraints to achieving the actual benefits of the research? | |
| **Potential for application of findings:** Is there potential for real-world application of findings in the long-term? When assessing this criterion, trained consumer reviewers may want to consider some or all of the following: | Nil = 0  
Moderate = 1  
Substantial = 2 |
| - Is it likely that the research findings will be able to be put into practice (in either the short, medium or long term)?  
- Are there likely to be significant barriers to putting the research findings into practice?  
- How compatible are the research findings likely to be with existing laws, public policy, resources, etc.?  
- Where relevant, does the researcher include the groups they will work with to overcome barriers to applying research findings? | |
| **Equity:** Is there adequate justification for the selection of the study sample that demonstrates potential for equity, e.g. the research does not exclude groups who could potentially benefit from its outcomes, and/or it addresses an understudied group and/or a group with a high burden of | Nil = 0  
Moderate = 1  
Substantial = 2 (also includes if more) |
illness? When assessing this criterion, trained consumer reviewers may want to consider some or all of the following (the research is not required to meet all these expectations):

- Does the researcher explain how the findings could be generalized or applied to similar people outside the research?
- Does the research have the potential to provide benefit across all relevant persons, groups and/or places?
- Does the research address an understudied group?
- Does the research address a group with a considerable burden of illness?

**Consumer involvement:**

(a) Development phase: have experienced consumers (e.g. from consumer or cancer groups) been involved during the development of the research proposal?

(b) Ongoing involvement: is there a plan for ongoing consumer involvement in the research? Is consumer involvement describer? Have the researchers identified the preferred approach of consumers for involvement in the research? Are there formal processes/structures in place that link the researchers with consumers?

**Dissemination of results:**

Is there a plan for circulating lay information about all research results to participants and/or the general community? Are there plans for consumers to be involved in the dissemination of research results?

<table>
<thead>
<tr>
<th>Criteria</th>
<th>No = 0, Yes = 1</th>
</tr>
</thead>
<tbody>
<tr>
<td>Level of reasonable explanation given by the researcher in the funding proposal against each criterion</td>
<td>an option is addressed</td>
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</table>
Patients have always been involved in research. For many years they were the subjects of research but often their involvement was through informed consent procedures. Some patient groups fundraised for research and did support research related to the disease they were interested in. In the 80s and 90s, two groups of patients became more active in advocating for research in their areas of concern. These were orphan diseases and AIDS.
Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement

Through their advocacy changes such as the Orphan Drug Act in the United States was passed.

At the time the Priority Medicines Report was written in 2004, there was a limited literature on the topic of involvement of patients and citizens in pharmaceutical innovation. Since then, there has been increased attention for patient and citizen involvement and this is also reflected in a growing body of literature.

The European Commission have now requested that WHO update the 2004 Priority Medicines Report as a background study for Horizon 2020 planning and as an input to the planning for the next Innovative Medicines Initiative. The Belgium government (National Institute for Health and Disability Insurance) has offered to provide support for this process by funding an activity to be undertaken as part of this update project. NIHDI has agreed to organize two meetings. A first relatively small meeting was held on September 27th 2012 and focused on defining terms and concepts, sharing information and suggesting resources that could be used to develop a background paper on this topic. The background paper was the central topic of the second meeting. Several contributions were invited to give additional input for the background paper and to stimulate discussion on identifying the most pressing research questions. The meeting was chaired by Bert Leufkens from Utrecht University. In this report, we summarized the presentations and discussions held on 22 February 2013.

Introduction by Bert Leufkens

Professor Leufkens introduced the topic of the meeting. He referred to the experiences of the Medicines Evaluation Board (MEB) in the Netherlands. The MEB has developed closer interaction with patients’ and citizen’s organizations with the aim to:

(i) become better informed about pertinent practice needs of patients in the drug usage system (operational goal),
(ii) learn about shared and different values and perspectives when regulating medicines (tactical goal),
(iii) promote transparency, accountability and trust about benefit-risk decisions made by the MEB (strategic goal).

Professor Leufkens also reported about discussions among scientist and regulators who see the question of How to involve patients and citizens as an important issue.

Lessons from involving patients and citizens in research agenda setting

A series of articles and book chapters have been produced by Jacqueline Broerse and her colleagues at the Vrije (Free) University in Amsterdam. Professor Broerse made the opening remarks during the February 22nd meeting. During the past 25 years, Professor Broerse and her colleagues have developed and tested the Interactive Learning and Action (ILA) approach for patient and citizen participation in priority setting. In her presentation she gave an overview of cases and outlined the four basic principles of the Interactive Learning and Action Approach: (1) partnership; (2) participation; (3) knowledge integration and learning and (4) joint reflection and alignment. The process and practice of ILA was explained through the presentation of the case of policy agenda setting with the burns foundation. The lessons learned from experience with patient and citizen involvement in priority setting were:

1. Patients are able to set research priorities on a variety of topics, including biomedical research. They have attention for long-term research and provide with new research topics.
2. Each stakeholder group in ILA has its ‘own’ priorities, although there is clear overlap as well.
3. It is difficult to address power differences adequately and subtle ways of exclusion can occur.
4. Empowerment of patients through good preparation is crucial, as well as process facilitation and support.
5. Patient and citizen involvement is approached with a mix of distrust and enthusiasm, and a mixed dialogue is mostly valued.
6. In general there is no adherence to quality criteria such as diversity, knowledge integration and reflection. Also transparency, validity and reproducibility are not warranted.
7. There is a lack of competences.
8. Incentives may be established through trans-disciplinary research programs.
9. Switch from a supply-driven towards a more problem-oriented research system requires a paradigm shift, which is never easy.
10. There is a lack of sense of urgency.
11. Dominant structures and procedures are an obstacle for patient involvement.
12. A learning network is of great importance as it can create a transition arena.

Past experiences by IAPO with Priority Medicines project
Joanna Groves from the International Alliance of Patients’ Organizations (IAPO) shared experiences with the Priority Medicines Project. IAPO responded to consultations during development of Priority Medicines Report in 2004 and based their response on consultation with IAPO members. In addition, patient representatives attended a meeting on the first Priority Medicines Report in Brussels in October 2004 and the launch in The Hague in November 2004. In those days, the key area of input was around the need and value of patient involvement. For IAPO, their involvement in the Priority Medicines project in 2004 was one of instigators of development of IAPO’s Policy Statement and Guidelines on Patient Involvement (published 2005). A case example of how patients/citizens have been able to influence priority setting for pharmaceutical innovation was presented, namely the Consumers Health Forum. In this Australian project, patients were involved in development of policy and guidelines on promoting and applying research. A high impact result was the overturning of a decision by Australian Government in 2011 which would have threatened access to vital medicines. The following conclusions were presented:
* Patients’ organizations are involved in priority setting for pharmaceutical innovation but there is much scope and need to support and promote involvement.
* There are many barriers to involvement including:
  A lack of awareness, identification of patient groups, questions of representation, credibility and independence, a lack of evidence of impact, lack of knowledge (on both sides), understanding how to do it, lack of resources etc.
* All involvement should be based on strong values and frameworks to ensure accountability, independence, transparency and value.
* There is a need to promote greater research into patient involvement in all aspects of the drug development process including priority setting for pharmaceutical innovation to assess the impact of patient involvement to date and to investigate the most impactful and appropriate methods for involvement in these processes.
Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement

Where are we? Surveys
In addition the two patient’s organizations from Europe and internationally have helped undertake a short survey of their members. The PPRI network that works on pricing and reimbursement has also undertaken a survey of their members to discover what role patients and citizens play in pricing and reimbursement decisions.
- Patients & Citizens associations
- PPRI network
For the results of the surveys see Annex 8.5.3.

Presentation of the Background paper
The draft background paper was discussed in detail.

Session 2: Team discussion (three groups)
The presentations were followed by group discussions to encourage active interaction between the different groups. The meeting also had as an important goal to allow for a discussion of the background paper and to answer key questions about the role of patients and citizens in priority setting.

The participants were divided in three groups. Each group discussed the role of patients and citizens in priority setting. The discussions were guided by two questions:
* Which are the best models that could be used?
* What are the indicators (input and process) that could be used to measure or describe this involvement?
The chairs of the subgroup sessions were:
J. Groves (IAPO)
K. Immonen-Charalambous (EPF)
V. Thomas (NICE).

Session 3: Next steps
In session 3, a report was given of the three groups discussion. Rapporteurs were asked to address the following topics:
* What can be agreed?
* What research is needed to identify the most effective ways for patients and civil society to contribute to priority setting for research?
* Which recommendations could be included in the ‘Priority Medicines in Europe and the World 2013’ report?, Richard Laing (WHO)

Group 1 Report
Group 1 agreed on the following:
* Participation is a value in itself;
* No one model “fits all”;
* But some key principles could be identified that should be applied across all situations;
* Clear goals and clear roles for all actors;
* Capacity building to support patient/citizen involvement; and
* Transparency is essential – relates to capacity of patients and citizens to give informed input;

Group 1 identified the following research topics:
* A specific research agenda should be developed
Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement

* Theoretical framework
* Indicators – process and outcome
* Impact research on different levels
* Evaluation of initiatives
* Collecting and documenting models and initiatives

Group 1 Recommended:
* Regulatory framework for transparency is needed
* Develop research to identify best practice and the impact of participation, while bearing in mind that participation should not be seen instrumentally but as a value in itself
* (EPF): mandate or strongly prioritise research projects that include patients and citizens involvement in identifying the research priorities.

Group 2 Report
Group 2 agreed on the following:
* In general patient/citizen involvement has been acknowledged as important
* But it is recognised that it might be complicated and patients/citizens have interests and perspectives
* Roles and responsibilities regarding involvement are not clear enough
* Representativeness needs to be clear
* Components to underpin the involvement include: accountability, education, transparency.

Group 2 identified the following research topics:
* Develop a specific research agenda:
  * Indicators
  * Impact research, on different levels e.g. society
  * Develop theoretical framework
  * Evaluation of initiatives
  * Collecting and document models and initiatives – and at the same time develop indicators.

Group 2 Recommended:
* Not only use one model and replicate it
* Bring in different models
* Invest in research (how to do it and evaluation).

Group 3 Report
Group 3 agreed on the following:
* Involvement of patients from start is necessary
* Top-down and bottom-up approach
* Flexibility of methods
* Regulatory requirement
* Training and education in working with patients
* Patient involvement in research design (asking the right question)
* Transparency in declaring interests and resolving conflicts
* Skills and capacity building

Group 3 identified the following research topics:
* Build research networks on the role of patients
* Risk-benefits evaluations from the patients’ point of view
Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement

* Value of paying participants
* Evaluation of measures of the added value of participation itself + of different models for participation.

Group 3 Recommended:
* Expand EUPATI
* Acknowledge the issues of a multilingual constituency.

In a closing session the results of the group meetings were presented.

Participants

<table>
<thead>
<tr>
<th>Name</th>
<th>Organization</th>
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Update on 2004 Background Paper, BP 8.5 Patient and Citizen Involvement

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March 2013