8.5 Patient and citizen involvement

See Background Paper 8.5 (BP8_5Stakeholder.pdf)

At the time of the 2004 Report, patient and citizen participation in priority setting was uncommon and knowledge about and experience of the impact of such participation was limited. Today, the 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. A variety of underlying motivations drive the efforts to involve patients and citizens in priority setting for pharmaceutical innovation.

First there are political reasons, based on the desire to promote democratic ideals of legitimacy, transparency and accountability. In the year 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.

Second, the promotion of patient and citizen involvement can be driven by arguments of transparency and trust. For example, an analysis of the benefits of patient involvement by the EMA led the agency to conclude that: “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.”

A third category is health-related motivation that stems from the need to better align pharmaceutical innovation with the real, unmet needs of patients. Pharmaceutical innovations do not always meet the needs of patients effectively. 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 an emphasis on chronic but not acute conditions, severe but not common health problems, and disease-specific but not cross-cutting issues, such as social care, improved surgery, and anaesthesia. Evidence shows that health professionals’ research priorities differ from those of patients.

Another health-related motivation focuses on the actual contribution which especially 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. 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{7,8}

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.

\textit{Perspectives of patients and citizens}

One of the first questions to be asked is: how should patients and citizens be
distinguished between? While there is widespread belief that values for health states
differ between patients and the general public, there is a long-standing debate among
health economists about the evidence to support this belief. Research findings suggest
that patient and population preferences can both be used to set priorities for
pharmaceutical innovation,\textsuperscript{9} although there is evidence that patients may give higher
valuations to certain health states compared to members of the general public.\textsuperscript{10} In
general, it seems that patient and citizen involvement can be captured by the term
‘public involvement’ in many but not all cases. For a start, patients and citizens may
have competing or contrasting interests in priority setting for pharmaceutical
innovation. Second, there are circumstances that call for a more specific use of
experience. This is the case when involvement is sought with the explicit aim of
seeking to use the experiential knowledge of a patient, or a well-described group of
patients or care takers.

\textit{Models for involvement and their impact}

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. In
general, the literature on roles of patients and citizens in health policy and research
appears to reflect a joint search for models for involvement that not only yield a patient
or citizen perspective, but also allow real influence on the decisions made. However,
one aspect of patient and citizen involvement 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 or control)
and in a variety of models.

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 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
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valuable, they provide no indication of the extent of impact, its magnitude or how it compares across different areas of impact.\textsuperscript{11}

\textit{Validity and representativeness}

One of the main arguments for patient involvement concerns the contribution 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 questions: To what extent is the experienced perception of a patient representative credible? And how can this specific experiential knowledge be absorbed into the scientific process? The same questions may also occur in citizen involvement: (How) can one representative account for the perspectives of citizens with a variety of social and cultural backgrounds? And how can a lay perspective be preserved when citizens (or patients) are educated to participate in the scientific process?\textsuperscript{12} What methods can be used to enhance the credibility of the contribution of patients and citizens to the decision making?

A second problem for validity and representativeness is the potential for conflict of interest. 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 argue that patient groups are able to defend their independence from the influence of any sponsor.\textsuperscript{13} Other patient organizations refuse drug industry funding in order to maintain their autonomy.\textsuperscript{14} With the rise of patient and citizen involvement, the focus of attention on conflicts of interest has also grown. Accepting funding from the pharmaceutical industry clearly puts patient organizations in a position of potential conflict of interest. As with conflict of interest among professionals, the response of most regulatory authorities is to request transparency. The EMA, for example, formulated criteria to be met by patients' and consumers' organizations involved in EMA activities.\textsuperscript{15} 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.

Questions of validity need to be addressed since they limit acceptance of non-expert involvement.\textsuperscript{16} 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.

\textit{Recommendations for research}

Priority research on patient and citizen involvement is recommended in the following areas:
• Framework for patient and citizen involvement: Although the wide variety of approaches for patient and citizen involvement has helped in the accumulation of experience, this has not yet resulted in a 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. To help facilitate this, further research is needed on models for patient and citizen involvement, based on a combination of five variables. These variables or key questions are: what is the goal of the involvement of patient and citizen organizations in priority setting and decision-making; who should be involved; what is the role or expertise of the patient or representative involved; what level of involvement is pursued; and what is the most suitable structure for involvement.

• Research on capacity building: In the absence of people who are willing and able to realize the full potential of this kind of stakeholder involvement, it will remain a paper tiger, weak and indecisive. What is needed now is research on methods for capacity building to help ensure the meaningful involvement of patients and citizens in priority setting for pharmaceutical innovation. In addition to the framework development discussed above, other research efforts are needed to identify and establish best practices, 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 supported European Patients' Academy on Therapeutic Innovation (EUPATI) will play an important role in this capacity building.

• Outcome assessment: A third research recommendation is to assure structured 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.

Patient and citizen involvement can strengthen the quality and legitimacy of the decision-making process. Its potential is currently widely acknowledged and much experience has been gained over 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.

References

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


