

PATIENT INVOLVEMENT IN HEALTH TECHNOLOGY ASSESSMENT

*An interim report on EPF's survey with
Patient Organisations across Europe*

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1. INTRODUCTION

Health Technology Assessment (HTA) is a multidisciplinary tool that ideally should involve patients' perspectives; however, in reality there is still a long way to go in achieving meaningful patient involvement in HTA. The existing material on patient involvement in HTA, though not extensive includes literature, surveys and tools (e.g. literature in IJTAHC, INAHTA surveys and HTAi Glossary for Consumers and Patients). It has been produced to either directly support or to inform and initiate discussion on involving patients, patient organisations, citizens, informal carers and/or consumers in HTA. Despite doing some good groundwork, patient organisations are still struggling to gain a foothold in the HTA process, as was highlighted in EPF's HTA Seminar¹ held in May 2010. The need to follow up on the seminar, during which the patients' organisations clearly called for support to be meaningfully involved in HTA processes, led EPF to conduct this research to further explore and address some of the issues around patient involvement in HTA.

The research is divided into three stages. It involves collecting primary data, mainly through surveys and discussions with three main stakeholder groups: HTA agencies (first stage), HTA appraisal committees/policy makers (second stage) and patient organisations (third stage). The reports of the previous stages are available in EPF website. This report describes the main findings obtained from the third stage. As a final step, a comprehensive report will integrate the results of all phases aiming to provide an as accurate as possible state of the art of patient involvement in HTA in Europe. In this way, EPF intends to get a complete overview of the three stakeholder groups and their perspectives to inform and shape HTA patient involvement in Europe.

¹ The seminar report is available at: <http://www.eu-patient.eu/Documents/Publications/ConferenceSeminarReports/hta-seminar-2010-brussels-report.pdf>.

1.1 Research Aim

The aim of the research is twofold:

- I. To identify the current situation, good practices in place and the challenges of patient involvement in HTA in European countries.
- II. To contribute this knowledge to the process of informing and building the capacity of patient organisations, HTA agencies and HTA appraisal committees and decision-makers in Europe, by producing a toolkit (manual, guide or other format).

1.2 Methodology

The scope of patient involvement in the survey is intended to comprise three levels and types of involvement:

Involvement at organisational level:

1. Patient organisations through their representatives

Involvement at individual level:

2. Lay patients
3. Informal carers (relatives and friends).

The survey was piloted with two patient organisations and the questions were modified based on their input. The questionnaire comprised multiple-choice questions and open and closed ended questions. It also ensured flexibility in order to collect qualitative perspectives. The Survey Monkey online tool was used to conduct the survey and it was sent electronically to participants' official email addresses.

The first step to conduct the survey was to contact patient organisations from across Europe. The following channels were used: (1) with their agreement to take part, the survey was sent to all participants of Patient Involved in NICE (PIN) group that includes patient organisations that give their input to HTA (2) EPF members and (3) EPF directory of patient organisations in Europe. 42 organisations started the survey and partially answered while 23 fully completed it. The partially completed answers were excluded from analysis. A response rate could not be calculated as the survey employed a snowball sampling technique.

2. RESULTS OF THE SURVEY

Out of the 23 respondents who completed the questionnaire, 10 indicated that their organisations have been involved in HTA. Out of these 10, eight responded to the subsequent questions detailing their experiences in HTA involvement.

The findings are presented in two sub-sections:

- **Background on patient involvement in HTA:** it describes the current status of involvement of the patient organisations;
- **Facilitators and Challenges to patient involvement:** in addition to enablers and barriers, this section also describes the ideal type and level of involvement wished by patient organisations and some good practices.

2.1 BACKGROUND

Respondents were asked to list a maximum of five HTA agencies that they interact with and they listed HTA agencies, national health insurance agencies, national health services, ministries of health. They were also asked to list the decision-making body/HTA appraisal committees that their organisation interacts with. The bodies they listed are national organisations of pharmaceutical and medicinal products, national health insurance, ministries of health, and national health services.

2.1.1 Span of involvement

Out of the eight organisations that responded to the specific questions regarding their involvement in HTA, five were represented by staff members who contributed an organisational perspective to HTA, two were represented by lay patient members of the organisation who contributed an organisational perspective, and one was represented by lay patient member of the organisation who contributed an individual experience.

These organisations started contributing patient perspectives to HTA at different time periods. Two of them have been involved for more than 10 years, one has been contributing for 5-10 years and five of them for 3-5 years.

2.1.2 Type and level of patient involvement

Out of these eight patient organisations, the majority were lowly or not involved in HTA stages such as identification, filtration, prioritisation, production of information, and internal review. On the other hand, the majority were highly or moderately involved in phases such as assessment, external review and diffusion/dissemination.

Type and level of involvement in HTA stages	High to moderate involvement	Low to no involvement	Don't know
Identification	2	3	3
Filtration	2	3	3
Prioritisation	3	4	1
Assessment	4	3	1
Produce information	3	5	
Internal review	3	5	
External review	5	2	1
Diffusion and dissemination	6	2	

Table 1: The type and level of involvement in different phases of the work of HTA agencies

The findings related to their involvement in decision-making on health technologies based on HTA have been summarised in the following table. There is no or low involvement in prioritising HTA research topics, topic selection or scoping. And similarly in appraisal committees. Patients are involved mainly through public consultations, in providing patient evidence and in appeals against decisions.

Involvement in HTA stages through	High to moderate involvement	Low to no involvement	Don't know
Appraisal committees	3	5	
Public consultations	5	2	1
Provide patient evidence that has been included in HTA reports used by decision-makers	5	2	1
Appeals against the final recommendations of the decision makers	5	2	1
Involvement in prioritising HTA research topics/topic selection/scoping	2	6	

Table 2: Type and level of involvement in decision-making on health technologies

2.1.3 Reasons behind patient involvement

Five respondents indicated that they got involved in HTA upon their own initiative/interest. Four of them stated that it was also due to the demand from the members of the patient organisation and another four stated that it was because of policy-makers'/decision-makers' initiative. The other reasons were HTA agencies' initiative (3), observation of experiences or good practices from other patient organisations (2), recommendation by international or European HTA bodies and decision makers/HTA appraisal committee's interest (2), and HTA appraisal committees' interest (1).

2.1.4 Competencies required to participate in HTA

According to the respondents, all five areas of competency are almost equally important for patient organisations in order to be involved in HTA. The competencies that patient representatives should have even at a minimum level are knowledge of evidence based medicine and HTA's basic concepts, understanding of HTA methodology, in depth knowledge of disease condition, practical knowledge of how and when to contribute evidence, and knowledge to interpret standard clinical research.

Involvement in HTA stages through	Highly to moderately important	Lowly or not important	Don't know
Knowledge of Evidence Based Medicine and HTA's basic concepts	7	1	
Understanding of HTA methodology	7	1	
In depth knowledge of disease condition	7	1	
Practical knowledge of how and when to contribute evidence	7		
Knowledge to interpret standard clinical research	7		1

Table 3: The competencies that a patient organisation/representative should have to be involved in HTA

2.1.5 Impact of patient involvement in the work of HTA agencies and decision-making on health technologies

When asked to rate the impact of their organisation's involvement in the work of HTA agencies, respondents rated 'better understanding of technology impact in real life context' and 'better understanding of the quality of life aspects' as the two areas they were able to have the most impact. 'Better quality of assessments and comprehensive information' and 'higher accuracy in measuring needs and preferences of patients' are other aspects where a high impact is also observed by respondents. On the other hand, respondents think that they do not have much influence on 'higher

reliability and relevance of reports to the local context' and 'increased timeliness in producing information'

Impact	High to moderate impact	Low to no impact	Don't know
Better quality of assessments and comprehensive information	4	3	1
Better understanding of technology impact in real life context (e.g. barriers to comply to current therapy, side effects, patient capacity to pay etc.)	5	3	
Better understanding of the QOL aspects	5	3	
Higher accuracy in measuring needs and preferences of patients	4	3	1
Higher reliability and relevance of reports to the local context	2	4	2
Increased timeliness in producing information	2	5	1
Lower costs for producing reports		5	3

Table 4: Impact of involvement in the work of HTA agencies

When it comes to the impact of patient organisations' involvement in HTA decision-making on health technologies, we see that the highest impact is perceived to be on 'decisions made meeting patients' needs in terms of quality of life and patient expected outcomes'. While moderate effects are seen on other aspects such as 'higher reliability and relevance of decisions', 'increased transparency and accountability in decision-making', 'addressing unmet needs of patient groups', 'patient-centred health expenditures', and 'decisions being more consensus-driven'; the least impact is seen on 'increased timeliness in making decisions'.

Impact	High to moderate impact	Low to no impact	Don't know
Decisions made meet patients' needs in terms of QOL and patient expected outcomes	5	3	
Higher reliability and relevance of decisions (e.g. which treatment and care should be available)	3	4	1
Increased transparency and accountability in decision-making	3	5	
Increased timeliness in making decisions	1	7	
Addressing unmet needs of patient groups	3	5	
Patient centred health expenditures	3	5	
Decisions will be more consensus-driven	2	5	1

Table 5: Impact of involvement in HTA decision-making on health technologies

We also asked if the perspective brought by patients/informal carers/patient organisations (needs, preferences, patient evidence) are currently being integrated into HTA reports or decision-making based on HTA reports. Three respondents indicated that their perspective was being integrated into reports, while one did not know. Four other respondents stated that patients' contribution was not being integrated into HTA reports. On the other hand, five respondents indicated that patients' perspective was integrated in decisions made while three of them stated the opposite.

Some respondents who claimed that patients' perspective is not taken into account provided detailed explanations. The problems they point out are the following: patient organisations being neglected, patients being asked very simplistic questions which are not really taken into account, lack of transparency during the production of reports.

“Speaking spontaneously I am not aware of any of the reports. Anyway my own impression is that the health technology lobby are asking patients to supply the answer to questions which really could be answered by themselves, without recourse to patients, with a minimum of reflection on what it is they are trying to do. Ironically I have the impression that the health technologists are looking for an apologia or endorsement from patients. There is also a smug and self-righteous element in this commerce and a lack of humility.”

“As far as I am aware patient organisations [...] are hardly ever included in the drafting of HTA reports. We are allowed to comment as a stakeholder for specific dossiers, but this is only in writing. We do receive final reports afterwards and sometimes a covering letter as to why our comments are incorporated yes or no. Since other stakeholders (physicians, pharmacists and industry) are also invited to comments and we have no view on each other's comments you can never say in the end what the effect of ours is on the final decision and whose arguments had the biggest weight in the final decision.”

When thinking about impact it is important to have a proper evaluation to really understand whether patient involvement is making a difference and is taken seriously. We therefore asked about any formal evaluation process carried out by an HTA agency, a decision-making body (e.g. HTA appraisal committee) or by their patient organisation itself. Only one out of eight responded positively. While four of them reported that their organisation was not evaluated, three indicated that they did not know.

2.2 FACILITATORS AND CHALLENGES FOR PATIENT INVOLVEMENT

Based on the results reported below we can conclude that the most used methods for supporting respondents' organisations are 'easy to read HTA summaries' and 'easy access to key reports/guides/protocols'. On the other hand, the least used methods are 'public documents that describe transparent mechanism in how patient views influence decision-making', 'easy accessibility to journals', and 'regular distribution of newsletters'.

Methods	Often or sometimes used	Rarely or never used	Don't know
Easy, understandable and timely accessibility of information on HTA and how to contribute	3	5	
Easy to read HTA summaries	4	4	
Education and training courses	3	5	
Regular distribution of Newsletters	2	5	1
Easy accessibility to Journals	1	6	1
Workshops, seminars, conferences	3	5	
Easy access to key reports/guides/protocols	4	3	1
Dedicated websites and forums	3	5	
Public documents that describe transparent mechanism in how patient views influence decision-making		7	1

Table 6: How a HTA agency or decision-making body supported involvement

When asked how the patient organisation facilitates the involvement of its representatives in HTA, we found out that the 'easy, understandable and timely accessibility of information on HTA and how to contribute' is the most used facilitator while 'organising workshops, seminars, conferences' and 'easy access to reports/guides/protocols' are quite low in the list. One respondent wanted to detail the way their organisation enables involvement in HTA of its representatives and gave specific information on this:

"As an umbrella organisation we are assisting the disease bound organisations whenever one of their products is being assessed with the "procedural and legal" part of the process and we help them to report the disease related comments as much as possible. We also answer their questions on how to best influence the decisions."

Facilitators	Often or sometimes used	Rarely or never used	Don't know
Easy, understandable and timely accessibility of information on HTA and how to contribute	4	3	
Easy to read HTA summaries	2	5	
Education and training courses	2	5	
Organising workshops, seminars, conferences	1	6	
Easy access to key reports/guides/protocols	1	6	

Table 7: How patient organisations facilitate involvement of their representatives

The majority of the respondents agreed to most of the challenges proposed in the questionnaire whereas only a few disagreed. The 'don't know' option was selected quite often and the highest numbers in this respect relate to the 'commitment from HTA staff' and 'conflict of interest'. The two items which were agreed on the most are 'knowing the stage at which the engagement is needed or most useful' and 'difficulties to understand the technical language'. Moreover, the two elements that more respondents disagreed compared to the others are 'lack of capacity of the patient organisation/representative' and 'commitment from patients/informal carers/patient organisations'.

Challenges	Strongly agree or agree	Strongly disagree or disagree	Don't know
Lack of agreed and good method to provide patient evidence	12	2	8
Knowing the stage at which the engagement is needed or most useful	14	2	5
Credibility of patient evidence	13	2	6
Lack of capacity of the HTA agency to involve patients	12	2	8
Lack of capacity of the patient organisation/representative	12	9	1
Commitment from patients/informal carers/patient organisations	11	8	3
Commitment from HTA staff	9	3	9
Difficulties to understand the technical language	14	5	3
Time intensive	13	4	5
Lack of financial affordability	13	2	6
Lack of interest of HTA agency	13	3	6
Conflict of interest	7	5	9

Table 8: Challenges to patient organisations being meaningfully involved in work with HTA agencies

The main challenges to patient organisations being meaningfully involved in decision-making on health technologies are clearly identified as ‘lack of commitment from decision-makers’ and ‘lack of legal or policy framework for patient involvement in HTA decision-making’. Overall, most of the choices offered were accepted as challenges. Besides these, one respondent indicated “lack of medical expertise among patients and patient organisations” as another challenge.

Challenges	Strongly agree or agree	Strongly disagree or disagree	Don't know
Understanding who/which institution makes the decisions	14	7	1
Finding an interlocutor within the decision-making body/institution	13	4	5
Understanding the decision-making process	13	7	2
Lack of commitment from decision-makers	17	2	3
Lack of legal or policy framework for patient involvement in HTA decision-making	16	4	2

Table 9: Challenges to being meaningfully involved in decision-making on health technologies

The respondents were also asked to give their opinion about the ideal type and level of patient involvement in HTA that their organisations would like to see. The five highest ranked stages of involvement in HTA stages were prioritisation (19/23), diffusion and dissemination (18/23) and producing information (18/23).

Type and level of involvement in HTA stages	High to moderate involvement	Low to no involvement	Don't know
Identification	16	3	4
Filtration	11	5	6
Prioritisation	19	3	1
Assessment	17	1	5
Produce information	18	1	4
Internal review	15	4	4
External review	16	1	4
Diffusion and dissemination	18	1	4

Table 10: The ideal type and level of patient involvement in HTA stages when working with HTA agencies.

With regard to their ideal involvement in decision-making on health technologies the two highest ranked areas were public consultations (20/22) and providing patient evidence that has been included in HTA reports used by decision-makers (18/22). One comment was also received:

“We think consultation, decision-making and that kind of thing should not be a matter of blood, sweat and tears; a matter of life and death (although that, literally, is what is involved). But it should be honest and genuine.”

Involvement in HTA stages through	High to moderate involvement	Low to no involvement	Don't know
Appraisal committees	17	3	2
Public consultations	20	2	1
Provide patient evidence that has been included in HTA reports used by decision-makers	18	1	4
Appeals against the final recommendations of the decision makers	16	1	5
Involvement in prioritising HTA research topics/topic selection/scoping	16	2	4

Table 11: The ideal type and level of patient involvement in decision-making on health technologies

Respondents were then asked whether they know any **good practices** supporting patient involvement in HTA from their experience or that of other patient organisations or sources. 10 respondents responded positively and six of them listed these good practices.

These were described as involvement in appraisal committees; participation in evaluation of the critical trial process for the development of a new drug; ongoing collaboration with the HTA agency/institution.

Respondents were finally asked what they would recommend in very concrete terms to HTA agencies in order for them to actively support patient involvement. Fifteen responses were received. Issues identified included need for better and timely communication with patient organisations, for partnership, for technologies to be better explained, for wider dissemination of information on the benefits of HTA for patients, for transparency, need to ask for patients' opinions more often:

“Patient representatives and organisations should be trained to do the work. They should be involved in a timely fashion (not asked to comment within a week like [...]). They should be

involved in setting the outcome measures and criteria for assessing added therapeutic value and effectiveness, not only in the end in the review and assessment phase, but much earlier when drafting the studies in order to prepare the reports.”

“We recommend HTA agencies to not make decisions for patients, without the patients' opinion. It's important to have an inside view of how a product affects someone. That is necessary for both agencies and patients, since both the patient's life and the agency's reputation are at stake.”

One respondent also mentioned that patient organisations should try to improve their capacity in order to be considered as partners in decision-making:

“Above all, the HTA agencies should see patient organisations as partners in decision making. For that matter, I don't think we should expect any support from HTA agencies because it's the HTA agencies that need the support of the patients organisations during the decision making process. We, as patient representatives, have to work on the technical capacity of our members' board so it's important to have more professional / full-time organisations. So we must conquer the HTA agencies with our capability. Although, we think the decision-makers should write regulations which should open the door to the involvement of those associations that demonstrate the capability to participate in the decision-making process”.

After their recommendations for HTA agencies, respondents were also asked what they would recommend in very concrete terms to the decision makers/HTA appraisal committees in order for them to actively support patient involvement in HTA. The responses raised issues such as need for education and training to improve understanding and competencies, need for cooperation with patient organisations and for adequate time for patient organisations to provide meaningful contributions, and need to discuss what the needs of patient organisations are before making decisions. One of the respondents stated that the HTA agencies that have been in contact with patient organisations should be supported by decision-makers.

3. CONCLUSIONS

Before the main findings are discussed, it is important to mention that the conclusions are formulated based on a limited number of responses. Despite several hundred patient organisations receiving the survey and several follow-ups, only 23 responded. 10 out of the 23 said they are involved in HTA; and of these 10, only eight responded with details about their involvement. Of the remaining 13, 12 of them had never been involved in HTA and one reported that he/she did not know about his/her organisation's involvement. This is a finding in itself indicating that very few patient organisations are active in HTA.

Nonetheless in many respects the responses received serve to validate the understanding developed in the previous surveys with HTA agencies and decision-makers about the need to improve the level, scope and quality of patient involvement in HTA.

Current status of involvement: In both aspects of HTA and decision-making patient organisations are poorly or not involved in stages like scoping and prioritisation where decisions are made about which treatments to assess and what aspects. Without this early involvement there is a serious risk that treatments made available do not respond to patients' needs and that crucial needs remain unmet.

Impact of patient involvement: Respondents clearly think that patient organisations can have a high impact in helping HTA agencies and decision-makers to better understand technologies' impact in real life context and also the quality of life aspects' and thus, leading to decisions that meet patients' needs. The downside is however coming from the fact that patient involvement does not necessarily lead to the integration and weighing of the patient perspective in HTA reports and decision-making. Some of the respondents resented the 'tokenistic' approach and the lack of transparency; the clear message is that patient involvement needs to be taken seriously.

Capacity building: In considering what can be drawn from this survey, a critical issue is one of capacity building and competency. Some of the patient organisations that responded acknowledge that their understanding of and related skills needed to contribute to HTA and decision-making processes need to be improved. On the other hand, the main tools used by the organisations as well as HTA agencies and decision-makers are 'informational' type of tools like reports, summaries, guides, etc. An as complex subject as HTA is requires much more than information to enable patients to contribute actively.

Commitment to partnership: The survey indicates the fact that some patient organisations have the feeling they are not considered equal partners and called for a real commitment from HTA agencies and decision-makers. Commitment is essential, but needs to go hand in hand with know-how on how to work together and a clear framework. These are currently lacking.

Good practice: Very few examples were provided in the survey responses; this seems indeed a field to be further explored. The patient organisations had however a number of suggestions for improving their involvement in HTA. They were expressed as needs, but we could certainly consider them also as good practices: better and timely communication with patient organisations; partnership; better explanations of technologies; wider dissemination of information on the benefits of HTA for patients; transparency; education and training to improve understanding and competencies; adequate time for contributions and discussing what the needs of patient organisations are before making decisions.