How to strengthen the presence of patients in health technology assessments conducted by the health authorities

Marie-France Mamzer, Sophie Dubois, Christian Saout, Nicolas Albin, Jehan-Michel Béhier, Anne Buisson, Vincent Diebolt, Olivier Delaitre, Christophe Duguet, Jean-Yves Fagon, Ségolène Gaillard, Claire Le Jeunne, René Mazars, Joëlle Micallef, Hervé Nabarette, Laurent Piazza, Catherine Raynaud, Nathalie Varoqueaux

Laboratory of medical ethics, university Paris Descartes EA 4569, functional unit of medical ethics, Neckers children's hospital, AP–HP, 75015 Paris, France
Takeda, 92977 Paris La Défense, France
Haute autorité de santé, department of medical, economic and public health assessment, 93218 Saint-Denis-La-Plaine, France
Daniel-Hollard institute of cancerology, 38000 Grenoble, France
AFA, 75019 Paris, France
F-CRIN, 34000 Montpellier, France
Boehringer Ingelheim, 75013 Paris, France
AFM Téléthon, 91000 Evry, France
Ministère des solidarités et de la santé, 75350 Paris, France
Inserm, UCB Lyon I, UMR 5558, CIC 1407 hospices civils de Lyon, 69677 Bron, France
Cochin hospital, AP–HP, 75014 Paris, France

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Corresponding author. Laboratoire Takeda, immeuble Pacific, 11–13, cours Valmy, 92977 Paris la Défense cedex, France.
E-mail address: sophie.dubois@takeda.com (S. Dubois).
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Summary  The constant development of health technologies, combined with the increase in the cost of treatment, means that States must continually make choices about the introduction of new technologies into their healthcare system and how they are to be funded. In France, the systematic participation of patients in these processes is one of the targets to be met in terms of healthcare democracy. Although, on an international level, patient involvement in these assessments is constantly growing, it is difficult to define due to the presence of unstaobilised elements in terms of both terminology and assessment methods. As a result, patient and public involvement in health technology assessments varies considerably from one country to the next, from one field to the next and even from one type of technology to the next. Several types of involvement exist, ranging from studies conducted to collect patient “‘insight’” (experience, perception, needs, preferences, attitudes to treatment and health, etc.) to processes aimed at including patients in assessments (as individuals, as representatives of associations, etc.). Given the scope and complexity of the subject, and the difficulty involved in understanding all the different aspects of health technologies and innovations, the members of the Round Table chose to concentrate on health technology assessments (medicinal products and medical devices) to develop national recommendations on all possible types of patient involvement in the health technology assessment processes conducted by the health authorities in France.
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Abbreviations

ANSM French national agency for medicine and health product safety
ARS French regional health agencies
ATU temporary authorisations
CADTH Canadian agency for drug and technologies
CEESP public health and healthcare products pricing committee
CNEDiMTS committee for the evaluation of medical devices and health technologies
CPP committees for the protection of human participants in research
EMA European medicines agency
EUPATI European patients academy on therapeutic innovation
EUnetHTA European network for health technology assessment
eYPAGnet European young persons’ advisory group
HAS French high authority of health
HTA health technology assessment
HTAi health technology assessment international
HQO health quality Ontario
INHATA international network of agencies for health technology assessments
MCDA multi criteria decision analysis

Introduction
In France, the provisions of act n° 2016—41 of 26 January 2016 [1] designed to modernise our healthcare system reflect the theoretical yet nevertheless real progress that has been made with respect to health democracy issues by expanding the individual rights of patients and strengthening the collective rights of users in our healthcare system. Although the healthcare policy is still “defined by the State”, user participation is to be stepped up, in terms of both its implementation and assessment. In this respect, the legislation authorises the national union of healthcare system users’ associations (Union nationale des associations d’usagers du système de santé [UNAASS], now France Assos Santé) to share its insights with the public authorities on questions relating to the operation of the healthcare system, running associative networks, taking legal action
and representing users with regard to the public authorities. Definition of the national healthcare strategy must now be preceded by a public consultation. Informing the population and its participation in public discussions on health-related issues are included either directly or through representative users’ associations. A series of measures encourage users of the healthcare system (and therefore patients) to participate to a greater extent in the operation and assessment of the healthcare system at various levels — national, regional and local (healthcare establishments, for example) — and in various ways, either by being informed of the assessment results or by contributing directly to the assessments. The Round Table was more concerned with issues relating to patient and public participation in the technology assessment processes implemented by the health authorities and especially with drawing up recommendations designed to strengthen the presence of patients in health technology assessments in France, by comparing data from the existing international literature on the subject with the complementary experience of various participants from different professional areas, more or less directly involved in the existing processes (members of the French high authority of health [Haute autorité de santé (HAS)], representatives of patients’ associations who may or may not be members of the normalising organisations concerned, professionals from the pharmaceutical industry, health care professionals and the ministerial delegate for health innovation). To achieve these aims, the members of the Round Table first clarified the notions and concepts used, circumscribed the field of their reflection and attempted to define the interests and limits of current health technology assessment (HTA) practices in relation to health products before identifying the different forms of public and patient involvement in the HTA process, taking into account the expectations and possible contribution of patients, and proposing, with a health democracy aim, recommendations applicable to all the parties concerned by technology assessment.

Definition of the field covered by the Round Table’s reflection and the subject of the recommendations made

The notion of “health technology” covers all methods, techniques and procedures that can be used to promote health, to prevent, diagnose and treat acute and chronic illnesses, and to further recovery. The field of health technology therefore includes medicinal products, devices, procedures, strategies and systems used for healthcare. The ongoing development of new health technologies, combined with the increase in the cost of treatment, has rapidly forced States with finite budgets to continually make choices about the introduction of these new technologies into their healthcare systems and the choice of funding methods. It has become necessary to dispose of reliable tools to inform health policy decisions as objectively and as transparently as possible, which in turn has given rise to the notion of health technology assessment (HTA). In this context, all health innovations are likely to be concerned by HTA processes, whether these innovations are therapeutic, technological, digital, organisational, behavioural or related to medical diagnosis. Today, however, although the culture of systematic health technology assessment is well-established in the health product sector (medicinal products and medical devices), and particularly in France, most other health-related innovations are not yet covered by systematic formalised HTA processes, even though their use is developing and could easily change treatment choices (digital systems, connected objects, etc.).

Although, on an international level, patient involvement in these assessments is constantly growing, it is still difficult to define due to the presence of unstabilised elements in terms of both terminology and assessment methods. Therefore, considering the scope and complexity of the subject, and the difficulty involved in understanding all the different aspects of health technologies and innovations, the members of the Round Table decided to focus their recommendations on health technology assessment processes (market authorisation, assessment for health insurance reimbursement) to the exclusion of research conducted upstream of market authorisation, although they also reflected on the subject of patient involvement in clinical research. Thus, although patient involvement in research assessments may be pertinent, particularly with regard to the inclusion of notions of loss of opportunity and the respect of safety rules, patient representatives are only present on committees for the protection of human participants in research (Comité de protection des personnes [CPP]), and, in this capacity, called upon to deliberate on issues pertaining to the protection of human participants from an ethical viewpoint rather than on assessment of the benefit-risk ratio in relation to product safety, which lies within the field of expertise of the French national agency for medicine and health products safety (Agence nationale de sécurité des médicaments et des produits de santé [ANSM]). Yet, the theoretical advantage to be gained from patient involvement upstream of these assessment processes at the time the research projects are being designed, including every step from research prioritisation to project completion, has been emphasised as being of great pertinence and poses the question of which stakeholders should be brought on-board to completely overhaul the mechanics of health research. The complexity of the practical aspects of this type of involvement on a scale that is usually international, with variable and often largely implicit levels of patient involvement at this level, means that any recommendations in this respect are illusory. Yet one of the targets to be achieved at this stage would be to introduce assessment criteria into research projects which express patients’ viewpoints or at least take them into account, from the design stage onwards. The ultimate aim of the Round Table is therefore to draw up national recommendations on all the possible forms of patient involvement in health technology assessment processes in France. Patient and user involvement is defined as the use of studies which collect their “insight” (experience, perception, needs, preferences, attitudes to treatment and health, etc.) or processes aimed at including them in assessments (as individuals, as representatives of associations, etc.).
Interest and limits of global health technology assessment (HTA) practices concerning health products

Today, the data used by the public authorities to develop their HTA processes in this field often reflect the “biomedical” spirit in which product development research is conducted. Benefit/risk and cost-effectiveness assessments are based on hard criteria that are clearly objective and reproducible (such as mortality and morbidity) and which only take the notion of context into account at a later stage, if they take it into account at all. The findings of a questionnaire-based survey conducted by the World Health Organisation (WHO) in 2015 on global HTA practices show that the criteria most frequently taken into account for decision-making by the 117 respondent countries are safety and clinical effectiveness followed by economic and budgetary considerations [2]. Little consideration is given to issues of ethics, equity and feasibility, and patient acceptability is largely ignored, regardless of the health technology being assessed i.e. medicines, vaccines, medical devices, clinical interventions, surgical interventions, service delivery models and public health interventions. In more than three-quarters of these countries, HTA-related organisations only play an advisory role and civil society representatives are given the opportunity to comment on the recommendations of an HTA report in fewer than half the countries [1].

Citing the stringent requirements of scientific rigour, objectiveness and transparency, numerous HTA-related agencies hesitate to involve patients in assessment processes, on the pretext or grounds of lobbying risks on the part of the associations, the introduction of a survey bias, the existence of conflicts of interest, even a lack of representativeness or simply the level of knowledge and training, and therefore the pertinence, of the opinions expressed. Alongside these conceptual obstacles, material barriers are mentioned such as the need for resources to support patient participation and the possible need to change processes in order to make their participation more effective (the problem of assessment deadlines, in particular, is often mentioned). Finally, the difficult question of the proper way to process patient information with regard to scientific evidence is a recurring issue [3]. The difficulties involved in making the right regulatory decisions in relation to health technologies have been known for some time. They are due as much to the uncertainties of the experimental data itself as to the difficulty of incorporating heterogenous results from expert appraisals carried out from diverse perspectives, in a unique decision-making context, which itself is distorted by power dynamics when several stakeholders are involved [4]. Analyses carried out by human and social sciences researchers on decision-making mechanisms in the wake of assessments carried out by agencies suggest that the decisions are sometimes based on values rather than on factual arguments [5]. By way of example, a recent study compared health insurance reimbursement recommendations and the processes at the end of which the decisions were made by national health technology assessment agencies, for ten orphan drugs in four different countries (National institute for health and care excellence [NICE] in England, Scottish medicines consortium [SMC] in Scotland, Tandwârs-och läkemedelsförmånsveket [TLV] in Sweden and HAS in France). It objectivised diverging assessments for 6 out of the 10 drugs concerned. An analysis of the processes that led to these different recommendations confirms the very wide variety of practices used not only to select the evidence appraised but also examines why these same data were interpreted differently, and the different ways of dealing with the same uncertainty [6].

Likewise, an ethnographic study of regulatory decision-making processes regarding the cost-effectiveness of three expensive medicines at NICE was carried out from 2011 to 2014 by an independent multidisciplinary team. Based on the well-argued statement that the factual scientific data at the disposal of assessors and experts to confirm or refute the cost-effectiveness ratio proposed by the industrial spon- sor are insufficient to make a well-informed decision (based on a small number of short-term results from clinical tri- als with imperfect assessment criteria), the researchers demonstrated that the decision-making mechanisms were influenced by the feelings of (dis)trust generated by the persons concerned, under the influence of stereotype representations that encourage greater trust in clinical experts than in patient experts. Other criteria such as end-of-life impact or the technological novelty of a medicinal product when they are applied in accordance with good practice recommendations, were taken into account without any real appreciation of the quality of the data provided by the spon- sor to define them [7].

The place of patients in global HTA models: the English and Canadian examples

The assessment of patient and society needs was recognised as being useful in informing political choices at the outset of HTA in the seventies in the United States [3]. In the nineties, the growing pace and number of these processes, mainly aimed at making health insurance reimbursement decisions, led to a decrease in the number of patient-based evidence studies in favour of decisions mostly based on cost-effectiveness calculations. These trends encouraged the increase in patient-involvement processes in order to “complete” this type of assessment. Today, patient and public involvement in health technology assessments varies considerably from one country to another, one field to another and even one type of technology to another. However, despite the initial reticence of the agencies who were afraid that patients would not be able to provide objective, unbiased information, several countries organised public and patient involvement in their HTA processes. Great Britain is one of the pioneers in effective patient and lay involvement in HTA processes which are placed under the responsibility of an independent public organisation called NICE. This model is probably one of the most accomplished models today in the terms of patient and carer involvement, with regard to both the scope of the fields concerned and the possible level of involvement. Patient involvement is multifaceted: consultation of associations both before and after committee meetings, written contributions from associations to be read at meet- ings, participation of patients/carers in meetings and the
committees’ deliberations through the presence of two to six lay members. Patients receive compensation for participating and are invited to speak on their own behalf during committee meetings, even when they are designated by an association. They benefit from a support service that sends them preparatory documents. The documents drawn up by the associations are sent to the committee and published on the NICE website along with all the other documents. During meetings, rapporteurs include a synthesis of patients’ insights at the beginning of the first part of the discussion on clinical factors. Patients and carers are invited in the role of experts on the illness concerned and can answer the rapporteurs’ questions alongside the clinical experts. However, the patients cannot vote. In addition to the assessment committees, a lay committee writes a report on social values and the guidelines adopted are published on-line in a patient/public version. A recent sociological study shows that although NICE has developed ways to incorporate patient insights in its formal processes, in actual fact, only some of the data collected by user representatives is taken into account. The place attributed to patient insights also seems largely dependent on the committee Chair, as the pertinence of patient contributions is a subject of disagreement among committee members, particularly when they speak about their illness but admit that they are not in a position to comment on the assessed product. The associations are also suspected of having conflicts of interest. In the extreme, this sometimes leads to disengagement on the part of patients who are disappointed in how little their contribution is taken into account [8].

In Canada, public and patient engagement in the HTA processes exists on a federal, provincial and hospital level. For example, the HTA agency in Ontario (health quality Ontario or HQO) set up a public engagement subcommittee in 2007 to strengthen patient engagement in health technology assessments carried out by the Ontario health technology advisory committee (OHTAC). Since 2012, the role of the subcommittee is to help HQO strengthen public and patient engagement in its evidence review process in order to foster transparency, awareness, legitimacy, acceptability and trust in OHTAC recommendations. HQO’s policy is now very clear, based on recommendations and measures that facilitate their implementation published in 2015 in a public report [9]. On a federal level, the purpose of the Canadian agency for drug and technologies (CADTH), an independent non-profit organisation, is to provide public health policy decision-makers with all the objective elements available concerning drugs and medical devices to help make informed decisions. Patient groups have been involved since 2010 in the drug assessments carried out by the CADTH to support health insurance reimbursement decisions. The CADTH is known for its voluntaristic policy in the field, the wide variety of involvement methods used and the first assessments of the impacts of patient contributions.

On an international level, hospital HTA processes have emerged over the last fifteen years that are designed to carry out hospital-based health technology assessments to help local decision makers. Most of the HTA’s hospital units are lacking experience in how to involve patients in the assessment process but the subject has been identified as a pertinent assessment field [10] with the appearance of experiments showing the advantage of hospital-based assessment [11,12].

**Public and patient involvement in HTA processes: lay participation in the construction of a patient-evidence-based review process**

The International network of agencies for health technology assessments (INAHTA) comprises 55 government and regional non-profit agencies in 32 countries, including the French HAS. Most of the agencies in the network involve patients and citizens in the assessment process, but there are disparities in the type of person involved, the ways in which they are involved, the fields concerned and the level of engagement which will result in proper, effective involvement.

So in some respects, it is as though there is no real definition of patient involvement tools, although international experiments and experience, empirical studies and conceptual research on the subject exist. In Canada, where public decisions in healthcare have been officially recognised as being intrinsically based on values, and where numerous efforts have been made to effectively involve the public and patients in decision-making processes, theoretical considerations have been widely addressed. In 2010, Gauvin already proposed a tool designed to compare the different conceptual frameworks of public and patient involvement in health technology assessments according to their intrinsic characteristics (attributes) and the importance given to each attribute. For Gauvin, these attributes included engagement (in terms of overall funding policy, organisation of assessment processes and the actual assessments), the type of public (individual citizens, representatives of citizen groups, other citizen representatives, such as MPs, individual patients and users, patient and user representatives) and the level of involvement (no involvement, passive or active information receivers, data producers, commentators, collaborators, participants and regulators) [13]. Many reasons for furthering patient involvement in HTA have been identified: democratic (decisions concerning health technologies must be as legitimate, transparent and informed as possible; all citizens are concerned by public policy making and patients are the first to be concerned by the technologies developed), scientific (patient experience is being increasingly recognised as unique, irreplaceable experiential knowledge), instrumental (making better quality decisions across all stages of the HTA process) and with the twofold objective of increasing public understanding of health technologies and strengthening the public’s and patients’ capacity to contribute to health policy issues [9]. Despite this, the results of a study on integrating patients’ insights into the CADTH’s research and assessment of new drugs in view of making public health insurance reimbursement decisions, are mixed. An analysis of the reports of 30 consecutive assessments carried out between December 2012 and June 2014 shows that patient insights on the drugs examined were effectively taken into account, both by the reviewers to frame an assessment and by the expert committee to interpret the evidence. However, this study underlined the fact that drug trials do not always
measure outcomes that patients consider important, such as survival, symptom relief, the process of recovery, and maintaining health, as no allowance is made for collecting them during the research [14]. Yet the notion of “patient evidence” seems to emerge [3] in addition to the notions of clinical evidence and economic evidence, without its contours being very clear at present. Patient evidence takes into account healthcare experience, viewpoints, insights, needs, preferences and attitudes to produce data and a body of specific knowledge in view of producing health technology assessments, which are more in keeping with the emerging conception of patient-centred healthcare. Literature on patient engagement in HTA processes shows different types of research that may enrich the panel of useful data so that patient insights can help to inform public (and individual) decisions based on more subtle contextualised criteria, particularly since the quality-of-life scales used in international studies are often inappropriate. Among the approaches used, patient-report outcomes measures (PROMs) are the most widespread and the most often studied. The notion of PROM covers all types of individual health status measures directly reported by patients. Their computerization makes them easier to normalise [15] and increasingly convincing studies have been published concerning their scientific exploitability, particularly in cancer [16]. However, their many forms, designed to specifically take patient experience into account in particular contexts, is now considered to be a disadvantage for their normative use in a different pathological context [17]. Similarly, approaches based on ethnographic research and qualitative research summaries are generally given little recognition by biomedical researchers, industrial sponsors and clinicians, who know very little about human and social sciences methodologies or consider that they do not correspond to the required scientific level, even though they are probably the most appropriate when it comes to taking contextual elements into account. Even though they were built according to identified methods (DCE, multicriteria hierarchical analysis) and take preferences into account, these tools, like all multi-criteria decision analysis (MCDA) tools, are not systematically used by the regulatory bodies, despite the fact that these models are particularly useful when the benefit-risk ratio is uncertain and there are contrary attributes [18].

The relevance and limits of virtual patient communities for health product research were examined during the Ateliers de Giens in 2016. The members of the Round Table came to the conclusion that the appropriate use of patient data produced by existing digital platforms could be very useful for research needs as it puts the patient at the centre of the research [19]. Provided they are used rigorously and possible biases are identified, these tools could be a way of producing pertinent data on patient experience, therapeutic experience and the needs and criteria considered to be important by patients.

At the present time, the standardisation of tools and the sharing of experience among assessment agencies are handled by HTA’s global scientific and professional society, health technology assessment international (HTAi). An interest group on patient and citizen involvement develops and disseminates resources at the disposal of HTA agencies and patients’ associations. European network for health technology assessment (EUnetHTA), which supports collaboration between European HTA agencies, is starting to identify practices to be promoted on a European level.

Place of patients in health product assessments conducted by health authorities: official feedback

In France, national health product assessments are mainly carried out by two State agencies: ANSM, the French national agency for medicines and health products safety, and HAS, the French national authority for health. Their missions are organised to inform public authority decisions at the different stages in the life cycle of health products: research authorisation, market authorisation and health insurance reimbursement. However, in actual practice, there are other more local assessment levels, such as regional health agencies (ARS) and healthcare establishments. The ANSM is officially involved in the initial assessment and reassessment of the benefits and risks related to the use of human healthcare products. As the competent authority, it plays a major role in the entire life cycle of health products, from the research phase onwards. It authorises clinical trials which have been approved by the committee for the protection of human participants in research (CPP) and decides on refusals and suspensions. It can implement patient monitoring and efficacy and tolerance data collection studies, including post-authorisation, when needed [20]. It also makes marketing authorisation, extension, renewal and transfer decisions. The ANSM’s policy regarding the involvement of healthcare system users is fairly open—approved patients’ associations can report suspected adverse effects. They are authorised to ask the ANSM to examine a temporary recommendations for use dossier for the safe use of a medicinal product that has not yet been given marketing authorisation. Representatives of approved user associations sit on the ANSM’s board of directors and can vote at meetings of its consultative committees (initial evaluation of benefit-risk ratio of health products, monitoring of benefit-risk ratio of health products and narcotics and psychotropic substances). An interface committee has been set up to establish ongoing dialogue between patients’ associations and the Agency. Thus, the ANSM has a long-standing partnership with the representatives of healthcare system users, in the spirit of the act of 29 December 2011. However, patients are only indirectly involved in the ANSM’s clinical trial authorisation processes and other interventional research through their participation in the deliberations of the CPP, and in marketing authorisation decisions for medicinal products. In Europe there are four marketing authorisation procedures for medicinal products: a centralised procedure, a mutual recognition procedure, a decentralised procedure and a national procedure which only results in marketing authorisation for the Member state who implements it [21]. The marketing authorisation for medicinal products obtained through the national procedure is delivered by the Director General of the ANSM on the recommendations of the marketing authorisation commission. European marketing authorisations are delivered by the European medicines agency (EMA) on the recommendations of the committee for medicinal products for human use. The ANSM is also
Place of patients in health technology assessments

responsible for delivering temporary authorisations (ATUs), which can be for either cohorts or individual patients. Patients partake in these decisions through their participation in the initial consultative assessment commission which examines all ATU and RTU dossiers, without there being a direct involvement system, even though there is the possibility of collecting patient outcomes, at this stage.

In the field of medical devices, the ANSM’s role is to monitor and control the market but it is the manufacturer who is responsible for the CE marking procedure and, in the case of high-risk medical devices, the manufacturer must apply to a notified body (designated and monitored by the competent authority). This body must ensure that the manufacturer’s dossier meets the regulatory requirements of CE marking according to a procedure that depends on the identified risk level for the patient. Where applicable, the patients’ opinion of the medical device can be sought by the manufacturer to meet an essential marking requirement if risks have been identified relating to use, ergonomics or human factors concerning the use of the device. However, there is no patient involvement in the CE certification decision for a medical device which is the sole prerogative of the notified body at the end of the CE marking procedure. The post-authorisation monitoring studies implemented by the manufacturers or academic bodies (particularly teaching hospitals) can provide the opportunity to collect patient insights and patient-reported outcomes. For medical devices, this phase can require several years before a health insurance reimbursement dossier can be submitted.

The HAS, as an independent public authority of a scientific character whose missions have just been redefined by act 2017–220 of 23 February 2017, is directly involved in HTA processes. It is responsible for assessing the relevance, efficacy and advances represented by medicinal products and medical devices, in view of reimbursement by public health insurance, and fixing of their retail price by the CEPS (healthcare products pricing committee). It periodically assesses the actual benefit of health products, procedures and services in France. Through its recommendations, it contributes to decision-making relating to the registration and health insurance reimbursement of health products, procedures and services and to special reimbursement conditions for treatment given to patients with long-term conditions, via three commissions: the Transparency commission (CT) which assesses medicinal products in view of reimbursement, the National committee for the evaluation of medical devices and health technologies (CNEDIMTS) which assesses medical devices and professional patient procedures, and the Public health and healthcare products pricing committee (CEESPP) responsible for ensuring that the relevance of a strategy or product for society is taken into account in any related decisions, especially those of pricing and reimbursement. The HAS also gives recommendations on the prescription, production and user conditions of health procedures, products and services, in addition to their efficacy and efficiency. It carries out and validates the medico-economic studies required to assess certain health procedures, products and technologies. Patient involvement by the HAS in HTA processes takes several forms. A patient collaboration framework was drawn up in 2008, with the aim of treating patients on the same level as professionals (compensation rules, etc.). The representatives of approved associations of health system patients and users are voting members of the three commissions involved in the central assessment of health products (CT, CEESPP, CNEDIMTS). For several years now, the HAS has involved patients and users in its long-term actions (assessment of public healthcare, certain procedures and medical devices), including guideline, reporting, consultation and validation phases, which all offer opportunities for involvement which is defined according to the need felt by the assessors and different methods can be used depending on the service concerned. The “first-come first-served” activity is different because of the short deadlines which make involvement more complicated, especially due to the absence of a guideline phase upstream of the research. Historically, there is a public hearing tradition for medical devices at the CNEDIMTS: the HAS invites patients’ associations to targeted hearings from time to time. More recently, it developed a formal procedure aimed at increasing the presence of patients’ associations in HTA processes by creating organisational conditions that will enable associations to propose contributions to medicinal product and medical device assessments. With this aim in mind, each week, the HAS updates the list of scheduled assessments for which a patient contribution is possible, that is, those which require in-depth studies and for which the manufacturer concerned has accepted to put relevant information on-line. The associations can access the following information: name of product, marketing authorisation indication (medicinal product) or CE marking (device), reason for assessment and contribution deadline. Patients’ associations can then submit their contributions using the patient’s viewpoint questionnaire, according to a process inspired by models implemented by other HTA agencies (NICE in England, SMC in Scotland, CADTH in Canada) and tools developed by HTAi. Once the contribution has been drawn up and submitted to the HAS, it is sent to the project manager and all the commission members before the meeting takes place. The initial results of this recent initiative designed to increase patient involvement in assessments are highly contrasted. There is limited acceptance on the part of manufacturers to put information on-line that will enable patients to contribute (on-line agreements were given for 76 medicinal product dossiers out of 84 in 6 months with agreements for 7 out of 12 medical device dossiers). During the six months of the experiment, the number of contributions was higher than expected (24 contributions concerning 22 medicinal products but only one contribution for six assessable medical devices). A total of nineteen associations made contributions for all the health products combined, including twelve approved associations. All the contributions made within the deadline were sent to the project managers and members of the commissions concerned, along with the other preparatory documents. Out of the 24 contributions received for medicinal products, only one could not be included because it concerned an indication that was not part of the assessment to be made by the commission. Incorporation of the contributions during the meetings varied. Mention was not always made of the patients’ contributions during the commission meetings. When they were mentioned, they were not cited in full and their use was
fragmentary. The incorporation of these elements into the recommendations made by the commission was also varied. A debriefing was subsequently organised by the HAS with the associations who made contributions [22]. During the Round Table, the feedback was positive on the whole and underlined the quality of the support provided by the HAS throughout the process. The patients’ associations were satisfied with their role of catalyst capable of circulating pertinent information between the patient community they represent and the health authorities. Given the inappropriateness of the quality-of-life scales generally used to determine usefulness (particularly QLQ-C30), producing suitable alternative data of sufficient quality within the prescribed deadline was seen to be a real difficulty that requires specific resources and know-how on the part of the associations concerned (for example, some of the associations develop their own quantitative questionnaires in the absence of a validated questionnaire, or work with data scientists to use big data tools and perform data mining on experiential data published on forums). The manufacturer’s right to refuse publication of its application for health insurance reimbursement and provide the association with access to the product dossier are seen by the associations as preventing them from gauging their input before submitting their contributions. Despite this encouraging feedback, patients’ interrogations correspond to international interrogations concerning the real expectations of the public authorities and assessment agencies, the possible impact of patient contributions, their opportunity cost and, more especially, the way in which patients can hope to contribute as fully-fledged partners.

In conclusion, the emergence in France of the HTA hospital process may offer a new opportunity to provide the national authorities with feedback on the real-life use of health technologies and involve patients in decisions on a local level [23].

Recommendation framework

Given the many different possible forms of patient involvement, its place and posture are not always the same and correspond to different roles. In the commissions, for example, patients are considered to be experts like anyone else and they are not there to be the official spokesperson for the person at whom the product is aimed, which explains why it is important that the patient’s viewpoint concerning a given product should be available well beforehand.

The different points that the Round Table participants consider should be taken into account when drawing up recommendations are as follows: the assessment framework (organisation, national or regional level), the type of technology to be assessed, the assessment time frame (authorisation, initial registration, reassessment, pre-registration, post-registration, etc.) and expected patient contributions.

The definition of the type of involvement and the patient profile depends on the type of viewpoint needed to meet the different assessment aims. Patients can participate as commission members (either representing civil society or as experts) or be involved as concerned persons in the assessments through direct contributions and can express themselves in different ways: questioning (for reasons of transparency, for example), a statement of facts, production of data (on experience, viewpoint, patients’ preferences, etc.).

The question of training patients is crucial and is the subject of reflection on a European level, with, for example, the implementation of European patients’ academy on therapeutic innovation (EUPATI) and European young persons’ advisory group (eYPAGnet), both aimed at training patients, young persons and adults in the requirements of biomedical research. The question of how to train members to sit on committees, represent patients and participate in assessments is not limited to patients’ representatives but applies to all commission members. The institution chooses these members according to their individual characteristics and resumés.

General insight skills on the part of patients are needed when collective interests are to be defended. This can only happen if the members concerned are able to offer pertinent patient insight during the discussion and demonstrate the relevance of patients’ contributions, a lack of knowledge in the field, etc.

The following recommendations drawn up at the end of the Round Table are addressed to all the stakeholders. Some of them can be implemented without changing the regulatory framework while others cannot.

Recommendation 1: patients and users should be able to participate systematically in all health product assessments

All institutions responsible for health technology assessment should not only include health system patients and users among the regular members of its commissions but also systematically welcome patients’ contributions. The interested patients should have the skills required for the type of assessment concerned and the level of involvement expected. When collective interests are to be defended, the patient profile is that of an approved association representative, whose role is to contribute to discussions as a patient. In addition to these general contributions, it must be possible to take specific contributions into account based on the life experience of the persons concerned. When these contributions are made by associations, the associations may or may not be approved. These possibilities should be offered for all future health product assessments, throughout the HTA process or at least during all the key phases (clinical trials, ATU and RTU applications and other derogations, marketing authorisation applications, post-marketing authorisation, CE marking for medical devices, etc.) and all reassessments, at whatever level (national, regional or hospital). They should not depend on obtaining the authorisation of the manufacturer concerned.
Recommendation 2: patients’ contributions should be an integral part of the assessment dossier throughout the assessment process

When a contribution is proposed by patients, it should be systematically taken into account during the deliberations that lead up to the final decision. The formal contribution of patients should be considered to have the same importance as that of other experts, regardless of the type of information concerned (demonstration, questioning or simple statement of fact). Each institution involved in the health technology assessment process should offer patients’ associations the possibility of developing formalised interactions with its assessment services, regardless of the type of interrogations, hearings or technical exchanges during preparation of the dossier, and before submission to the commission. During meetings, patients’ contributions must be presented by the assessors, as early as possible during the process, so that they can be used during all the discussions. Transcriptions, videos and minutes of meetings should be published quickly, at the same time as the recommendation. All recommendations made by commissions should include a synthesis of patients’ contributions in the form of a separate paragraph.

Recommendation 3: to meet the objective of incorporating the patient’s viewpoint into the assessment, it must be possible to carry out studies. One of the factors that determines the quality of these studies is patient involvement in their conduct/design

It appears to be urgent to develop and validate quality-of-life tools and define criteria to use them in a standardised way. This implies patient involvement in prioritising research (definition of patient outcomes by the patients themselves, research design particularly in terms of quality of life and loss of opportunity). The development of these tools and the production of these data should not be the responsibility of public institutions alone but should be shared with the manufacturers as part of their responsibility to society.

Recommendation 4: targeted patient contributions should be requested by assessment bodies without prejudice to the rules of openness and transparency

All the institutions concerned must create a cooperative framework with patients’ and users’ associations that is conducive to health technology assessments that take patient insights into account. All dossiers must be submitted for consultation, and the informative content (product, population, comparator, assessment criteria) must be systematically shared with the associations, in the absence of the manufacturer’s complete dossier.

Over and above these rules of openness and transparency with respect to patients, dossiers for which patients’ insights are particularly important and expected should be identified (according to criteria) by the assessment organisations and notified to the patients’ associations. A decision tree would be useful to prioritise dossiers for which patient feedback is essential before they are examined. When the patients’ insights are essential, they can be inputted either by patient participation or the use of data from the literature and published studies. When the pathology concerned by the health product is not represented by an association, but the patient’s viewpoint seems to be particularly pertinent, it must be the responsibility of either the patients’ community or the assessment agencies to find a way of inputting their insight during the assessment.

Recommendation 5: the transparency requirement with respect to potential conflicts of interest applies to all stakeholders

The application of legislation concerning the transparency of potential conflicts of interest must be the same for all the stakeholders, without any special treatment for patients with respect to the other stakeholders during health technology assessment processes. When the patient sits on a commission as an expert, the rules that apply to the patient in terms of transparency and the management of potential conflicts of interest are the same as for all the other experts. This should not make any difference to the way in which the contributions of patients’ associations are taken into account during deliberations.

Recommendation 6: the training of members of associations in participating and making contributions during assessment processes is the associations’ responsibility

The representatives must be trained by the associations, either by the individual associations or by France Asso Santé, in conjunction with the institutions. Dedicated public funding must be provided in this respect.

The aims of this training must be to foster proper understanding of the issues and expectations relating to patients’ contributions so that they can be exploited to the full during assessment.

Conclusion

Patient and public involvement in health technology assessment is theoretically self-evident on an international scale, even though its practical implementation remains difficult and complex due to the presence of unstandardised elements with regard to both terminology and assessment methods.
Under these conditions, and after deciding to limit the field of their recommendations to health technology assessments carried out by the French health authorities, the members of the Round Table concluded that the possibility of participating in all health technology assessments should be systematically offered to patients in order to respect the rules of health democracy. Given the assessment processes used today by the official bodies, the priority issue is not that of widening the scope of users’ tasks but rather of increasing the involvement of the persons concerned, through contributions that are specifically designed to share their insights. It therefore appears that, under these conditions, patients’ contributions should be an integral part of the assessment dossier throughout the assessment process. Strengthening patient participation in these assessments must therefore necessarily include the systematic inclusion of patients’ insights. This also means that studies that are specifically geared to collecting patient insights must also be taken into consideration, which means patient involvement in the research design stage in order to contribute to the development of useful tools for the production of pertinent data. The question of the necessary training of association representatives is a priority, which is the responsibility of the associations, in conjunction with the official bodies concerned. The need to diversify the tools, methods and joint research approaches would seem necessary for effective patient involvement based both on participation processes and patient-evidence data.

Disclosure of interest

The authors declare that they have no competing interest. Hervé Nabarette is in charge of the associations’ contribution project for the assessment of medicinal products and medical devices.

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Place of patients in health technology assessments

105

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