



eunethta

**EUnetHTA Stakeholder
Discussion Topic Catalogue**

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Since EUnetHTA was established in 2006 it has been the ambition for the Collaboration to be responsive to external actors and exchange expectations and views on HTA with HTA stakeholders. Thus part of Work Package 6 has been dedicated to strengthening the interaction between EUnetHTA and HTA stakeholders. During the years stakeholders have been given the opportunity to comment on EUnetHTA output just as stakeholder information has been provided at EUnetHTA's website. EUnetHTA appreciate all comments and suggestions received; they are all listened to and considered in relation to EUnetHTA's developmental work. To develop dialog between EUnetHTA and HTA stakeholders members of the EUnetHTA Work Package 6 have developed this paper trying to synthesise all received stakeholder comments and opinions to overview issues which need clarification. By providing this catalogue we hope to build a platform for qualified discussion with stakeholders regarding the further development of the EUnetHTA Collaboration and European HTA processes. We are looking forward to engage in constructive dialogue with stakeholders at the planned stakeholder meeting in Rome, June 2008.

The paper mainly builds upon comments to the EUnetHTA Proposal of November 2007. Comments have been received mainly from the industry, but EUnetHTA has also received responses from patient organisations, national ministries of health, national HTA agencies, the International Network of Agencies for Health Technology Assessment (INATHA) and Center for Medical Technology Policy (CMTP), which cover interests of both patients, clinicians, payers, manufacturers and researchers.

Further sources of information contain positions papers on HTA from the industry, an article discussing key conceptual and policy issues related to Coverage with Evidence Development (CED) brought up at an Health Technology Assessment International (HTAi) Policy Forum Meeting, a summary report of a workshop on rare diseases held by the European Platform for Patients' Organisations, Science and Industry (EPPOSI), a visionary book on future health care from Health First Europe (HFE), which is an alliance of patients, doctors, nurses, academics, experts and industry. The sources of information have been obtained via direct communication from stakeholders to EUnetHTA and via search for position papers and the like on websites, which have been considered relevant (see annex 1).

Viewpoints presented in the paper may not cover opinions of all HTA stakeholders, but they do nevertheless reflect the material available to the authors. We consider the development of this discussion catalogue to be dynamic. Therefore we will be pleased to receive further input to qualify the paper, just as questions can be removed from the catalogue when they have been clarified.

The following topics have been identified:

1. Organisation of the future EUnetHTA Collaboration

1.1. Aim of EUnetHTA 2009+

Should the EUnetHTA Collaboration develop common HTA methods, outcomes or both? Should focus be on best or good HTA practice?
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Almost all respondents to the Proposal for future EUnetHTA Collaboration commit to the overall aim of a sustained European HTA Collaboration to advocating and facilitating robust, transparent analysis, which can improve decision-making on the quality of national health care services (1-12). The Scottish HTA agency, the Icelandic Directorate of Health and patient organisations point to the possibilities of ensuring development in countries with limited HTA capacity via the EUnetHTA Collaboration, and in relation to this INATHA ask for further clarification on how extensive the input from EUnetHTA to the countries will be (6,8,9,13).

The respondents also generally support the aim of developing common methods and information sharing. In relation to this it is emphasised by stakeholders from the industry that methods for assessment varies between countries, that HTA methods are still in an evolving face, and that there are considerable national differences in the relation between national HTA agencies and policy makers (1,2,14). In addition to these responses it was concluded at the 8th EPPOSI Workshop that development of a common European model for HTA of orphan medicine will be beneficial as it will simplify the task for national HTA agencies, make efficient use of scarce resources and knowledge, and speed the time between development and access (13). Regarding information sharing INATHA stress that non-European HTA institutions also should have access to the products of EUnetHTA activities (6).

Considering the development of common methodology, concern has been raised that too much standardisation implies a risk for stagnating the development of assessment approaches (4). Stakeholders from the industry therefore encourage the EUnetHTA Collaboration to make clear if focus should be on developing *best* HTA practice or *good* HTA practice, and further what the incentives (and sanctions) should be for (not) adhering to such methodological recommendations (1). The question is how bound national HTA agencies should be by the methodological developments obtained within the EUnetHTA Collaboration.

When it comes to developing common outcomes (Core HTA) and transferring HTA results between national contexts, stakeholders from the industry generally remain sceptical about the idea. They question the realism of this aim, as they stress the importance of considering the local context when assessing health technologies (1-3,14). The national Italian HTA agency on the other hand strongly supports a common HTA production within EUnetHTA (12). It should therefore be clarified how the common outcomes of EUnetHTA differ from the national HTA production, and if/when the EUnetHTA Collaboration should engage in activities at this level of collaboration (elaboration in section 1.5).

1.2. Governance of EUnetHTA 2009+

Which relations should the EUnetHTA Collaboration have to external actors at national, european and international level?
How to assure the accountability and reliability of EUnetHTA?

Regarding the EUnetHTA Collaboration's relations to external actors, stakeholders stress the need for clarification of the nature of these relationships. Stakeholders point at external actors at three levels: national, european and international. At the national level clarification is needed regarding the EUnetHTA Collaboration's working relationship with political units making coverage decisions. At the European level stakeholders point to unclear relations to the European Commission, and finally at the international level it does not seem clear how the EUnetHTA Collaboration relate to existing international networks operating in the HTA field (e.g. INATHA, ISPOR and HTAi) (4,9,14). The national HTA agency of Poland also emphasise the importance of clarifying the legal identity of EUnetHTA as this will have a profound impact of the development of the Collaboration (10). The arguments presented on this topic mainly reflect considerations about the credibility of the Collaboration. Considering the relations to external actors at national and european level, stakeholders from the industry state that the EUnetHTA Collaboration must be clearly separated from and independent of both national political units making coverage decisions and the European Commission to be seen as an independent and credible voice on issues related to HTA (1,4,15). At the international level INATHA welcomes collaboration with EUnetHTA and suggest a detailed collaboration plan is developed and agreed by both parts (6). In the light of the above comments it seems relevant to clarify who is included in the EUnetHTA Collaboration, who is considered external actors at national, european and international level and how the working relationship with these external actors can be characterised.

Again touching upon the credibility of the EUnetHTA Collaboration stakeholders from the industry emphasise that it is unclear who the Collaboration will be accountable to, and how the quality of EUnetHTA outputs can be guaranteed (1,2,14,16). One stakeholder encourages EUnetHTA to share yearly objectives

and measurements with stakeholders and call for audit of EUnetHTA (1). As a framework for assessing quality of EUnetHTA outputs, it is suggested by another stakeholder from the industry that the benefits of HTA should be assessed in both process improvements and improved health care (4). Building on this it seems that the EUnetHTA Collaboration would benefit from undertaking a more general discussion about assurance of the Collaboration's credibility, and in relation to this it should be discussed how the quality of the Collaboration's work can be assured so the products seem credible to stakeholders.

1.3. Financing of EUnetHTA 2009+

How should the EUnetHTA Collaboration be financed, and how do the financial and the organisational structures mutually influence each other?

Several respondents to the EUnetHTA Proposal point to the need for clarification of the financial structure of the EUnetHTA Collaboration, and encourage consideration of the mutual influence of organisational and financial structures (1,4,7,10,17). More specifically one stakeholder from the industry encourages exploration of public-private financing options; particularly if the EUnetHTA Collaboration will be structured as a member organisation (4). The Polish HTA agency on the other hand clearly states that the Collaboration should rely on public financing to ensure its credibility (12). The Dutch Ministry of Health hold the view that EUnetHTA should prioritize development of structural funding mechanisms, while the UK Department of Health show concern about being "double charged" as they already fund national HTA agencies. Building on these comments it should be discussed how possible financial and organisational structures of the network match each other and which structures that best allow obtainment of EUnetHTA's objectives.

1.4. Stakeholder involvement in EUnetHTA 2009+

Which stakeholders should be involved in the EUnetHTA Collaboration and how?
How can stakeholders contribute to the Collaboration and what do they gain from it?

Several respondents opt for greater influence of stakeholders in a future EUnetHTA Collaboration. Stakeholders from the industry generally show concern about stakeholders playing a limited a role in the EUnetHTA Collaboration. They stress that the current organisational model only allows stakeholders an advisory role as they are not represented as a direct partner in the Collaboration (1,2,4,14,16). The Stakeholder Forum is seen as insufficient to assure stakeholder influence as there is no clear accountability for the EUnetHTA Collaboration to take on any opinions from stakeholders or publicly respond to them (1,2,4). The Scottish national HTA agency urge for more productive links with industry and health care providers (9) and the national HTA agency of Austria calls more specifically for inclusion of representatives from social insurance institutions and health care administration in addition to the already defined stakeholder groups (11). Further it was concluded at the 8th EPPOSI Workshop that patient representatives should be included in the conduct of HTA, in the same way as they are already included in many other aspects of assessment of new medicines (13). Considering the above comments it should be clarified how stakeholders can be involved in EUnetHTA activities, which kind of influence stakeholders should have and how such influence can be assured.

Stakeholders from the industry suggest that manufacturer organisations, patient organisations, scientific and learned societies are included in the "shell" of the Collaborations and/or represented in the Steering or Executive Committee (3,4,16). The Italian national HTA agency states however that committee members should be selected only from EUnetHTA "nucleus organisations" to ensure the credibility of the Collaboration (12). Stakeholders from the industry further call for development of formal procedures for stakeholder involvement (1,4,18) and initiation of regular assessments of the impact of HTA on stakeholders (4). In relation to this it should be elucidated which EUnetHTA activities such formal procedures should target and what they should include. Further it should be clarified if assessment of HTA impact on

stakeholders should be undertaken within the EUnetHTA Collaboration and eventually how. Likewise it should be discussed how the EUnetHTA Collaboration can make use of certain stakeholder groups' direct experience with target technologies and therapeutic areas (16), and the industry's special competences regarding horizon scanning (1,16), and finally it should be considered how stakeholders can gain from contributing to the Collaboration.

1.5. Levels of collaboration in EUnetHTA 2009+

How to distinguish between national and european HTA responsibilities and how to understand the division between appraisal and assessment with regard to EUnetHTA activities?

The challenge for the EUnetHTA Collaboration, as pointed out by the national HTA agency of Poland, is to balance the considerations about national identity and effective network management at the international level (10). It seems from stakeholder comments that this balance is not perfectly clear.

Considering collaboration levels stakeholders from the industry generally argue that the EUnetHTA Collaboration is not ready to advance to a more binding collaboration as the internal structures of the network are not clarified (1,2,16). That is the EUnetHTA activities should for the present focus upon horizon scanning, development and maintenance of databases along with methodological development (14). One stakeholder from the industry does however open up for possibly undertaking economic modelling at the international level when more specific economic assessment is undertaken locally (18).

The same stakeholders are further concerned that joint actions and common decisions in relation to european HTA processes (Level 3 Collaboration) relate more to *making* than *informing* policy. They stress a risk of blurring the division of responsibility between the national and the european level (1,2,4,14,15). In addition to this it seems unclear to stakeholders which actors are responsible for undertaking assessment processes and which are responsible for making appraisals based on the assessments (2,4,15). It is suggested by one stakeholder that appraisal processes are undertaken at the national level as it involves considerations about local needs and resources (15). Along with this the Polish national HTA agency emphasises that technology assessments should be clearly separated from the policy level, and explains joint HTA production is not about influencing each other's health policy but about saving resources by joint efforts (10). It is also one of the main conclusions from the 8th EPPOSI Workshop that HTAs cannot replace political discussion about health care priorities, and that politicians should withstand their responsibility to undertake such morally influenced decisions (13). The interface between national HTA agencies, national policy makers and the EUnetHTA Collaboration should therefore be considered, and EUnetHTA's role in relation to HTA assessment and appraisals should be clarified.

2. European HTA processes

2.1. Transparency

How should transparency of EUnetHTA processes be assured?
Who should get insight in which processes?

Stakeholders from the industry and patient organisations have pointed to the transparency of HTA processes as an important topic to consider. They generally agree that European HTA processes must be fully transparent (4,13,15,16). In line with the criticism of lacking stakeholder influence the stakeholders from the industry criticise EUnetHTA for lacking transparency in work and results (1). They note among others that no proposals are made by EUnetHTA in terms of guaranteeing transparency of the joint activities undertaken within the Collaboration (14) and they encourage reflections about the limitations of HTA findings when communicating HTA results (4). It should be considered how the Collaboration can ensure transparency of its work and how the Collaboration will consider communication of HTA results.

2.2. Timing of HTA

How to assess “right” time for assessment?

Regarding the timing of HTA assessment, an important question resembles how to balance the considerations of early access to health care innovations towards the need for prioritising with regard to limited resources in the health care sector (19). Several stakeholders opt for a more flexible timing of the assessment process, e.g. continual consideration of a technology throughout its life cycle (iterative instead of one-off assessment) (1,3,4). Some stakeholders from the industry state that real life clinical data may be needed to make sound evaluations of a technology’s effectiveness (3,15), and they urge EUnetHTA to pay attention to the risk that HTA might work as a delaying factor hindering patients’ rapid access to health interventions (4). Discussions in HTAi Policy Forum have also made clear the uncertainty related to the timing of assessments. An article summing up key issues on CED thus describes that assessment early in a technology’s life-cycle will often result in great uncertainty about long term effects, which makes coverage decisions very difficult (20). In relation to this decision makers on the other hand face several important tradeoffs as they must balance the risk of undertaking inappropriate health policy decisions without unduly delaying access to innovations, ensuring flexibility in urgent situations without creating a precedent for generalisation, and finally they face a tradeoff between rewarding truly innovative technologies while keeping within the national health care budget (21). Building on these considerations a more general discussion highlighting the important dilemmas related to health technology innovation should be undertaken, and in relation to this it should be considered when European HTA processes should be obtained to best balance the need for innovation against the need for cost containment.

2.3. Relation between HTA and other assessment processes

Who should investigate which topics?

Stakeholders from the industry point to the fact that various requirements and formal assessment procedures already exist and must be met before medical devices and pharmaceuticals can be introduced on the European Market (18). To ensure that the work of EUnetHTA adds new value over existing assessment procedures it should be clarified how HTA differs from existing procedures.

3. HTA methodology

3.1. Choice of assessment approach

Should different approaches for different technologies be developed?

There seem to be disagreements among stakeholders whether HTA as a methodological approach is suited for all health technologies or if the approach should be adjusted to the technology in question. Thus it is argued by a stakeholder from the industry that no single assessment approach will suite all technologies as they vary a lot (18), while other stakeholders also from the industry claim that HTA is a useful method regardless of the type of technology assessed; that is HTA should apply to all health care interventions including methods for prevention, diagnosis, treatment and health care systems (2,4,15). In the article building on discussion in HTAi Policy Forum the authors focus instead on different types of evidence suitable for assessment of different types of technologies (20). The scope of the HTA methodology should therefore be considered with and among stakeholders.

3.2. Assessment criteria

How to assess “value” in relation to health technologies? How to perceive and obtain “evidence”?

Many stakeholders call for clarity and consensus about definitions of the criteria used when health technologies are valued, and they further call for transparency regarding prioritization in the health care sector (2-4,15,18,22).

Stakeholders from the industry and patient organisations support a broad approach to HTA including several academic/scientific disciplines and they take a holistic view of what constitutes benefits (3,4,13). The call for a comprehensive approach is most often mentioned in relation to economic evaluations. It is stated that economic assessment of health technologies should include a societal perspective and take into account issues such as quality of life, savings related to avoided hospitalisation and lost working capacity (3,13,15,18). On the other hand stakeholders warn against a narrow, so-called silo-mentality in economic evaluations (4,18), and Health First Europe (HFE) more specifically states that elimination of the boundaries between social services and health care services would add to a better platform for efficient resource allocation (23).

Patient organisations also call for a broader perspective in the economic evaluations of health technologies. They further stress, and HFE support this view, that the focus on costs in the HTA discourse is overwhelming while almost nobody talks about *investments* in the health of European citizens (22-23). They urge for transparency in economic evaluations – both in regard to industry’s pricing of new technologies and measures included in HTA (13).

Criteria for assessing value of health technologies are a fundamental question of greatest importance for the further methodological development within EUnetHTA. As clarity seems to be lacking in this field a discussion of what constitutes value in relation to health technologies and how to measure such value should be undertaken. Regarding prioritization in the health care sector with regard to technological innovation it seems to be a very relevant topic to discuss among stakeholders and decision makers at national level.

Considering the perception of evidence, stakeholders from the industry and patient organisations remark that it may not be valuable to rely solely on a narrow scientific perception of evidence. They explain that in the case of for example rare diseases the relevant patient populations may be too small to obtain enough statistical power to demonstrate a significant effect of the technologies in question (18,13). They therefore encourage use of data obtained from other studies than randomised controlled trials (18,13). One stakeholder suggest a collaborative development of research portfolios for emerging technologies within EUnetHTA thereby commonly defining the evidence that should be developed to make a sound assessment of the technologies in question (5). A more general methodological discussion about the strengths and limitations of different study designs should therefore be obtained with the aim to facilitate convergence in the perception of evidence.

4. Policy considerations

4.1. Appeal processes

How to assure formal appeal processes for stakeholders and which level to apply appeal options at?
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Several stakeholders from the industry call for formal appeal processes in relation to HTA processes. They encourage establishment of appeal options both in relation to coverage decisions (18) and EUnetHTA outputs/recommendations (1,2,16). Building on this it seems relevant to clarify if and eventually how appeal

options can add value to European HTA processes. It further seems relevant to discuss the nature of EUnetHTA outcomes with stakeholders.

4.2. Relation between HTA and policy level

How to prevent “misuse” of HTA?

Stakeholders from the industry call for clarification of the relation between HTA and the policy level, especially how HTA impact on decision-making regarding technological innovation. They are concerned that policy makers may “bend” the evidence from HTA to limit choice of and access to innovative technologies thereby making HTA a tool for economic retrenchment in the health care field (2,14,15). It seems that greater mutual understanding between industry and governments on the functioning of HTA mechanisms is crucial (15), and that further consideration about the links between European HTA processes within the EUnetHTA Collaboration and political decision making with regard to health technologies is needed.

4.3. Flexibility

How to assure flexibility in reimbursement decisions?

Stakeholders acknowledge the difficulties related to balancing the need for technological innovation in health care against the need for cost containment. Holding the view that eventual uncertainty about the effect of health technologies should benefit the patients; stakeholders from the industry encourage considerations about more flexible reimbursement decisions (15). They suggest implementation of interim funding along with continuous assessment of new products while they are launched at the market (18,3). This is also the main point made in the article summing up key issues from an HTAi Policy Forum discussion. It is stated that Coverage with Evidence Development (CED) is one of several policy options, which possibly can overcome problems associated with making coverage decisions under uncertainty, thus increasing the access to treatment while reducing the risk of implementing potential harmful technologies (20). One stakeholder considers EUnetHTA as a valuable forum in which possibilities for conditional reimbursement can be systematically critiqued (5), and the English national HTA agency consider the controlled introduction of new technologies when evidence is still sparse a unique opportunity for the HTA community to directly inform policy making (19). HFE warn that too great uncertainty regarding reimbursement decisions can in worst case function as disincentives for industry to invest in research and development. HFE further foresee that EU Member States will need to adopt significant reforms of their social security systems in order to ensure both their financial and social sustainability, and in addition to the use of HTA they consider rebalancing of public and private sources of financing beneficial options for development (23). As coverage decisions are the responsibility of national decision makers, further discussion about the abovementioned suggestions could with advantage be obtained at local level with participation of stakeholders and national decision makers.

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11. Gesundheit Österreich: Proposal Comment 2007
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Annex 1: Search for positions papers on HTA

Search for position papers on HTA has been undertaken at the following websites:

- Commission of the Regions, www.cor.europa.eu/pages/HomeTemplate.aspx
- Council of European Municipalities and regions, www.ccre.org
- Council of Europe, www.coe.int
- World Health Organisation, www.who.int
- Organisation for Economic Co-operation and Development, www.oecd.org