QUESTIONNAIRE FOR ADMINISTRATIONS, ASSOCIATIONS AND OTHER ORGANISATIONS

Fields marked with * are mandatory.

INTRODUCTION

QUESTIONNAIRE FOR ADMINISTRATIONS[1], ASSOCIATIONS AND OTHER ORGANISATIONS [2]

GENERAL CONTEXT

In recent years a number of Member States have introduced so-called health technology assessments (HTA). Typically HTA measures the added value of a new technology in comparison with existing technologies. For the purpose of this survey, health technologies include, pharmaceuticals, medical devices, medical and surgical procedures and other measures for disease prevention, diagnosis or treatment used in healthcare. More information on health technologies is available at http://ec.europa.eu/health/technology_assessment/policy/index_en.htm.

HTA is a very useful tool, as it helps Member States to decide which health technology to favour at national/regional level. It also helps Member States to keep their health budgets under control, as products with no or limited added value cannot expect to be reimbursed or to obtain high prices. Last but not least HTA encourages industry to invest in innovation with substantial added benefits for patients.

Traditionally two types of assessments have been distinguished, namely (1) assessments focusing on clinical/medical benefits of the new technology (does a given technology work better than an exisiting one) and (2) assessments focusing on the economic benefits of the new technology (value for money). These assessments can be carried out jointly or consecutively, by dedicated HTA bodies or other organisations (e.g. regulators for pharmaceuticals).

At this stage, the vast majority of HTA are carried at national/regional level, i.e. EU Member States assess the new technology according to its national legislation. This leads to duplications of efforts for Member States and industry which translate in unnecessary costs throughout the HTA process. It can also lead to diverging results/outcomes (i.e. health technologies available earlier in some countries compared with others), which in turn can result in limited business predictability for industry and delayed access for patients.

Several projects funded by the EU have allowed Member States to share best practices on how HTA is carried out at national and/or regional and local level. Also a limited number of joint HTA reports have been prepared, but the use of these results is still decided at national level. In practice this has meant that the joint reports have not (yet) been used on a large scale.

There is consensus that HTA requires significant scientific, technical and economic expertise, and is costly. Currently not all Member States have such expertise at their disposal. Budget constraints also mean that even advanced Member States considered to be more advanced in this field cannot asess all new technologies. This has triggered the question whether there is a need to strengthen EU cooperation for HTA, in particular for the period beyond 2020 when the current financing of EU cooperation ends (so-called EUnetHTA Joint Action 3[3]).

For further details please refer to the Inception Impact Assessment on strengthening EU cooperation on Health Technology Assessment (HTA)[4].

OBJECTIVE OF THE CURRENT SURVEY

The aim of this public consultation is to gather detailed views and opinions regarding the future of the EU cooperation on HTA. The results of this public consultation will feed into the envisaged impact assessment which the Commission services are currently preparing on strengthening the EU cooperation on HTA.

This questionnaire is addressed to administrations, associations and other organisations. Citizens are asked to fill in a separate non-specialised questionnaire.

[1] For the purpose of this survey, administrations refer to both public administrations, as well as private administrations with public service obligation

[2] For the purpose of this survey, associations and other organisations refer to trade associations, professional associations, academia and scientific societies and organisations representing the interests of specific stakeholders

[3] European Network for Health Technology Assessment (EUnetHTA) is a Joint Action, co –funded by the Health Programme of the European Commissions (DG SANCO) and participating organisations. It gathers mainly national and regional HTA bodies. Its scope of activities is on scientific and technical issues. www.EUnetHTA.eu

[4] http://ec.europa.eu/smart-regulation/roadmaps/docs
/2016_sante_144_health_technology_assessments_en.pdf

1. INFORMATION ABOUT THE RESPONDENT

Please provide the following data on your organisation/association/administration:

*1.1. Please indicate the name of your organisation/association/administration

LOMBARDY REGION HEALTHCARE DIRECTORATE

*1.2. Please enter the country where your organisation/association/administration is based

ITALY

1.3. Please indicate whether your organisation/association/administration is listed in the Transparency Register?

No

* In the interest of transparency, organisations and associations have been invited to provide the public with relevant information about themselves by registering in Transparency Register and subscribing to its Code of Conduct. If the organisation or association is not registered, the submission will be published separately from the registered organisations/associations.

*1.4. Please enter your e-mail address (this data will not be made public).

michele_tringali@regione.lombardia.it

*1.5. The name of a contact person (please note that the name will not be made public and is meant for follow-up clarification only)

Michele Tringali

*1.6. Do you consent to the Commission publishing your replies?

- (●) a) Yes (*On behalf of my organisation/association/administration I consent to the publication of our replies and any other information provided, and declare that none of it is subject to copyright restrictions that prevent publication*)
- ( ) b) Yes, only anonymously (*The replies of my organisation/association/administration can be published, but not any information identifying it as respondent*)
- ( ) c) No (*The replies provided by my of my organisation/association/administration will not be published but may be used internally within the Commission. Note that even if this option is chosen, your contribution may still be subject to 'access to documents' requests.*)*

* As set out in Regulation (EC) No 1049/2001, any EU citizen, natural, or legal person has a right of access to documents of the EU institutions, including those which they receive, subject to the principles, conditions and limits defined in this Regulation.

2. IDENTIFICATION OF RESPONDENT

*2.1. Main field of work of the responding organisation/association/administration (*one answer possible*):

- (●) a) Public administration (other than payers)
- ( ) b) Patients and consumers
- ( ) c) Healthcare provider
- ( ) d) Payer (irrespective of status i.e. public or private)
- ( ) e) Industry or service provider
- ( ) f) Academia or scientific society
- ( ) g) Other

*2.1.a.Please specify the type of administration (one or more answers possible):

- [x] a) HTA body
- [ ] b) Marketing authorisation body
- [ ] c) Pricing and reimbursement body
- [ ] d) Ministry
- [ ] e) Other

** Small and medium-sized enterprises (SMEs) are defined in the Commission Recommendation 2003 /361. The category of micro, small and medium-sized enterprises is made up of enterprises which employ fewer than 250 persons and which have an annual turnover not exceeding EUR 50 million, and/or an annual balance sheet total not exceeding EUR 43 million.*

*2.2. Please specify the geographic coverage of your organisation/association/administration (*one answer possible*):

- ( ) International/European
- ( ) National
- (●) Regional/local

*2.3. Are you an organisation/association/administration representing the interests of the stakeholders mentioned in question 2.1 (*one answer possible*):

- ( ) Yes
- (●) No

*2.4. Please specify which health technologies are of interest for your organisation/association /administration (*one or more answers possible*):

- [ ] a) Pharmaceuticals
- [x] b) Medical devices[*]
- [ ] c) Other

** "Medical device" means any instrument, apparatus, appliance, material or other article, whether used alone or in combination, including the software necessary for its proper application intended by the manufacturer to be used for human beings for the purpose of: diagnosis, prevention, monitoring, treatment or alleviation of disease; diagnosis, monitoring, treatment, alleviation of or compensation for an injury or handicap; investigation, replacement or modification of the anatomy or of a physiological process; control of conception, and which does not achieve its principal intended action in or on the human body by pharmacological, immunological or metabolic means, but which may be assisted in its function by such means (Council Directive 93/42/EEC of 14 June 1993 concerning medical devices). Please note that the current legislation has been revised and the new requirements will be published soon.*

3. STATE OF PLAY

3.1. Please indicate your opinion on the following statements:

	Strongly agree	Agree	Neither agree nor disagree	Disagree	Strongly disagree	I don't know
*a) There are differences between **HTA procedures** among EU Member States (e.g. responsibilities of authorities, including advisory vs decision-making role and product scope; prioritisation /selection of health technologies to be assessed; duration of procedures; rights/obligations of sponsors during the procedure)	◉	○	○	○	○	○

*b) There are differences between **HTA methodologies for the clinical assessment (REA [= relative effectiveness assessment])** among EU Member States (e.g. different data requirements for the submission dossier; choice of comparator; endpoints accepted; way of expressing added therapeutic value).	○	◉	○	○	○	○

*c) There are differences between **HTA methodologies for the economic assessment** among EU Member States (e.g. different approaches for economic models, budget impact and health-related outcomes; importance of local economic context).	◉	○	○	○	○	○

*3.1.a. For a) please provide concrete examples of the differences you are aware of and their effects for your organisation:

Responsibilities of authorities: a wide range of possibilities, from a fully devoluted system like in Germany to a highly centralised architecture like in France. In general the procedural clarity and the methodological robustness of MS authorities applied to the reimbursement of medical devices are not apparent. This does not help my administration in identifying good models to look at.
Prioritisation/selection of health technologies to be assessed: only in UK (NICE) it seems to be present a full fledged prioritisation dynamic. In Lombardy a prioritisation procedure was built very slowly and with shortcomings.
Duration of procedures: ample variation. In Italy and in my region there is no timeliness of HTA activities. This is also related to a very scarce amount of full time dedicated professional resources.
Rights/obligations of sponsors during the procedure: an issue too complex to be in full scope for my organisation. We neverthless do have a policy on different stakeholder's role. There not apprears to be a clear and followable path in place at EU level with respect to assessment of medical devices.
A mayor barrier is the complexity of the technical language, that prevents otherwise knowledgeable clinical experts to appreciate, to contribute and to use HTA reports.

*3.1.b. For b) please provide concrete examples of the differences you are aware of and their effects for your organisation:

Data requirements for the submission dossier: no way to easily derive some data from national websites. In Italy the few regions that have a HTA system, and the national agency itself (AGENAS) do have different formats (this is going to be fixed by a recent Ministry level iniziative with strategic document to which my organisation did contribute).
Choice of comparator: in Lombardy we always use as first comparator the "usual pattern of care" unless a specific comparator is available (a minority of cases). In general HTA agencies in EU do a good job in selecting proper comparators, we always check in MS authorities websites (mainly HAS France and NICE UK).
Endpoints accepted: no obligation to use a set of endpoints, but a better culture toward patient-oriented outcomes measured with relevant and strong endpoints is spreading in the HTA community and the clinical community alike in Italy. EUnetHTA guidelines offered a strong support in diffusing this culture.
Way of expressing added therapeutic value: in France ANSERM and HAS do a great job with Service Medical Rendu (SMR). In Germany there seems to be a lack of clearness on the subject. Again the scarcity and lack of clarity of possible models have been a barrier for my administration to advance local policies.

*3.1.c. For c) please provide concrete examples of the differences you are aware of and their effects for your organisation:

Approaches for economic models: CEA/CUA is apparently considered everywhere, but in practice only in UK and few nations. Germany approach seems not clear. In Lombardy recent local regulation specifically excluded CEA/CUA from consideration, while it listed 4 criteria for effectiveness and 3 criteria for financial issues and introduced MCDA as a way to synthetyse net benefit and overall value of a medical device with respect to used alternatives or to no alternatives.
Budget impact: very important in Lombardy and Italy in practice (sometimes with a risk to be the only real criterion considered), but it seems to be only recently considered in most EU Member States.
Health-related outcomes: only in UK and Commonwealth nations QALY is considered a valuable way to systematise and to compare levels of outcome. In central and latin EU QALY are less used, or probably never used, expect from academics.
Importance of local economic context: critical piece in the perception of Health Care Directorate leading and management team. Direct budget impact on health care expenses more and more considered.

*3.2. In your opinion, differences among EU Member States regarding HTA procedures and/or methodologies may contribute to (*one or more answers possible):*

- [ ] a) Duplication of work for your organisation
- [ ] b) Less work for your organisation
- [x] c) High costs/expenses for your organisation
- [ ] d) No influence on costs/expenses for your organisation
- [x] e) Diverging outcomes of HTA reports
- [ ] f) No influence on the outcomes of HTA reports
- [x] g) Decrease in business predictability
- [ ] h) No influence on business predictability
- [ ] i) Incentive for innovation
- [x] j) Disincentive for innovation
- [ ] k) No influence on innovation
- [ ] l) Other
- [ ] m) None of the above
- [ ] n) I don't know/No opinion

*3.3. In recent years EU-funded projects and two Joint Actions have been carried out which aimed at strengthening cooperation on HTA across the EU. Are you aware of these initiatives? (*one answer possible*):

- ○ a) Yes, I have participated in one or more of these
- ◉ b) Yes, I am aware of them, but did not participate
- ○ c) No, I am not aware

*3.3.1. In general terms do you think the **EU cooperation on HTA (e.g. projects, joint actions)** has been

- ◉ a) Useful
- ○ b) To some extent useful
- ○ c) Not useful
- ○ d) I don't know/No opinion

*3.3.1.1.Please indicate which of the following factors concerning projects and Joint Actions were relevant for your reply (*more than one answer possible*)

- ☐ a) Allowed for sharing best practices
- ☑ b) Allowed for better knowledge of procedures and methodologies in other EU Member States
- ☐ c) Allowed for savings in your organisation
- ☑ d) Contributed to building trust between organisations and professionals involved
- ☑ e) Contributed to HTA capacity building
- ☐ f) Provided access to joint work[*]
- ☐ g) Provided access to work done by other HTA bodies
- ☐ h) Provided access to expertise not available in my organisation
- ☐ i) Reduced workload for my organisation
- ☑ j) Contributed to increasing awareness and knowledge on HTA issues in my organisation
- ☐ k) Promoted involvement of patients' representatives in HTA activities
- ☐ l) Other

* *"Joint Work" refers to activities in which countries and/or organisations work together in order to prepare shared products or agreed outcomes. These may include, for example, literature reviews, structured information for rapid or full HTAs, early dialogues or scientific advice on R&D planning and study design. Joint work aims at supporting Member States in providing objective, reliable, timely, transparent, comparable and transferable information and enable an effective exchange of this information (according to HTA Network's "Strategy for EU Cooperation on Health Technology Assessment" adopted in October 2014)" (according to HTA Network's "Strategy for EU Cooperation on Health Technology Assessment" adopted in October 2014)*

*3.3.1.1.1. Please provide additional explanations and, if available, evidence supporting your answers to question 3.3.1.1. (please provide a link to supporting documents in English)

1) Radaelli et al. Implementation of EUnetHTA core Model® in Lombardia: the VTS framework. Int J Technol Assess Health Care. 2014 Jan;30(1):105-12. PMID: 24451150.
2) information in Italian language only in the regional HTA website: https://htadm-lombardia.ats-pavia.it/

3.3.1.1.2. Please indicate to the best of your knowledge to which degree **joint work from EU-funded projects or Joint Actions was used by HTA bodies at national/regional level** as part of their decision-making process:

	To a great extent	To a limited extent	Not used	I don't know
*a) Joint tools (templates, databases, etc)	◉	○	○	○
*b) Guidelines (e.g. for clinical and /or economic evaluations)	○	◉	○	○
c) Early dialogues	○	○	◉	○
*d) Joint reports on clinical assessments (REA)	◉	○	○	○
*e) Joint full HTA (clinical and economic assessment)	○	○	○	◉
f) Other (please specify below)	○	○	○	○

* Early Dialogue (ED or early scientific advice) aims to provide prospective, transparent and timely advice by regulators or HTA body/bodies (multi-HTA) or both (parallel) to product' sponsors so that they may integrate their specific needs in the product development and generate evidence appropriate for HTA purposes (definition proposed by the EU-funded study SEED)

*3.3.1.1.3. Please indicate which shortcomings – if any - you identified in the EU-funded projects and/or Joint Actions

```
The IT interface of the EUnetHTA projects and JA is cumbersome.
The format of REA and full HTA reports does require elaboration in order to
extract usable information for local HTA projects. A fully searchable
(semantically enriched e.g. through author-based or reviewers-based subject
assignement, possibly MeSH subjects, could help).
```

4. EU COOPERATION ON HTA BEYOND 2020

*4.1. In your opinion is there a need to continue EU cooperation on HTA after 2020 (when the EUnetHTA Joint Action 3 will end)?

- (●) a) Yes
- ( ) b) No
- ( ) c) I don't know / No opinion

*4.1.a. If yes, please specify:

```
An EU harmonisation initiative securing a stronger legal framework for HTA
cooperation by MS could help to streamline a set of interoperable technology
assessment national systems. This could ensure a better functioning of the
internal market of health technologies and could contribute to a high level
of human health protection only if the process and results requirements of
the two informational processes (EU cooperative assessment and then national
appraisal) could be properly aligned.
```

4.1.1. In your opinion, for which health technologies an EU cooperation on HTA would be more useful and respond to your needs?

	Very useful	To some extent useful	Not useful	I don't know
*a) Pharmaceuticals	(●)	( )	( )	( )
*b) Medical devices	( )	(●)	( )	( )
c) Other (please specify below)	( )	(●)	( )	( )

*4.1.1.c. Please specify 'Other':

```
Assessment of e-Health tools.
Assessment of health-related apps for smartphones and tablets.
```

4.1.1.2. For which activities and if so to which degree do you consider that continuing EU cooperation on HTA beyond 2020 would respond to your needs?

	Responds very much to your needs	Responds to some extent to your needs	Does not respond to your needs	I don't know / No opinion
*a) Joint tools (templates, databases, etc)	◉	○	○	○
*b) Guidelines (e.g. for clinical or economic evaluations)	◉	○	○	○
*c) Early dialogues	○	○	◉	○
*d) Joint clinical assessment (REA)	◉	○	○	○
*e) Joint full HTA (clinical and economic assessment)	○	◉	○	○
f) Other (please specify below)	○	○	○	○

*4.1.1.2.1. Please comment on the potential advantages and disadvantages of an EU initiative including the activities you consider useful for your organisation (e.g. workload, long-term sustainability of national healthcare systems, patients' accessibility to new technologies, business predictability, innovation)

HT assessment is a technical process that summarises information of clinical (universal: scope for EU collaboration) and contextual (local: scope for national action) nature and that can inform a subsequent appraisal (a decision process) to identify the relative benefit (added value) of health technologies.
Only HTA-informed investment, disinvestment and procurement decisions, and not HTA technical reports itself, can maintain an incentive for innovation by rewarding only health technologies that carry an evidence base of high benefit.
Only HTA-informed decisions, and not HTA technical reports itself, can also ensure enough business predictability, can reduce the risk of misallocation of resources and delays of access, and can help reduce the health inequalities.
An EU harmonisation initiative securing a stronger legal framework for HTA cooperation by MS could help to streamline a set of interoperable technology assessment national systems.
This could support MS actions toward a better functioning of the internal market of health technologies and could contribute to a high level of human health protection when and if the two informational processes (EU cooperative assessment and then national appraisal) would be properly aligned.
It is therefor important to strengthen the methodological bases for HTA information that could be usable by design within many different appraisal systems.

*4.1.1.3. In case EU cooperation on HTA will continue beyond 2020, in your opinion, what type of financing system should be envisaged? (*one possible answer*):

- a) EU budget
- b) Member States
- c) Industry fees
- d) A mix of A to C
- e) Other

*4.1.1.3.e. Please specify 'Other':

A mix of EU budget, industry fees and Member States budget:
a) HTA report production should be sustained by public procurement to Member States centers of competence through a joint effort by EU Commission and MS themselves.
b) Sustainability fees from industry should be reserved:
- to capacity building of those MS agencies that are clearly committed to full cooperation on clinical matters (REA at EU level) and to dissemination of results;
- to the commission of impact assessment reports by third parties (ideally not the same HTA agencies, or at least not the same department in the MS agency).

*4.1.1.3.1. Please explain your answer and comment on issues such as feasibility, advantages and disadvantages

2000 character(s) maximum

Option 4 (voluntary or mandatory cooperation on clinical matters only, specifically in the production of joint REA reports, and then mandatory uptake of REA at MS level, where the adding of further contextual matters would not need a EU-wide cooperation) seems to offer the best composition of factors.
MS authorities and insurance entities could leverage the efforts of an independent EU-wide (centralised or distributed) HTA system by adding contextual information to properly selected reusable Element Cards (from EUnetHTA models), extracted by agencies themselves from health technology benefits reports (both REAs and Full HTA reports when available).
While the process requirement of independence does not apply, by design, to the appraisal of health technologies by MS authorities, the result requirement of public availability of properly prepared decision reports by MS is important.
The mixed nature of these integrated reports could offer to customers both the high quality of science-oriented technical HTA reports and the high relevance of accountable decision reports oriented to equity of access, reduction of inequalities, and deontological considerations.

*4.1.1.4. In case EU cooperation on HTA will continue beyond 2020, in your opinion, the secretarial /organisation support should be ensured by (*one or more answers are possible*)

- [ ] a) European Commission
- [ ] b) Existing EU agency(ies)
- [x] c) New EU agency
- [ ] d) Member States HTA bodies on rotational basis
- [ ] e) Other

*4.1.1.4.1. Please explain your answer(s) and comment on issues such as feasibility, advantages and disadvantages

2000 character(s) maximum

Assigning HTA for medical devices to EMA could give too much power to EMA itself.
In a world where technologies are converging but do still require careful consideration to the diverse nature of their life cycles, balancing powers and competencies between EMA dna a new (dedicated to medical devices) agency could be a better option.
HTA agencies of MS could be incorporated into a new EU agency as business units, likewise MS drugs agencies are now with respect to EMA set of procedures.

4.1.1.5. In your opinion, regarding an initiative on EU cooperation on HTA beyond 2020, which type of cooperation would respond to your needs? Please rank the following options from the most to the least preferable option).

	a) Most preferred option	b)	c)	d)	e) Least preferred option
*a) Voluntary participation with voluntary uptake of joint work (i.e. as carried out by EUnetHTA Joint Actions)	○	○	◉	○	○
*b) Voluntary participation with mandatory uptake of joint work for the participants	○	◉	○	○	○
*c) Mandatory participation with mandatory uptake of joint work	○	○	○	○	◉
d) Other (please specify below)	◉	○	○	○	○

*4.1.1.5.d. Please specify 'Other':

Voluntary participation with mandatory uptake of joint work for the participants, bot limited to clinical and technical matters (domains 1-4 of EUnetHTA). Joint work on social and economic matters could be an option on a case-to-case way, but in this case full uptake (that is, uptake of extra-clinical issues)) should not be mandatory.

*4.1.1.5.1. Please explain your answer(s) and comment on issues such as feasibility, advantages and disadvantages

2000 character(s) maximum

The “Strategy for EU Cooperation on HTA” (HTA Network October 2014) reminded that any cooperation between Member States (MS) should be voluntary and that any EU initiative should respect the national competences for pricing and reimbursements decisions, as well as for the organisation and delivery of healthcare systems.

5. Any other comments. Uploading relevant documents is also possible.

2000 character(s) maximum

The appraisal of health technologies by MS authorities does require, on top of personal and population benefits, the consideration of contextual factors (financial, organisational, ethical, social and legal considerations) that are out of scope for a EU-wide technical assessment system and are a main responsibility of national health care services and local insurance bodies alike.

Please upload your file (2Mb max)

3aec7c7b-6f03-45cb-b423-68312d4372a0/Lombardy_region_on_HTA_impact_assessment_2017.pdf

Contact

SANTE-HTA@ec.europa.eu