

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 existing 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 assess 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

Prescrire

Note: This consultation response is also endorsed by the International Society of Drug Bulletins and the Medicines in Europe Forum. For more information, please visit respectively: www.isdbweb.org and <http://english.prescrire.org/en/79/549/49237/3676/ReportDetails.aspx>

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

France

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

Yes, under Association Mieux Prescrire, number 982539711698-79

* 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).

rkessler@prescrire.org

*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)

Rita Kessler

*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

** 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.*

*2.1.g. Please specify 'Other':

Non-profit organisation, made up mainly of health professionals, committed to providing independent information on drugs and therapeutic and diagnostic strategies.

*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
 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.*

*2.4.c. Please specify 'Other':

diagnostic and therapeutic strategies

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
<p>*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)</p>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

*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).

<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
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*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).

<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
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*3.1.a. For a) please provide concrete examples of the differences you are aware of and their effects for your organisation:

We observe that different evaluation procedures and subsequent recommendations about a given pharmaceutical product/medical device /diagnostic result in differences in reimbursement/coverage across various Member States. Nevertheless, such differences do not have a direct effect on our work.

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

We are aware that there are variations as to data required and endpoints considered as relevant by different HTA bodies. In our view, the assessment of a new technology - be it a pharmaceutical product, medical device, therapeutic intervention or a diagnostic - must be framed around a comparative evaluation of the new product vis-à-vis standard treatments, and based on outcomes that are relevant for the patient, namely better efficacy, increased safety and/or better convenience (improvement in the practical usability of a drug). In practical terms this would translate in an increase in survival, a decrease in morbidity, a reduction in symptoms and/or an improvement in quality of life. The fact that such a proof of therapeutic advance is not considered as a key criterion during marketing approval at regulatory agency level results far too frequently in the market entry of medicines which do not bring benefits to patients and, in some cases, even pose greater harms. At present, this needed comparative assessment is undertaken by HTA agencies, who then need to ask for additional data from manufacturers in order to be able to ascertain whether the new products are in reality superior to those already available. An upstream demand - enacted in legislation - by regulators to companies requiring them to present quality data demonstrating how their pharmaceutical product compares with available treatments during clinical studies responding to key clinical research questions, with adequate selection of comparators, would go a long way in facilitating the activities of HTA bodies, saving time and resources, and ultimately also benefiting patients and society.

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

The different methodologies for the economic assessment reflect how different Member States have different settings and different priorities thus asking different questions when assessing a new technology. Economic evaluations involve a subjective value judgement which varies from constituency to constituency. Countries might be interested in comparing a new medicine with others that are already available in their national markets, or to other non-therapeutic interventions. Such a variability should not be seen as a problem, but rather as an enriching attribute which echoes different sets of values and weights imposed by social, economic and cultural context variations.

*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
- c) High costs/expenses for your organisation
- d) No influence on costs/expenses for your organisation
- e) Diverging outcomes of HTA reports
- f) No influence on the outcomes of HTA reports
- g) Decrease in business predictability
- h) No influence on business predictability
- i) Incentive for innovation
- 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)

N.A.

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)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>
*b) Guidelines (e.g. for clinical and /or economic evaluations)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>
c) Early dialogues	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
*d) Joint reports on clinical assessments (REA)	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
*e) Joint full HTA (clinical and economic assessment)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>
f) Other (please specify below)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

* 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**

Early dialogues, or parallel EMA HTA scientific advice are presented as a means to enable pharmaceutical companies (“applicants”) to “receive simultaneous feedback from both regulators and national HTA bodies on their development plans for new medicines”. However, based on our experience in drug reviews, dialogues between the regulators and pharmaceutical companies are not necessary when the data are sufficiently robust and when clinical trials are designed to address important health needs. Moreover, a serious risk of institutional capture exists if there is no strong policy in place to avoid conflicts of interest (product team leaders directly in contact with companies without any interface, many oral explanations behind closed doors). In addition, as the content of early dialogue discussions remains strictly confidential it is impossible to draw any conclusion. Without transparency on lessons learnt and access to facts, data and rationale such opaque initiatives should not be used to build new policies and guidelines.

HTA bodies play an important role at national level as tools to improve the efficiency of health systems. They must remain independent from drug regulatory agencies, and from any influence of pharmaceutical companies. Our advice to HTA bodies would be to refuse to engage in early dialogues with the EMA and pharmaceutical companies. HTA bodies should rather require drug regulatory agencies to provide them with complete assessment reports, including comparative trials against the best proven available intervention /standard therapy, as well as any relevant data corroborating the Drug Regulatory Agency decisions. This would also include reports evaluating the names and packaging of new products (1). If HTA bodies had access to comparative data earlier on, then they would be able to quickly engage in their in-depth reviews and facilitate work-sharing schemes and exchanges among each other.

The aim of early joint scientific advice is not clear. Confidential dialogues with companies risk to be less about sharing scientific analysis and more about creating a platform that can lead to regulatory capture and enable companies to influence pricing and reimbursement decisions. HTA bodies should be aware and only set rules and requirements for their independent evaluations (as indicated in 3.1b) based on guidelines, rather than on a product by product basis. Such an approach would condition the evaluation criteria of the regulatory agencies and make them closer to the needs of patients and public health services.

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:

Yes but on a voluntary basis, based on the highest possible standards and centered around clinically relevant outcomes.

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	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
*b) Medical devices	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
c) Other (please specify below)	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>

*4.1.1.c. Please specify 'Other':

diagnostics

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)	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
*b) Guidelines (e.g. for clinical or economic evaluations)	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
*c) Early dialogues	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
*d) Joint clinical assessment (REA)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>
*e) Joint full HTA (clinical and economic assessment)	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
f) Other (please specify below)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

*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)

One of the problems that can be foreseen in a multi-country harmonization is that some HTA bodies which currently have higher methodological standards could be forced to downgrade or levelling down towards a lowest common denominator. That must be avoided.

*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 A and B

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

2000 character(s) maximum

Bearing in mind the paramount role played by HTA bodies when advising those responsible for paying for medicines on tangible therapeutic advance and reimbursement, it is vital to ensure their independence. A fee for service system would create an inherent conflict of interest between assessors and marketing authorization holders, establishing an environment of institutional capture and reciprocity, which eventually will threaten the integrity of reimbursement and pricing decisions. Therefore, we support a centralized financing system from the EU budget and/or Member States.

*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)
- 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

Since HTA bodies have expertise in benefit-risk assessment and in cost-effectiveness assessment, they are in a privileged position to act as the final gatekeepers. They can recommend that a new product that provides no therapeutic added-value compared to another safer or cheaper well-established treatment does not receive reimbursement. Such a gatekeeping function cannot be ensured by the European Medicines Agency, as it is not financially independent from pharmaceutical companies (83% of its budget comes from industry fees) and its conflict of interest policies are not sufficiently robust. We would recommend a rotating secretariat among Member States, but any common assessment should be done by a team representing the different types of expertise typical of any HTA activities and not necessarily by representatives of the national or regional agencies.

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)	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
*b) Voluntary participation with mandatory uptake of joint work for the participants	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>
*c) Mandatory participation with mandatory uptake of joint work	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>
d) Other (please specify below)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

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

2000 character(s) maximum

In our view, any participation in joint work and subsequent uptake needs to be based on a voluntary mechanism. This is fundamentally related to the subsidiarity principle, whereby decisions on reimbursement and pricing are a national responsibility. While we can agree that the exchange of best practices and sharing of methodologies can bring added value and be particularly enticing to smaller and more resource-constrained Member States, when determining the value of new health technologies, it should also be borne in mind that there are marked differences in the criteria used, cultural context and socio-economic determinants and health systems across EU countries. Cooperation relies on transferability - the extent to which HTAs done in one setting can be used in, or adapted to another setting. However, as stated in the Lancet Commission on Essential Medicines "When relying on an assessment done elsewhere to inform a local decision-making process, an HTA should include a crucial examination of the applicability of the evidence used to local conditions, societal values, and the prices offered for medicines under consideration (2)".

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

2000 character(s) maximum

Health Technology Assessment can contribute to the evidence-based for selection and reimbursement decisions related to medicines. In order for it to meet that remit, several criteria need to be met:

- European Union marketing approval needs to be based on high-quality evidentiary standards so that patients, healthcare professionals and HTA bodies are able to make informed therapeutic choices relying on rigorous comparative assessment demonstrating therapeutic added value.
- Transparency is essential: This relates to disclosure of the data used in the assessments as well as on the process, criteria and rationale for evaluation and publication of reports and recommendations. All this information must be available for review by health professionals and the public.
- Independence: HTA bodies should remain independent from the pharmaceutical industry and not be financed by industry fees. They should also be free from political pressure and other vested interests in medicines policy (2).

Bibliography:

- (1) Prescrire Editorial Staff "Drug packaging in 2015: risky industry choices and lax regulation" Prescrire Int 2016 ; 25 (172) : 159-163.
- (2) Lancet Commission Report on Essential Medicines. Available at: <http://www.thelancet.com/commissions/essential-medicines>

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