

# QUESTIONNAIRE FOR ADMINISTRATIONS, ASSOCIATIONS AND OTHER ORGANISATIONS

Fields marked with \* are mandatory.

## INTRODUCTION

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### **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](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](http://www.EUnetHTA.eu)

[4] [http://ec.europa.eu/smart-regulation/roadmaps/docs/2016\\_sante\\_144\\_health\\_technology\\_assessments\\_en.pdf](http://ec.europa.eu/smart-regulation/roadmaps/docs/2016_sante_144_health_technology_assessments_en.pdf)

# 1. INFORMATION ABOUT THE RESPONDENT

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

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

European Patients' Forum

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

Luxembourg

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

yes

\* 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).

valentina.strammiello@eu-patient.eu

\*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)

Valentina Strammiello

\*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

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\*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.*

\*2.2. 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':**

Hospital-based HTA, rehabilitation and prevention programmes, disease management programmes, organisational and supportive procedures (including surgeries) as well as ethical use of health technologies (e.g. with respect to end of life, assisted reproduction, prenatal diagnosis, health data) and non-pharmaceutical interventions (e.g. psychotherapy)







### 3. STATE OF PLAY

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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 <b>HTA procedures</b> 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 checked="" type="radio"/>	<input 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).

					
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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).

					
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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:**

Based on the feedback received by our members, we acknowledge that differences exist both in the structure of the entities conducting HTA procedures and, where this is the case, in the involvement of patients. We have selected a couple of key examples that show different approaches:

In France, the organisation in charge of the HTA procedures (Haute Autorité de Santé) is an independent public body with financial autonomy, while the setting of the medicines' price after negotiation with the pharmaceutical company, and the decision on whether a medicine will be reimbursed by the public health system (and the reimbursement rate) are decisions taken by other organisations. However, the lack of full access by patients and healthcare users' organisations to the information and data submitted by the industry in the framework of the HTA procedure remains a critical issue not only in the French system.

In fact, there appears to be a general assumption by industry and HTA bodies that patient organisations do not need full information as they are involved on an ad hoc basis. Unfortunately, this approach results in fragmented and partial information to patients, including a lack of feedback on the impact of their participation in HTA procedures.

As regards the involvement of patients and healthcare users in HTA procedures, the situation is rapidly evolving: since November 2015, the French system foresees a seat for healthcare users' representative in the HAS committees in charge for the evaluation of medicines. Since November 2016, an HAS new pilot project enables consultations with patient organisations on the evaluation of new treatments.

The Dutch Health Care Institute (ZIN) has a completely different structure: it involves patients in a limited way in the reimbursement decision-making

process regarding pharmaceuticals. This involvement does not include HTA. Patients are involved at a late stage in the decision-making process. The first opportunity for them to give their feedback on the results of the HTA is after the submission of the concept report produced by scientific committee. Patients, as one of the stakeholder groups, are asked to provide their feedback (comment round). Patients or their representatives can also be asked to comment on the concept advice, that is, the policy decision that is supported by the HTA. This means that this important stakeholder group is usually involved for the first time when the HTA is already finished and the results included in a report.

A very good case study shows the challenges patients face in the Dutch system: the debate on the reimbursement of new drugs for two rare diseases, Pompe and Fabry diseases, in the summer of 2012. This case is acknowledged as the most striking discussion in the history of the Dutch Appraisal Committee ACP and shows how the contributions of patients' organisations and healthcare providers resulted in a substantial shift in ZIN's recommendations. (Smith, Cees, Personal Reflections of a Patient Representative in an Appraisal Committee, in The Patient-Patient-Centered Outcomes Research, ISSN 1178-1653 Patient DOI 10.1007/s40271-014-0086-8).

In general, evidence to date reflects a highly-fragmented landscape as stated by EPF in the Report on the survey on Patient involvement in HTA. For further information please see "Patient Involvement in Health Technology Assessment in Europe" <http://www.eu-patient.eu/globalassets/projects/hta/hta-epf-final-report2013.pdf> ).

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

Whilst we have no direct experience from engagement in REAs, we would like to stress that there is no universally accepted definition of “added therapeutic value”. (Examples of different member states’ approach are given in the recent report to the European Parliament, “Towards a harmonised EU assessment of the added therapeutic value of medicines”, 2015). Moreover industry experiences a persistent heterogeneity of data requirements and methodologies implemented by HTA bodies (Weber, S., Jain, M., Nallagangula, T. K., Jawla, S., Rai, N., Dev, D., & Cook, N. Heterogeneity in Relative Efficacy Assessments (REA) across European HTA Bodies: Opportunity for Improving Efficiency and Speed of Access to Patients? Poster presented at ISPOR 18th Annual European Congress, 7-11 November 2015, MiCo - Milano Congressi, Milan, Italy ).

From a patient’s perspective, the divergent decisions reached by HTA bodies of different Member States, and sometimes within the same Member State (e.g. Scotland/England) on the same medicines are confusing and leave patients in a profoundly unequal situation. Patients are also not always aware of what criteria are used and who is involved, or how to engage in the process. Yet, patients’ engagement is vital both from a moral perspective, because the decisions directly impact patients’ lives and well-being, but also from a practical perspective, because a meaningful definition of “value” and “added therapeutic value” is only possible with the involvement of patients.

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

Alongside differences in institutional contexts, there are some challenges that arise when smaller Member States and those with less capacity to conduct economic evaluations rely on cost-effectiveness assessments conducted by HTA bodies from other EU Member States. (Gulácsi, L. Eur J Health Econ (2007) 8: 83. doi:10.1007/s10198-007-0046-5- The time for cost-effectiveness in the new European Union member states: the development and role of health economics and technology assessment in the mirror of the Hungarian experience). Other main paradigms for HTA that we are aware of are the so called Qualitative assessment and Balanced assessment (Dankó D. Health technology assessment in middle-income countries: recommendations for a balanced assessment system. Journal of Market Access & Health Policy. 2014;2:10.3402/jmahp.v2.23181. doi: 10.3402/jmahp.v2.23181.). These dimensions are used in several EU Member States.

\*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

\*3.2.1. Please specify if 'Other':

Differences among EU Member States regarding HTA procedures and/or methodologies may contribute to inequalities in access for patients and an unpredictable environment for meaningful patient engagement.

\*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.I. Please specify 'Other':

Despite collaboration at EU level, HTA has remained predominantly a domain for national HTA bodies which are less accessible to EU-level organisations. EUnetHTA joint actions have facilitated a more direct interaction with national bodies and their experts and enabled better understanding of the existing different approaches to HTA. In addition, HTA multi-stakeholder meetings organised in the framework of EU projects, have been useful to make a mapping exercise of stakeholder groups and their interests. EUnetHTA and the SEED project provided also an opportunity for patient involvement in 11 Early Dialogues. To our knowledge EUnetHTA has not led to improved patient involvement at national level.

**\*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)**

These views are based on EPF direct experience and involvement as patient and consumer representative in the EUnetHTA JA2 Stakeholder Forum: <http://www.eunetha.eu/eunetha-ja2-stakeholder-forum>

EUnetHTA Final Technical Report-Executive Summary: [http://www.eunetha.eu/sites/default/files/sites/5026.fedimbo.belgium.be/files/EUnetHTAJA2\\_FinalTechnicalReport\\_Executive%20Summary\\_May2016.pdf](http://www.eunetha.eu/sites/default/files/sites/5026.fedimbo.belgium.be/files/EUnetHTAJA2_FinalTechnicalReport_Executive%20Summary_May2016.pdf)

**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
<b>*a)</b> Joint tools (templates, databases, etc)	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
<b>*b)</b> Guidelines (e.g. for clinical and /or economic evaluations)	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
<b>*c)</b> Early dialogues*	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
<b>*d)</b> Joint reports on clinical assessments (REA)	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
<b>*e)</b> Joint full HTA (clinical and economic assessment)	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input 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**

Although the Joint Action on HTA has been working successfully, very few results translate into a real impact on the society and Member States at large. Often, the results show significant achievements in terms of improved dialogue among Member States, but they remain accessible only to the institutions directly involved in the Joint Action. This is because results are usually not taken up at national level, “translated” and communicated to the wider audience.

To our knowledge, some partners of EUnetHTA JA2 contributed to the production of the project outputs, and meanwhile re-did the whole exercise at national level, duplicating the effort and work investment. This undermines the added value of the joint work.

In addition, there is perception of persistent lack of trust between HTA bodies and a major challenge is for project partners to overcome their cultural and structural differences.

**\*3.3.1.2. Please indicate which of the following factors concerning projects and Joint Actions were relevant for your reply (*more than one answer possible*)**

- a) Provided for limited trust between organisations involved
- b) Provided limited added value for HTA priorities in my organisation
- c) There was a degree of uncertainty about the quality of the joint work
- d) Economic assessments cannot be carried out jointly due to specific socio-economic factors in each country
- e) Increased workload for my organisation
- f) Joint work is not recognised within Member States
- g) Accessing joint work and/or work done by other HTA bodies was difficult
- h) Joint work is not relevant for my organisation
- i) Other

**\*3.3.1.2.1. Please provide additional explanations and, if available, evidence supporting your answers to question 3.3.1. (*free text field, possibility to upload supporting documents in English.*)**

n/a

## 4. EU COOPERATION ON HTA BEYOND 2020

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\*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:

EU health systems face increasing expenditure that is not sustainable. At the same time, unacceptable disparities in patients' access to innovative technologies persist across the EU. We believe EU collaboration on HTA can improve the standards of HTA assessments and, through joint work, avoid inequalities caused by the so called "postcode lottery", where access to better healthcare depends on the place of residence. This will, ultimately, enhance equitable access to care for all.

We believe further EU collaboration should be able to integrate local and regional aspects to ensure procedures remain fair and acceptable in all Member States. Unfortunately, the current divergent assessments made by national/regional bodies are confusing and frustrating for patients, who often do not know on what criteria the decisions are taken.

The importance of incorporating the patient perspective in HTA is increasingly recognised, and the integration of patient-reported and patient-relevant outcome measures (PROMs) including quality of life, in HTA is considered necessary in order to arrive at an accurate assessment of a technology's added value. But in practice, patient involvement in HTA is still very limited, and so far there is no agreement on the best method of involving patients.

In our view, the value of an innovative medicine for patients needs to be always at the heart of HTA. The patient experience may be difficult to capture fully in formal (quantitative) measures, and therefore qualitative evidence also needs to be integrated. Taking as starting points initiatives such as the HTAi Patient and Citizens' Sub-Group and existing best practices in HTA agencies across the world, appropriate methodologies and structures can be developed.

EPF believes that a common understanding is needed on the concepts of "innovation", "value" and "added therapeutic value", with patients' views at the centre of this understanding. It needs to incorporate patients' perceptions of quality of life, patient-relevant clinical endpoints, and patients' views on benefits and risks. Patients should be recognised as the most important stakeholder group in health technologies' value assessment. Frameworks, structures and methodologies should be developed for meaningfully incorporating patient evidence at all stages, from early dialogue to Health Technology Assessments, relative effectiveness assessments, and pricing and reimbursement decisions taken at national level.



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

\*4.1.1.c. Please specify 'Other':

Patients' needs go beyond medicines and include non-pharmacological therapeutic options, social and community services and peer support. Innovation should be encouraged in this wider sense, encompassing better ways of structuring and delivering integrated health and social care; and ensuring the most effective and cost-effective therapeutic approaches are available for patients.

EU cooperation on HTA is needed also for hospital-based HTA, rehabilitation and prevention programmes, disease management programmes, organisational and supportive procedures (including surgeries) as well as ethical use of health technologies (e.g. with respect to end of life, assisted reproduction, prenatal diagnosis, health data) and, comparisons between pharmaceutical and non pharmaceutical interventions (e.g. psychotherapy / medicine in mental health)

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

EPF is a European umbrella patient organisation. From our perspective, EU-level joint work on HTA would have a positive impact on patients' timely access to new health technologies. Moreover, we believe that the definition of common standards on quality and procedural aspects would mitigate if not eliminate imbalances and inequalities in access to innovative technologies and ensure an improvement in patient involvement in HTA. So far, through our collaboration with EUnetHTA we have seen the possibility to liaise with national entities, helped influence their attitude to patient involvement in delivering their outputs.

Countries with lower GDP and less financial capacity will be able to participate in the future EU joint work. That will help to take into account the diversity, parameters, and needs of all countries in the EU.

Finally, EU cooperation on HTA should aim at influencing private and public R&D priorities to ensure that investments are directed towards actual unmet needs and areas in which research is currently missing (e.g. antibiotic therapy). Open discussions on how high unmet medical needs and research gaps can be addressed are in their infancy (e.g. IMI project Adapt Smart) but are developing in this direction.

- \*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

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

*2000 character(s) maximum*

The provision of a financial contribution to carry out the joint activities is also a way to ensure commitment, interest and accountability in delivering concrete results. In previous JAs, both public and private entities were involved in different capacities but, to our knowledge, their contribution has not always delivered results to the benefit of civil society and the public. A mixed financial contribution would ensure that resources are allocated to health technologies that are worth the investment.

The financial system should be based on fundamental principles of transparency, diversification, good governance and ethical conduct. Any ongoing and future EU cooperation on HTA should foresee and allocate funds to patients and Patient Organisations to ensure meaningful patient involvement in the joint work.

**\*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*

We believe joint work involving EU Member States should be facilitated by EU agencies, either an existing or new one, while the European Commission could have a more strategic and supporting role, content and policy-wise.

It would be pragmatic to co-host a European HTA Agency with the European Medicines Agency:

- EMA has developed a model for cooperation with Member States and other stakeholders that can be replicated in HTA assessments;
- Analyses produced by EMA to inform regulatory decisions could be re-used in Early Dialogues. ED involve regulators, HTA and other stakeholders and are already hosted by EMA;
- EMA already has gathered a wealth of knowledge within its secretariat and stakeholder groups representatives involved in consultative processes;
- Given the sense of urgency to drive EU collaboration on HTA towards joint work, EMA would provide an existing platform and avoid duplication of costs.

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

After 10 years or more of collaboration on HTA at EU level, we believe it is time for Member States to “run the extra mile” and commit to a more integrated work on HTA. Similar to the experience with the regulatory process at EU level, also HTA can be more integrated to the benefit of civil society and public health systems.

Advantages would include:

- Inclusion of patient and societal aspects in all assessments;
- a reduction in the fragmentation of information at national/local level;
- more reliable and agreed methodology and procedural rules that would ensure a structured patient involvement;
- More efficient and timely HTA process
- Reduced duplication and therefore costs of assessments for Member States;
- Mitigation of inequalities in access to high-quality healthcare in EU countries.

We do not see any disadvantage for patients but we acknowledge full HTA with mandatory uptake would bring more complexity to the healthcare systems, at least in the first instance and a transitional period would be needed. This implies considerable political will, leadership and systems’ readiness for change.

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

*2000 character(s) maximum*

This consultation is the opportunity to rethink and improve the way HTA procedures are conducted (including gathering and weighting valuable information on quality of life-QoL): clinical and non-clinical aspects are still not completely captured because of the impossibility to measure them (e.g. in neurological diseases). Research is needed to ensure that valuable patient input is reported and measured to inform assessments. HTA procedures should use protocols with tailored approaches to specific chronic conditions. Some chronic conditions have only episodic symptoms; others require a tailored approach based on the age, gender, cultural roots and religious beliefs of the patient contributing to the assessments. Therefore, mechanisms that include these variables should be included in standardised methodologies. Patients with chronic and/or life-long conditions tend to value most improvement in QoL. Indeed, secondary symptoms can be the most burdensome for the patient and, therefore, treating primary symptoms has little effect on their QoL. As a result, using improvements in these symptoms as a measure of cost-effectiveness, does not reflect the real patient experience. For example, improving the balance of a patient living with Parkinson's Disease may be less patient-relevant than treating their insomnia, which would improve productivity. This is why PROMs and patient-relevant/patient-driven clinical endpoints are essential to any clinical trial and, subsequently, HTA appraisals. However, these endpoints should be set during an early dialogue stage and included in the methodology for collating clinical trial results, and in the assessments. This could be achieved through pan-European POs that can help to provide the perspective of the wider patient community. In theory, when measuring costs, EU MS aim to consider the societal perspective, but de facto only costs and benefits of immediate relevance to the healthcare systems are considered.

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