



Strengthening of the EU cooperation on Health Technology Assessment (HTA)

Online public consultation report

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1. INTRODUCTION

HTA¹ is an important tool that helps national authorities to analyse and establish the added value of new technologies over and above existing ones. The EU has been supporting Member States in their HTA efforts for many years and fosters cooperation between HTA bodies, in particular through Joint Actions. In September 2016 the Commission launched a new initiative which addresses the question whether and how to continue HTA cooperation at EU level beyond 2020 (when the current EUnetHTA Joint Action 3 comes to an end). In this context the Commission launched an open public consultation which ran from 21 October 2016 until 13 January 2017.

The aim of this public consultation was to collect all stakeholders' views on the EU HTA cooperation, encompassing their experience with the on-going cooperation mechanisms, their specific needs and their opinion on the proposed approaches described in the Inception Impact Assessment².

This report provides an overview of the responses received, grouping them by category of stakeholder. Stakeholders' responses are published online in line with the Commission's applicable rules³.

2. THE QUESTIONNAIRES

Due to the technical nature of health technology assessment, and in order to cover all interested stakeholders, the online public consultation was carried out via two questionnaires. One questionnaire was dedicated to citizens and was made available in all EU official languages. A second one was directed to administrations (both public and private administrations with a public service obligation), economic stakeholders (in particular pharmaceutical and medical technologies⁴ industry), as well as associations and organisations representing stakeholders (e.g. patients and consumers, healthcare providers, payers⁵, industry and service providers, academia and scientific societies). A simplified version of the questionnaire dedicated to administrations, associations and organisations, tailored for SMEs was circulated via the SME Network of DG GROW. This questionnaire was also made available in all EU official languages.

The questionnaire for citizens was divided into two sections:

- Respondents' information and;

¹ Health technology assessment (HTA) is a multidisciplinary process that summarises information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner. Its aim is to inform the formulation of safe, effective, health policies that are patient focused and seek to achieve best value (Definition from EUnetHTA Joint Action)

² http://ec.europa.eu/smart-regulation/roadmaps/docs/2016_sante_144_health_technology_assessments_en.pdf

³ In accordance with paragraph 3 of the Specific Privacy Statement of this online public consultation, all the contributions received, together with the identification data of the respondent, have been published on the internet, except for those where respondents expressed explicit objection in the questionnaire.

⁴ Medical technology, or medtech, encompasses a wide range of healthcare products and is used to diagnose, monitor or treat diseases or medical conditions affecting humans. In this report medical technologies refer to medical devices and in vitro diagnostics (IVD).

⁵ For the purpose of this report, payers should be understood as insurance organisations or organisations acting on behalf of a public authority responsible for the payment of healthcare services.

- Specific questions on HTA, focusing on general awareness of HTA and national HTA systems, EU cooperation on HTA, and usefulness and need to access HTA information by patients, consumers and healthcare professionals.

The questionnaire for administrations, associations and organisations included the following sections:

- Key information about the type of organisation and stakeholders represented,
- Opinions on the current state of play and EU cooperation on HTA, and
- Opinions on EU cooperation on HTA beyond 2020.

3. THE RESPONSES

3.1. Overview of responses and statistics to the online public consultation

The online public consultation and the SME consultation gathered a total of 249 replies. Of these responses, 63 are from individuals/citizens (25%) and 186 are from administrations, economic stakeholders, associations or organisations ("non-individual respondents") (75%). Of the 186 non-individual contributions, 36 replies were received in response to the questionnaire dedicated to SMEs distributed to the SME Network in DG GROW.

Most of the respondents agreed with the publication of their answers:

- 94% of the individual respondents agreed with the publication of their contributions, however most of them (59%) consented to the publication of their input only anonymously.
- 90 % of the contributing administrations, associations or organizations consented with the publication of their input, of which a large majority (71%) also agreed with the publication of their input in a non-anonymous way.

It should be noted that 78 non-individual contributors of the administrations, organisations and associations who provided contributions (representing 53% of all contributors) stated that they are registered in the Transparency Register.

3.1.1. Replies provided by citizens (individual replies)

As regards the geographical distribution of all responses, contributions from citizens/individuals came from 21 EU Member States (62) and Switzerland (1). The highest number of replies came from citizens in Germany and Netherlands (8/MS), followed by Spain, France and Italy (6/MS), Portugal and United Kingdom (4/MS), Belgium and Sweden (3/MS) and Greece, Ireland and Poland (2/MS). Only one reply came from citizens in Austria, Bulgaria, Cyprus, Estonia, Finland, Malta, Romania and Slovakia.

Additionally, a breakdown of individual respondents by level of education (Fig. 1), work experience (Fig. 2a) and sector of employment (Fig. 2b) is presented below. These data show that the large majority of the individual respondents are well-educated, with expertise and work experience in either or both public and private sectors, in areas relevant for this consultation (e.g. healthcare sector, HTA sector, public administration, health technologies⁶

⁶ For the purpose of this report, health technologies refer to pharmaceuticals, medical technologies (including medical devices and in vitro diagnostics/IVD), as well as other technologies (e.g. medical and surgical intervention, screening programmes).

industry). In addition 78% of the respondents indicate knowing how their national HTA system is organised and 63% are aware of the current EU cooperation on HTA, confirming the contributors' interest and expertise in this field.

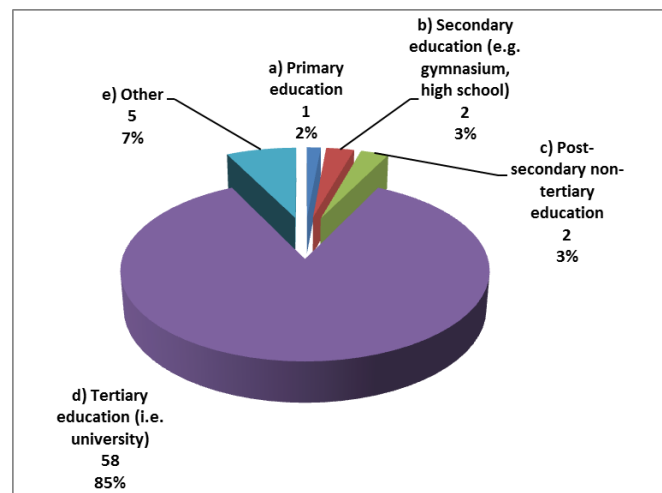


Fig. 1. Breakdown of individual respondents per level of education

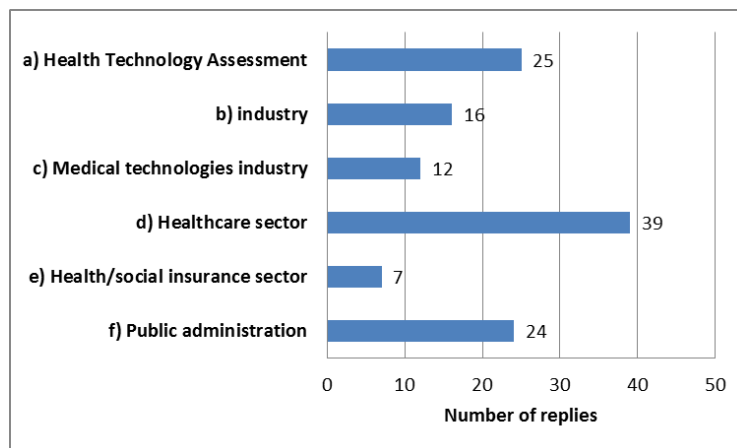


Fig. 2a. Analysis of individual respondents per type of work experience

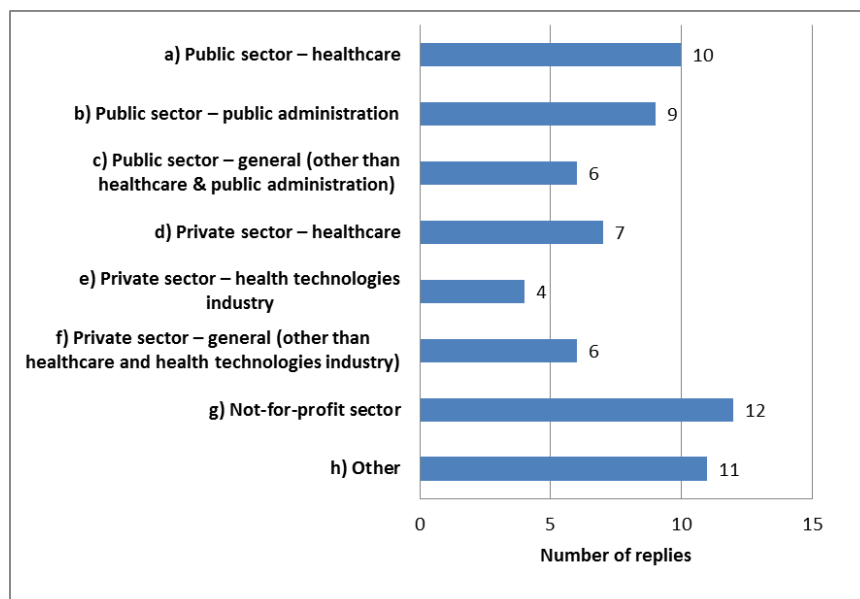


Fig. 2b. Analysis of individual respondents per type of sector of employment

3.1.2. Replies provided by administrations (both public and private administrations with public service obligation), associations and organisations representing stakeholders (non-individual replies)

As shown in Fig. 3, industry was the major contributor with 52 % of all replies, followed by public administration (14%), patients and consumers associations (13%) and healthcare providers' organisations and scientific societies (13%).

Both national and European/international organisations contributed to the online public consultation, and even organisations active at local and regional level showed interest and provided their input (Fig. 4).

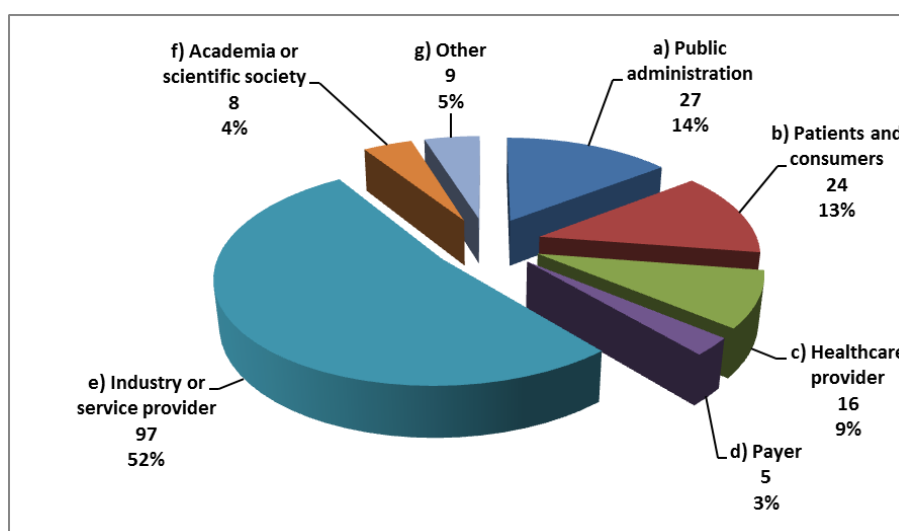


Fig. 3. Breakdown of non-individual contributions per category of respondents

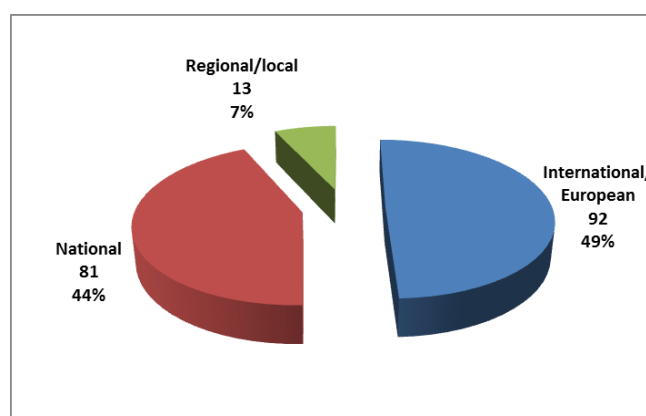


Fig. 4. Breakdown of contributions to the online public consultation based on the geographic coverage of the responding organisations

Concerning input from **industry**, most of the contributions were submitted by SMEs (46%) followed by big commercial operators (27%) and trade associations (26%) (Fig.5a). As illustrated in Fig 5b, most of the companies contributing to the public consultation are European or international companies active in more than one Member State or beyond the EU.

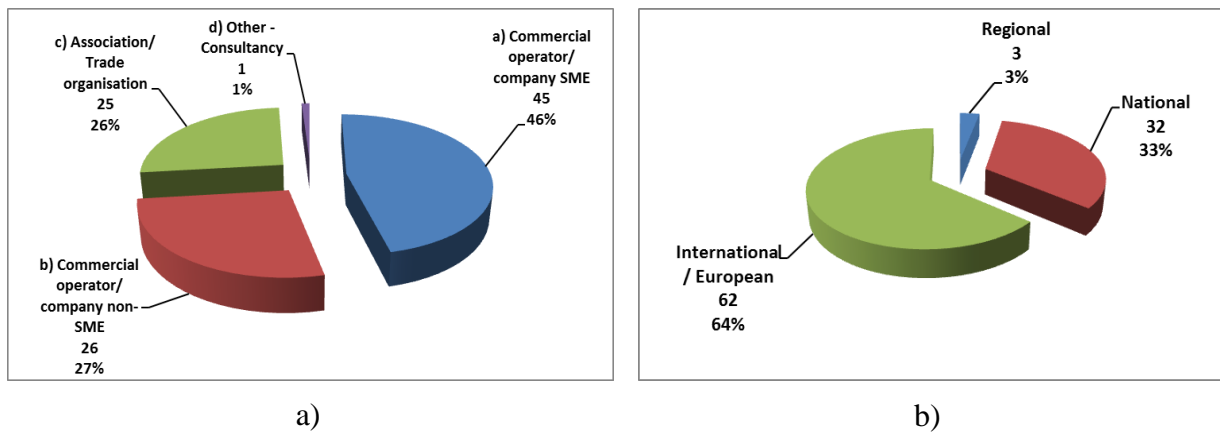


Fig. 5 a) and b). Analysis of replies from industry based on type (a) and geographic coverage (b)

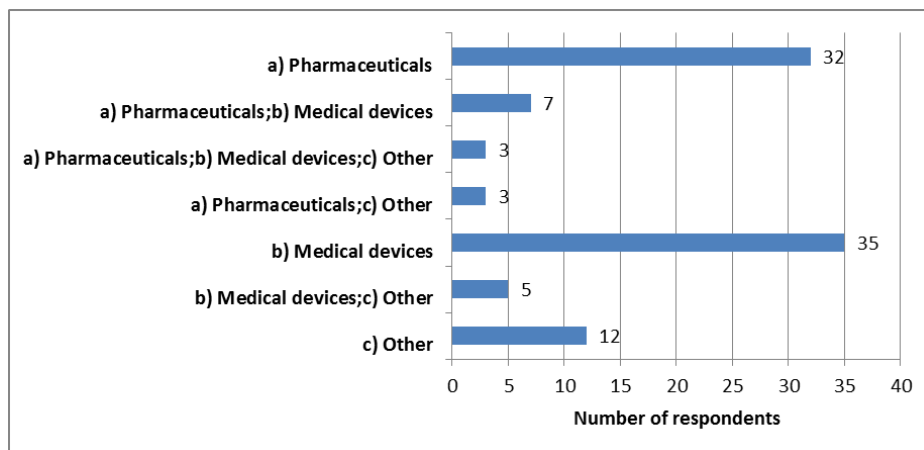


Fig. 6. Analysis of replies provided by industry per type of products

As shown in Fig. 6, a similar number of contributions were received from both pharmaceutical and medical technologies' industry. Under the "other" category, respondents indicated healthcare services, clinical and regulatory services, R&D services, diagnostics, software for medical applications, telemedicine and biotechnology.

As regards **public administrations**, most of the contributions were provided by HTA bodies, as well as organisations with multiple responsibilities, Ministries of Health, payers and other national or regional organisations (Fig. 7). Some Member States decided to submit their comments outside the public consultation, which are therefore not included in this report, but will be incorporated in the Synopsis Report.

Concerning the geographical distribution of responses from public administrations, contributions came from 15 EU Member States (Italy with 5 contributions, Germany, Finland and Spain with 3 contributions/Member State, Slovenia with 2 contributions, and Austria, Belgium, Czech Republic, Croatia, France, Hungary, Ireland, Poland, Portugal and United Kingdom with 1 contribution per country) and Norway (1 contribution).

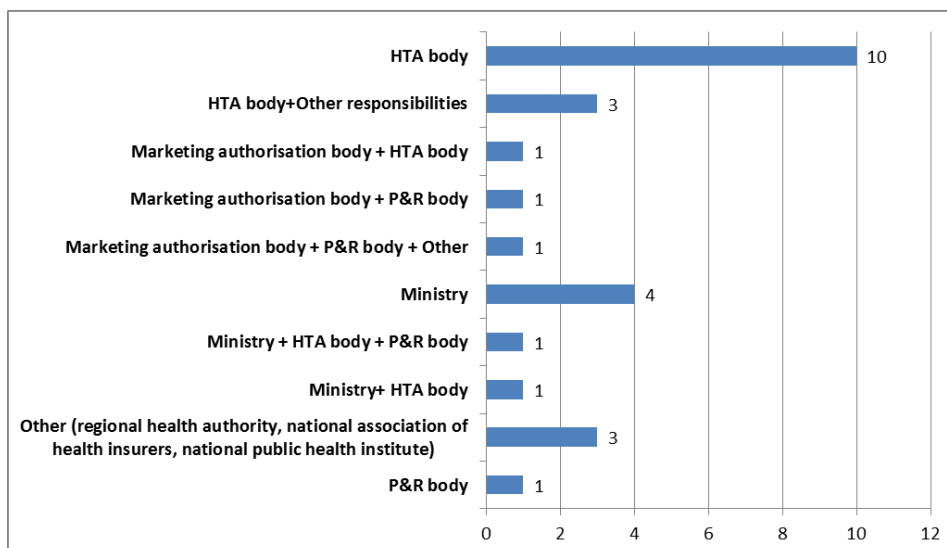


Fig. 7. Categorisation of public administration respondents

Patients and consumers were represented by an equal number of national and European patients' associations. Most of these associations (63%) acknowledged their interest for both pharmaceuticals and medical technologies. Additionally 4% of these organisations specified being interested in all health technologies.

Healthcare providers were represented in the consultation by national associations (50%), followed by European (31%) and regional organisations (19%). Fifty per cent of the respondents in this category indicated representing hospitals and the rest provided input on behalf of doctors, community pharmacists, optometrists and public health trusts.

Respondents from **academia** were mostly European organisations (63%), but also national and international ones (i.e. ISPOR). **Payers** were mostly represented by national associations (60%). The category **other** was selected by non-profit organisations promoting public health, information on pharmaceuticals and therapeutic and diagnostic strategies, improved access to medicines and their rational use, or the development of therapies in specific areas such as cancer or regenerative medicine.

3.2. Analysis of responses

3.2.1. Responses from citizens (individual replies)

3.2.1.1. Importance of HTA

Almost all respondents (98%) consider that it is useful to compare new health technologies with existing ones and assess whether they work better, equally well or worse, in order to provide guidance to decision makers.

Most of the respondents indicate that patients should have access to the best possible treatment, with the least possible cost, with HTA supporting "rational decision making and control the health care budget". Respondents also note that not all new health technologies are more effective than those already available in clinical practice. It was suggested that less effective technologies should be taken out of the market following appropriate assessments, with disinvestment in obsolete technologies potentially generating savings that could be better used in investing in truly innovative health technologies.

Regarding rare diseases, several contributors observe that for most of them there are no satisfactory health technologies available, and in such cases any new/innovative technology, even with a modest effect, will be considered as a major improvement for the patient and their families and carers. However, due to the small numbers of patients to benefit from the technology, the cost per person will be high and it is fair to ask if society is willing to pay (applying the solidarity principle). In such cases it was suggested to take into account not only the budget impact, but also whether the disease in question benefitted or not from public or private investments and the improvements in the life of the patients and their families if duly documented (patients' and social aspects).

Concerning medical technologies, some respondents deplore the lack of a European source of information including description and characteristics of the new technologies, as well as efficacy and safety aspects, which could provide key information for patients/consumers, but also for decision makers when accepting their coverage by the national insurance system.

3.2.1.2. HTA at national and/or EU level

When asked about the factors to be taken into account when carrying out health technology assessments, all the respondents confirm the need for ensuring:

- transparency of HTA processes which translates into clear HTA methodologies and an adequate involvement of all relevant stakeholders (e.g. patients, healthcare providers);
- appropriate expertise of the assessors carrying out the assessment which is essential for a high-quality report;
- independence of the assessors, which requires an appropriate mechanism to avoid conflicts of interest;
- timely delivery of the assessment thus allowing a well-timed informed decision making. A detailed overview of the replies provided to this question is presented in Figure 8.

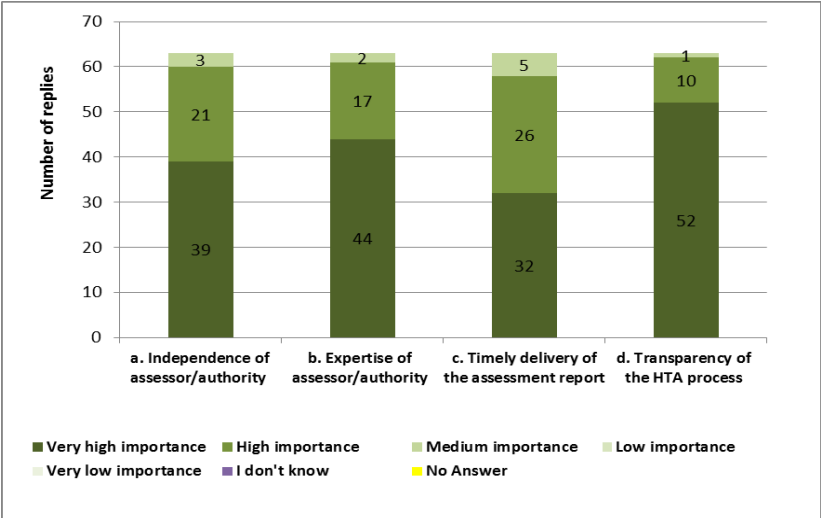


Fig. 8. Overview of the opinions of individual respondents on factors relevant when carrying out health technology assessments

When asked about clinical assessment, 57% of the respondents consider that that national/regional HTA bodies should not perform clinical/medical assessments of the same health technologies in parallel, independently from each other (Fig. 9). These respondents consider that several aspects related to clinical/medical assessments of health technologies

could be addressed by the EU cooperation on HTA (Fig. 10). Under the category other activities which could be carried out at EU level, respondents suggest an EMA-like centralised procedure for clinical/medical assessments and a mechanism of mutual recognition of national HTA reports.

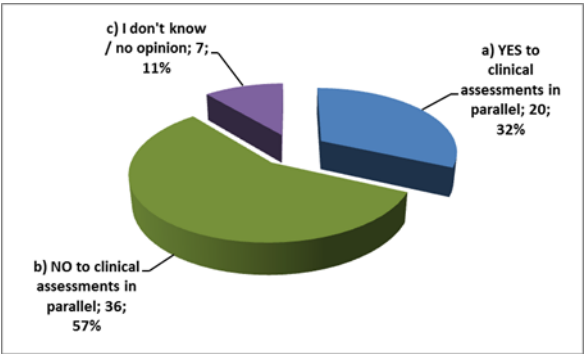


Fig. 9. Overview of the opinions of individual respondents regarding the possibility to perform **clinical/medical assessments** in parallel and independently by national and regional HTA bodies

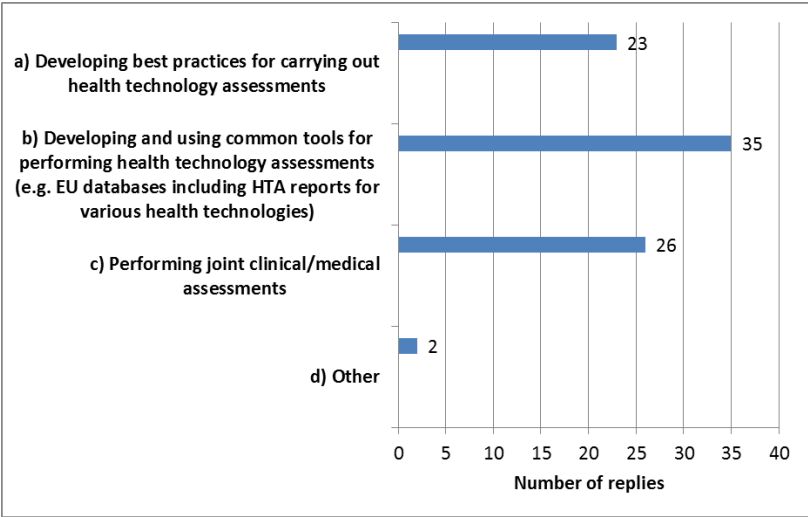


Fig. 10. Overview of the opinions of individual respondents on aspects related to **clinical/medical assessments** which could be addressed at EU level

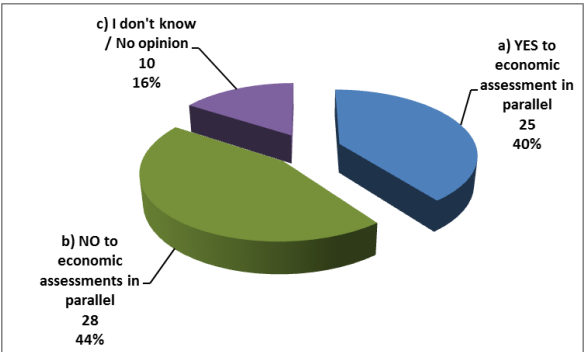


Fig. 11. Overview of the opinions of individual respondents regarding the possibility to perform **economic assessments** in parallel and independently by national and regional HTA bodies

When asked a similar question about economic assessments, 44% of the individual respondents agree that there is no need of performing economic assessments separately by national and regional HTA bodies (Fig. 11). Respondents argue that due to heterogeneity in terms of national GDP, organisation of health systems and socio-economic context, a pharmaceutical can be cost-effective in one country, but not in another. However, there were also voices who suggest that one general economic assessment could be centrally performed, followed by an adjustment in each EU MS according to their socio-economic conditions.

3.2.1.3. Access to HTA information for patients, consumers and healthcare professionals

The survey showed that most individuals (95%) believe that information on whether a new health technology works better, equally well or worse than a health technology already available in their country should be easily accessible to doctors to enable an informed decision when prescribing the treatment of their patients. Respondents consider that if easily available to doctors, HTA can help them to accurately inform their patients about the benefits of the new treatments compared to the current standard. In addition it was considered that information from HTA bodies/public sources could provide unbiased and independent information in addition to the guidance offered by the manufacturers, and should be taken into account when developing therapeutic protocols.

In the same way, most respondents (84%) consider that information on whether a new health technology works better, equally well or worse than a health technology already available in your country should be easily accessible to patients and patients' representatives. Several respondents pointed out that trust and transparency are essential and patients should be explained how different decisions within or across Member States are taken. Therefore authorities should help patients to understand how health technologies are evaluated and the rationale behind the reimbursement/coverage decision. It was emphasised that "without this effort, patients will continue to mistrust the process and think HTA is more a gatekeeper that prevents them from accessing new health technologies than a process to help determining which health technologies add value to treatment and care".

In addition, the contribution of patients' organisations to the work of authorities performing HTA is considered very important and important by 67% and 27% of the respondents respectively. It was highlighted that participation of patients' organisations can provide good understanding of the patients' point of view, especially on topics such as unmet needs, quality of life data, patients' preferences regarding their treatments, acceptance of side effects or therapeutic adherence. Respondents call on national and regional HTA bodies to start involving or involve more the relevant stakeholders and representatives of the public (e.g. patients' advocate, health care professionals) when preparing and/or reviewing their HTA reports and to make public their input. It was advocated that patients' involvement could improve credibility, fairness and equity to both the HTA process and the decisions based on the HTA.

3.2.2. Responses from administrations, associations and organisations representing stakeholders (non-individual replies)

3.2.2.1. HTA across EU - Opinions on the current state of play

The first section of the questionnaire addressed to administrations, associations and organisations aimed to verify whether the issues identified in the Inception Impact Assessment published by the Commission in September 2016 are shared by stakeholders. The main issues mentioned in the Inception Impact Assessment are: differences in HTA

procedures and methodologies among EU Member States, the (inadequate) level of uptake of joint work and the duplication of work for both authorities and industry.

As shown in Fig. 12, most of the respondents strongly agreed or agreed with the existence of differences in HTA processes and methodologies, from 80% on HTA methodologies for clinical assessments and 85% on HTA methodologies for economic assessments, to 91% agreement on the existence of differences in national HTA procedures.

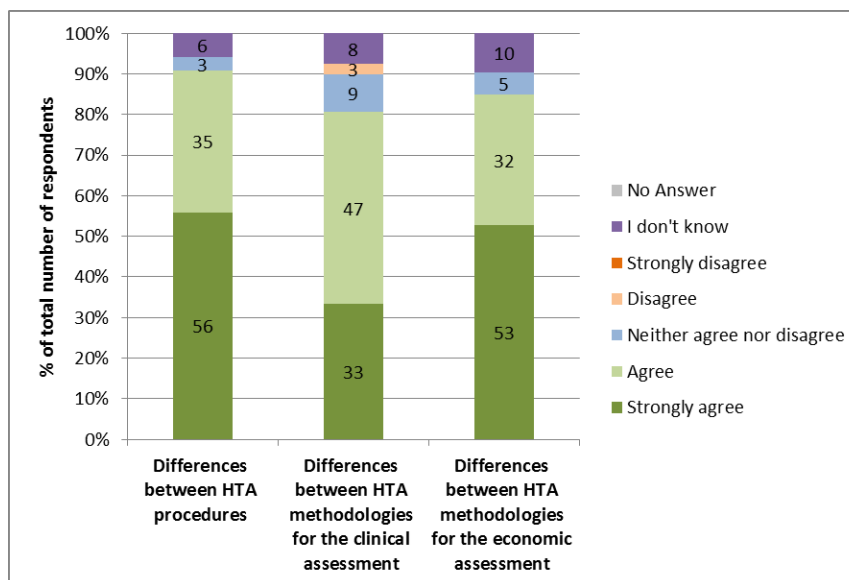


Fig. 12. Overview of the opinions expressed by administrations, organisations and associations on the existence of differences in HTA processes and methodologies across EU

- **Differences in national HTA procedures**

As regards differences in national HTA procedures, *public administrations and payers* highlight the different legal frameworks across EU with the structure, function, remit, and approaches of HTA bodies varying according to the health systems and political structures they operate in. Product scope and prioritisation of technologies to be assessed largely vary among Member States. Additionally there are differences in HTA capacity, with some Member States having more advanced HTA systems than others, and a few EU countries still in the process of building national HTA organisations/systems.

Representatives of *pharmaceutical industry* point out that, HTA procedures are very diverse across EU, and this diversity constitutes a hurdle for companies, as they have to adapt to various national requirements. It was underlined that there are differences regarding the starting moment (e.g. before or immediately after marketing authorisation is granted or at later stage) and length of the HTA procedures, scope of HTA (i.e. all vs a selection of medicinal products), type of assessment carried out on a regular basis (e.g. only relative effectiveness assessments/REA vs sequential clinical and economic assessments vs full HTA), data accepted (e.g. only published vs non-published data), opportunity for early dialogues⁷, level of engagement with stakeholders, availability of relevant documents and background information, and finally the purpose and weight of assessments (e.g. recommendation vs binding opinion for pricing and reimbursement decisions). Due to these differences, most of the big pharmaceutical companies have national affiliates who engage with national

⁷ 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).

authorities, preparing the documentation requested by HTA and pricing and reimbursement bodies. The national affiliates work mostly on the context-specific evidence (e.g. health economic impact, fit with local priorities). In contrast, smaller companies with limited resources may face difficulties when confronted with different HTA processes and requirements, which may create a discriminatory environment.

Respondents representing *medical technologies industry* note that currently HTA has limited role in market access and the role and timing of HTA for medical technologies is country specific. They deplore the lack of established HTA processes and expectations for medical technologies (i.e. HTA processes and methodologies are considered "pharma-biased", not always addressing the particularities of the medical technologies' sector), variable timelines and in some Member States disconnection between HTA outcomes and patient access to the technology assessed. Moreover, it was stressed that compulsory HTA for all medical technologies may become a market barrier with major implications on the development of new products.

Patients and consumers', as well as *healthcare professionals'* organisations and *academia* indicate the diversity of approaches across EU regarding the involvement of stakeholders in the HTA processes, their access to the information submitted by industry or to the outcome of HTA (e.g. publication of HTA report). A large majority of these organisations advocate for more involvement of patients and professionals in the HTA procedures, more transparency of HTA processes and clarification of the role of HTA in the subsequent national pricing and reimbursement steps.

- **Differences in national HTA methodologies for the clinical assessments**

With reference to the differences in national HTA methodologies for the clinical assessments (i.e. REA), stakeholders note that there are different data requirements for carrying out the assessment, and also different clinical practice approaches, including choice and acceptance of comparators (e.g. indirect comparators and off-label comparators are not always accepted), selection and acceptance of endpoints (e.g. surrogate endpoints), which ultimately may explain the different outcomes of national HTA clinical reports. In addition, there are different ways of expressing the added therapeutic value, which in some countries may be linked to the subsequent appraisal process. The level and ways of participation of interested stakeholders (e.g. patients, professionals, industry) in the clinical assessment process may also vary.

Representatives of *pharmaceutical industry* underline the areas where differences in HTA methodology for clinical assessments are evident, such as choice and acceptability of comparators and endpoints, acceptance of other data than randomised clinical trials, acceptance and interpretation of analysis of survival that adjust for trial cross-over, weighting of efficacy data versus safety and quality data. It was also pointed out that at launch HTA is largely based on the efficacy data provided to regulators (e.g. relative efficacy), with some Member States looking also into prediction of relative effectiveness to support their decisions. In relation to the appraisal step, industry organisations consider that, due to their context-specific interpretation, added therapeutic value ratings are difficult to share between jurisdictions.

While pharmaceutical companies agree that there are elements of the methodology for the clinical assessments which vary across EU, some *medical devices companies* report that since their products are rarely subject to HTA, the key differences in HTA methodology for clinical and/or economic assessments are less easy to identify. Additionally some contributors note that for some medical technologies (e.g. diagnostic imaging technologies) randomised clinical trials are cost-prohibitive or only indirectly linked to clinical outcomes, experimental studies can be more challenging due to ethical issues, and clinical performance often depends on end-

users. Therefore respondents from the medical technologies sector support the development of HTA methodology adapted to the particularities of their sector.

- **Differences in national HTA methodologies for economic assessments**

Finally, on the differences in national HTA methodologies for economic assessments, most of the stakeholders underline that these are more pronounced than those for clinical assessments. The importance of the national (regional) local socio-economic context and the need for contextual adaptation, the use of indicators such as cost/QALY or incremental cost-effectiveness ratio/ICER, and the differences in the evaluation perspective (payers or societal) are quoted among the major issues which would make difficult the formulation and acceptance of an EU joint economic assessment.

Patients and consumers organisations, as well as *providers and academia* confirm the existence of differences in national HTA economic methodologies, and emphasised the lack of transparency in some countries, which makes it hard for patients' representatives to adequately contribute to the HTA process.

Apart from these differences, some respondents point out that there are also differences in HTA methodologies for the assessment of other dimensions, such as organisation impact, ethical and legal impact, or social impact, which are also relevant to the HTA process.

Furthermore, the contributors to the public consultation confirm that differences in HTA processes and methodologies across the EU translate into: diverging outcomes of HTA reports which may affect patients' access to new technologies (e.g. delays, restricted access), duplication of work and high costs for both HTA bodies and industry, decrease in business predictability, and even affect innovation in a negative way (Fig. 13).

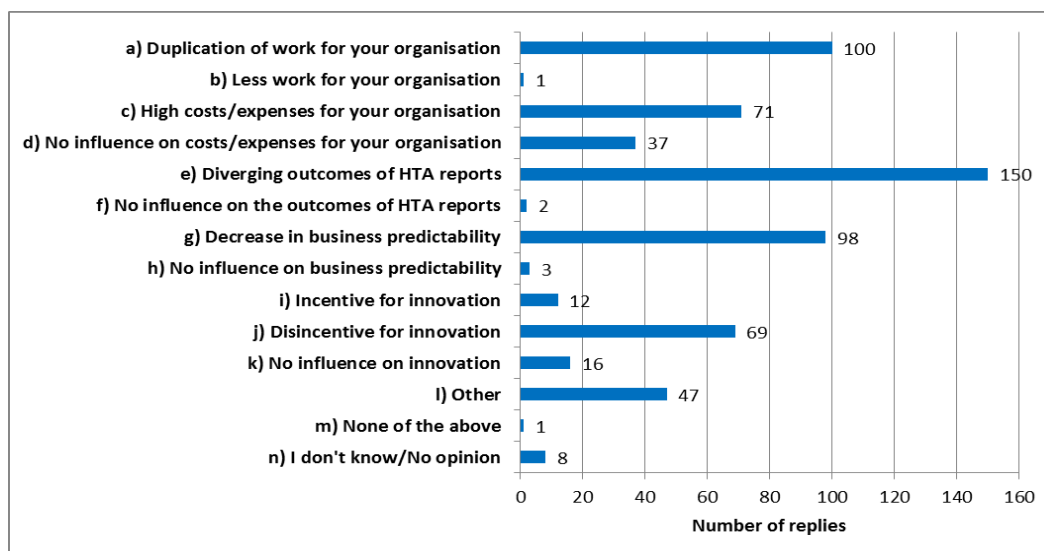


Fig. 13. Consequences of differences in HTA process and methodologies across EU as identified by public administrations, organisations and associations

3.2.2.2. Opinions on the current EU cooperation on HTA

The consultation shows that 32% of the respondents participated in EU-funded projects and joint actions. In addition 47% of the contributors state that even though they did not directly participate, they were aware of EU cooperation on HTA. **Participation to and awareness of EU-funded activities** varies among categories of respondents, as following: public administrations 70%/26%, payers 40%/60%, pharmaceutical industry 38%/56%, academia

37%/50%, patients' organisations 29%/58%, healthcare providers 25%/69% and medical technologies' industry 25%/65% (Fig. 14 and 15). The lowest level of participation and awareness was observed for SMEs; 9% of SMEs which contributed to the public consultation report participating and 31% about being informed about EU-funded activities on HTA without direct participation.

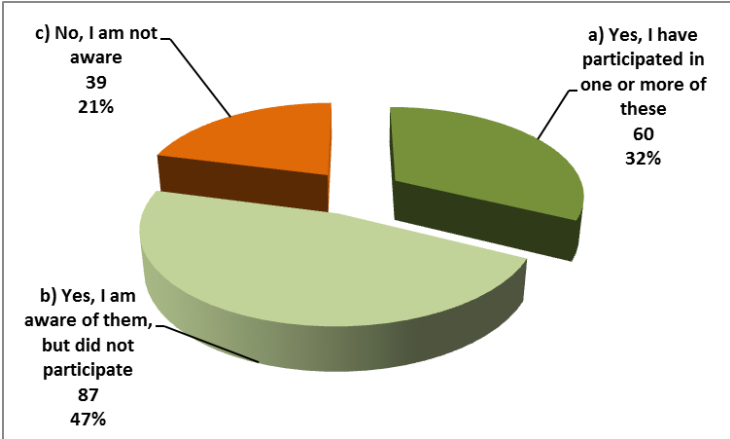


Fig.14. Awareness of EU cooperation on HTA (i.e. EU-funded projects and joint actions) - breakdown of non-individual responses

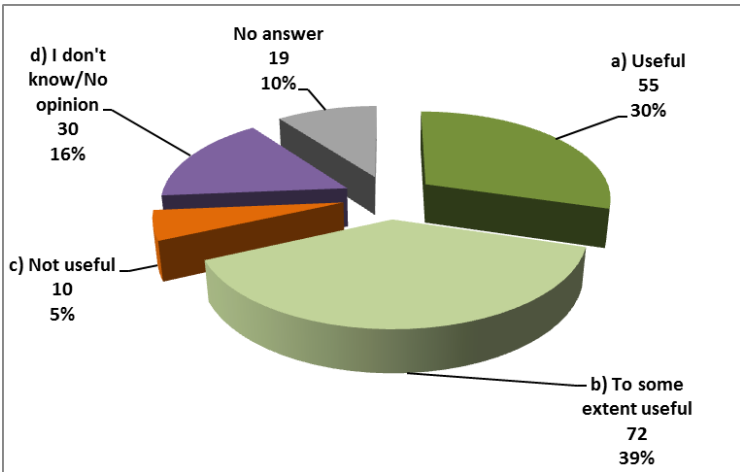


Fig.15. Usefulness of EU cooperation on HTA (i.e. EU-funded projects and joint actions) – breakdown of non-individual responses

Respondents who confirmed their participation in or awareness of EU funded activities were also asked to evaluate their **usefulness**. Most of them (69%) consider EU cooperation on HTA useful, with most benefit seen by public administrations, payers and academia (100%). A negative opinion is reported by medical technologies industry, SMEs, and a minority of respondents from the pharmaceutical industry with 28%, 22% and 4% of respondents from these three categories seeing EU cooperation not useful (Fig. 16). It has to be noted that despite the overall lack of awareness of SMEs, most of those who directly participated in EU-funded projects had a more positive opinion about their experience.

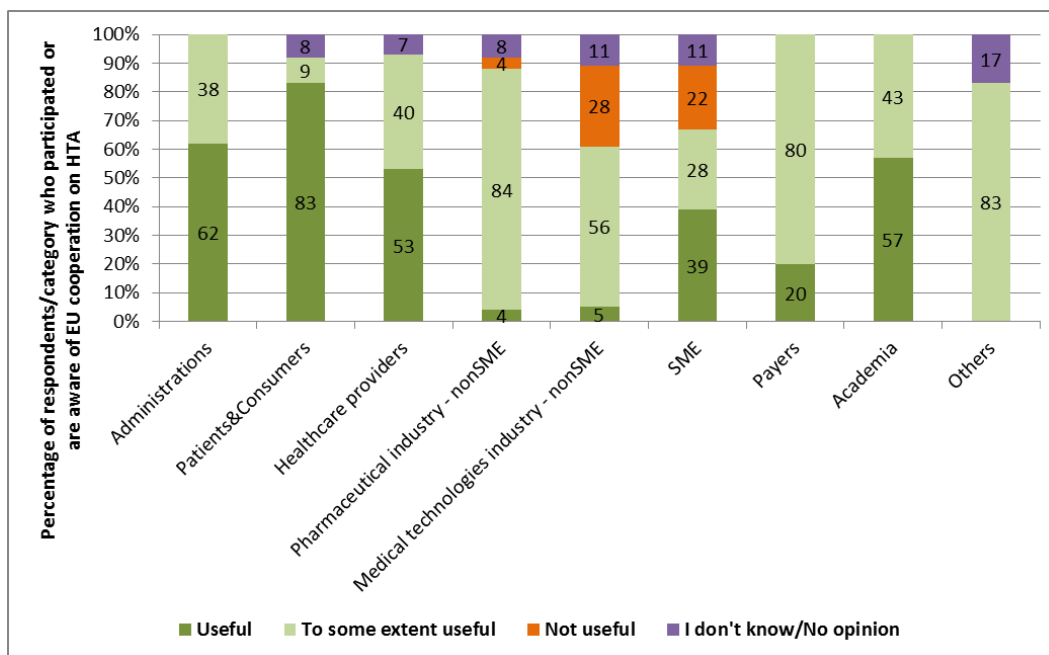


Fig.16. Usefulness of EU cooperation on HTA (i.e. EU-funded projects and joint actions) per category of respondents

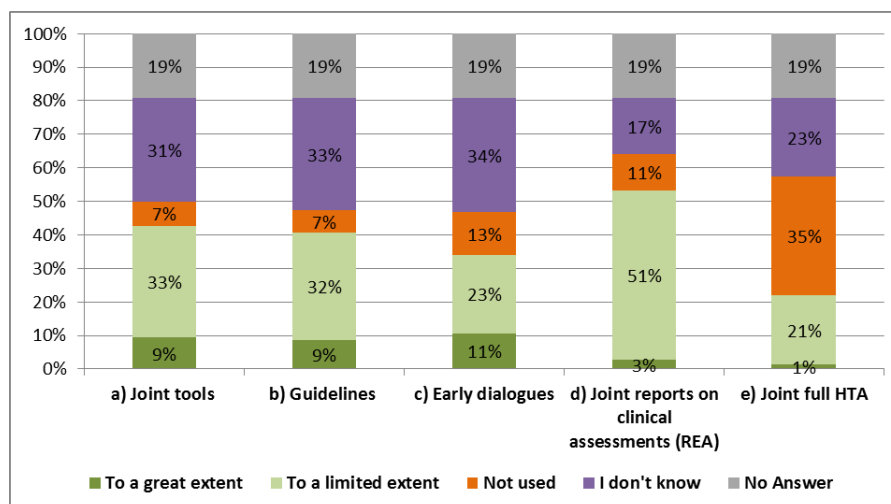
The survey enquired also about the drawbacks of the current EU cooperation on HTA. The limitations of the current type of cooperation most cited by public administrations are: the lack of flexibility of the framework for EU-funded projects which require high efforts for the preparation of a proposal, difficulties to put in place a sustainable IT platform (including IT tools) for the use of all participants and access of joint work, delays in performing joint work which affected the availability of joint reports, insufficient commitment from all partners to use the output, uncertainties about the quality of joint work, insufficient coordination and agreement on topic selection, lack of knowledge on the impact on decision-making and the limited participation of some categories of stakeholders such as health professionals and patients.

Organisations representing stakeholders other than HTA bodies note that in EU-funded projects and joint actions the discussions have been limited to HTA bodies which may have excluded expertise from other stakeholders (e.g. clinicians for assessing methodology and data, public procurement hospital-based HTA representatives, hospital-based pharmacists, payers). Scientific societies express their interest and availability to provide their expertise and become more systematically involved in activities of common interest such as early dialogues, evidence generation and harmonisation of clinical guidelines. Representatives of patients and consumers' organisations deplore their insufficient involvement in EUnetHTA Joint Actions. The limited duration in time and the lack of a sustainable funding mechanism are also mentioned.

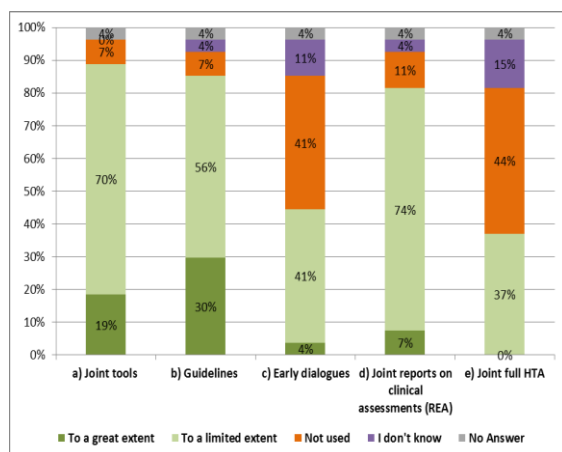
Patients' organisations note that voluntary participation from both Member States' authorities and industry may create a vicious circle, with industry hesitating to take part and HTA bodies hesitating to contribute as authors or to use joint work. They also note that Joint Actions did not address sufficiently the issue of patients' involvement in HTA and did not provide a consolidated methodology to consistently involve patients and their organisations in the health technology assessment. Additionally, it was considered that the lack of uptake of joint work even by partners who participated to the creation of the joint work undermines its added value.

Most of the respondents emphasise the importance of the local socio-economic context and differences in HTA methodologies, which decreases the possibility of producing meaningful joint (economic) assessments, and may explain their low uptake at national level.

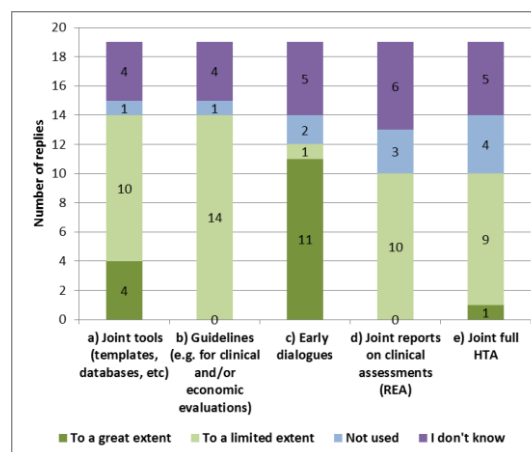
Respondents considering the current EU cooperation on HTA useful or to some extent useful (number of respondents = 121 = 81% of total number contributors to the questionnaire dedicated to administrations, organisations and associations⁸) were also asked to estimate the **uptake of joint work**⁹ from EU-funded projects or Joint Actions by national/regional HTA as part of their decision-making process. The survey shows that uptake of joint work remained low (Fig. 17a). It has to be noted that there were significant variations in the estimations provided by different category of respondents (Fig. 17b to g).



a) Overall non-individual responses



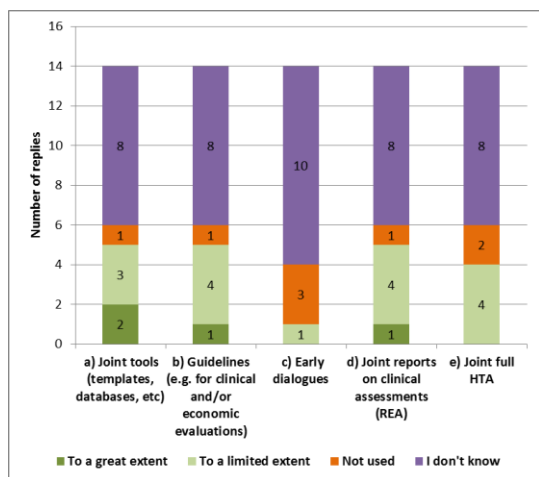
b) Public administrations



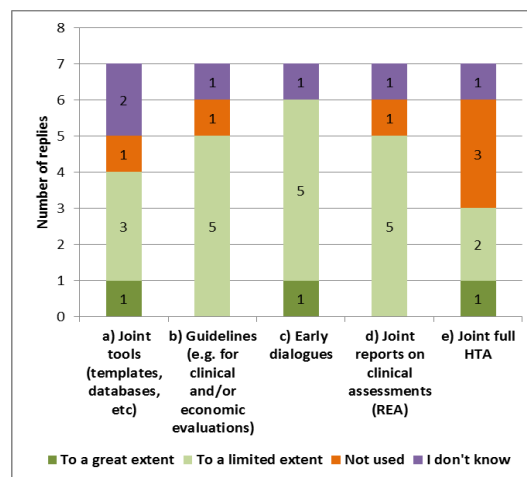
c) Patients and consumers

⁸ NB. The questionnaire circulated to the SME Network did not include this question.

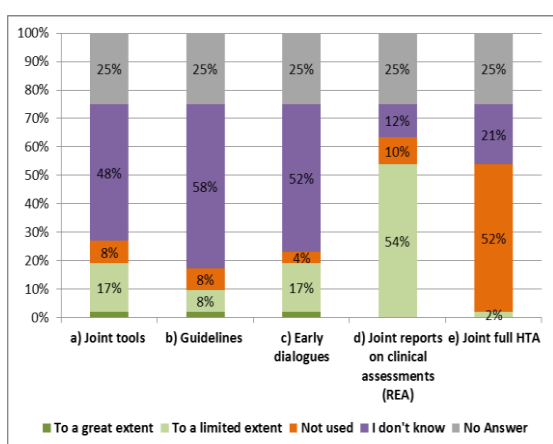
⁹ "National uptake" is defined by EUnetHTA as the general implementation of any EUnetHTA output (i.e. joint assessments, submission templates, guidelines, POP Database, HTA Core Model®, etc.) in a local (national/regional) setting.



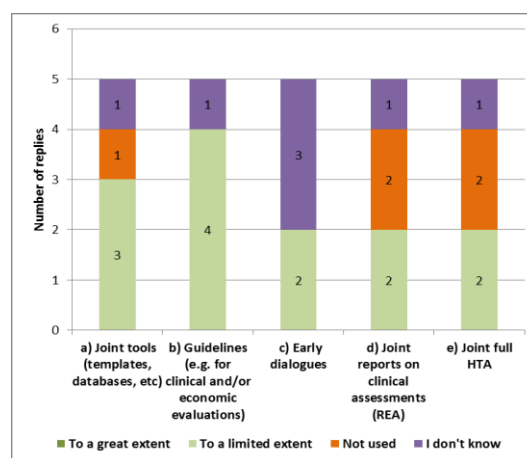
d) Healthcare providers



e) Academia



f) Industry (non-SME)



g) Payers

Fig. 17. Uptake of joint work from EU-funded projects and joint actions per type of joint activities and category of respondents: a) overall non-individual responses; b) public administrations; c) patients and consumers; d) healthcare providers; e) academia; f) industry (non-SME commercial operators); g) payers

Of the total number of non-individual respondents, 10% state that EU cooperation on HTA is not useful (Fig. 15 and 18). They justify their statement mainly by the fact that economic assessments cannot be carried out jointly due to the different socio-economic context across EU and that joint work is not recognised or appropriately used within EU Member States. Furthermore, several respondents use the "other" category to provide more details about the issues put forward in the survey. For example, it was noted that companies and especially SMEs do not have a structure or resources dedicated to HTA, and the time, complexity and demands from HTA bodies are considered too high with no significant return for their investment¹⁰. This was also stated by certain representatives of the pharmaceutical industry who note that the use of joint work by the national HTA agency rather increased the workload due to their additional more detailed requests. Medicines for Europe, the association representing the European generic, biosimilars and value added pharmaceutical industries,

¹⁰ NB. During the EU-funded SEED project which organised 11 early dialogues with health technologies' developers, 3 medical technologies' companies participated actively to joint multi-HTA early dialogues, and due to the limited budget of the project, 3 other selected companies had to be included on the waiting list.

observes that value added medicines¹¹ were not eligible to participate in the previous joint actions. Other respondents highlight the need for specific HTA methodologies for the medical technologies sector, especially for imaging equipment and IVD products, and observe that the HTA Core Model provides merely a useful starting point for preparing a context-specific report. Other respondents note that results of joint work have not been available at a time when they might have been useful.

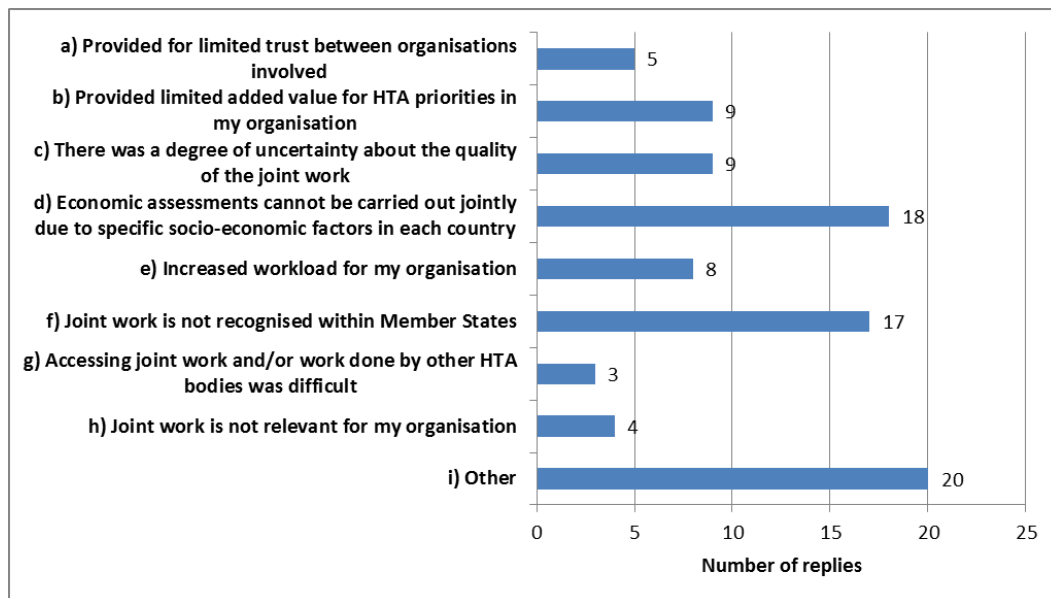


Fig. 18. Factors which were taken into account by the non-individual respondents who consider EU cooperation on HTA not useful

3.2.2.3. Opinions on continuation of EU cooperation on HTA beyond 2020

A large majority of the respondents (87%) consider that **EU cooperation on HTA should continue beyond 2020** when EUnetHTA Joint Action 3 will end (Fig. 19). Many respondents underline that EU cooperation is needed to ensure a constant exchange of information and knowledge between HTA institutions in Europe, to increase synergies between Member States, to streamline HTA methodologies, to increase transparency and evidence-based decision making, as well as business predictability. The possibility to access a larger number of HTA reports with less duplication of work and better allocation of resources by HTA bodies are also highlighted by some experienced public administrations. It is also noted by some respondents that EU cooperation can enhance access to added value and affordable technologies in a timely manner and in the long run can also lead to savings, improving resilience and contributing to the sustainability of health systems. Horizon scanning performed at EU level is seen as a joint activity which could support national healthcare systems to better allocate resources and ensure sustainability. There were also voices who advocate for a legal framework for EU cooperation on HTA to streamline interoperability of HTA national systems, thus improving the internal market of health technologies. Finally, several stakeholders note that significant public resources have been invested in EU cooperation on HTA and all the results achieved so far should be capitalised to support sustainable healthcare systems and guarantee equitable access to technologies with added value to all patients in Europe.

¹¹ Value added medicines is a concept introduced by Medicines for Europe and refers to medicines based on known molecules that address healthcare needs and deliver relevant improvement for patients, healthcare professionals and/or payers.

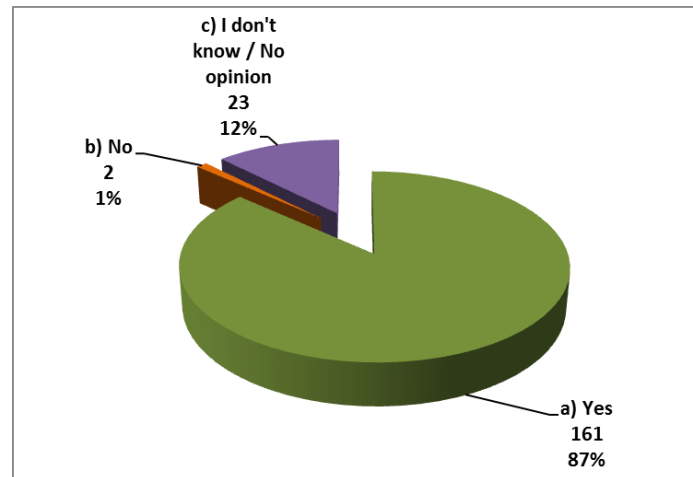


Fig. 19. Breakdown of responses regarding the need to continue EU cooperation on HTA beyond 2020 when the EUnetHTA Joint Action 3 ends (Total number of respondents = 186)

The highest number of the respondents with no opinion on the continuation of EU cooperation on HTA are SMEs (15 of the total of the 23 negative replies expressed by all contributors, and representing 33% of the contributions from SMEs), which correlates with the high number of respondents in this category who are not aware of the current EU cooperation on HTA.

As regards the **scope**, a large majority of the respondents found useful and to some extent useful to continue EU cooperation on HTA in the field of pharmaceuticals (80%), but also in the areas of medical technologies (72%) and other technologies (54%) (Fig.20).

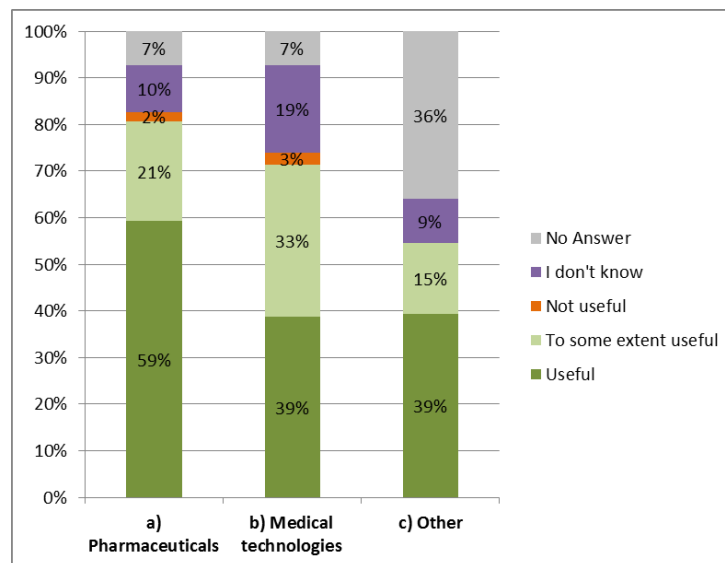


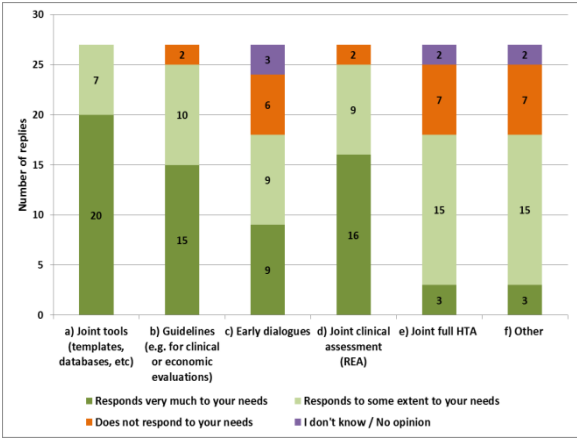
Fig. 20. Overview of opinions provided by administrations, organisations and associations, (including SMEs) regarding the scope of EU cooperation on HTA beyond 2020 (Total number of respondents = 186)

It has to be noted that of the representatives of the pharmaceutical industry who were in favour of a continuation of EU cooperation on HTA (25 of the total 27 contributors), 84% indicate that EU cooperation on HTA on pharmaceuticals is useful and the rest of 16% consider that it is to some extent useful. Concerning usefulness of EU cooperation on HTA beyond 2020 in the area of medical technologies, most of the representatives of the medtech industry who support future EU cooperation on HTA (17 of the 21 contributors) indicate that the cooperation in this sector is to some extent useful (76%), while 18% see it useful and 6% consider it not useful.

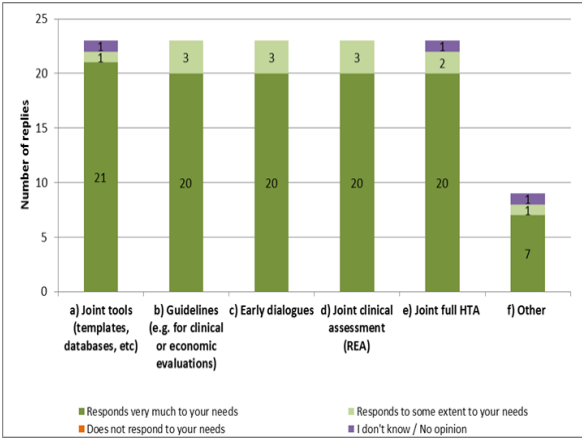
As indicated by many contributors, other technologies relevant for EU cooperation on HTA could be medical and surgical procedures, prevention (including vaccines and screening programmes), treatment and rehabilitation programmes, interventions not needing medical devices (e.g. life-style interventions, behaviour therapy), e-health tools, health-related apps for smartphones and tablets (m-health), complex interventions supporting medical decision making (health promotion, etc.), disruptive technologies (e.g. nanotechnology, personalised medicine and genomic medicine).

The consultation shows that the **needs for certain types of joint activities** vary among the different categories of respondents (Fig. 21). With reference to *joint tools* (such as templates and databases) and *joint guidelines* for clinical or economic assessments, their development and use is mostly supported by patients associations, academia, pharmaceutical industry and public administrations who indicated that they respond very much to their needs; it has to be noted that the other categories of respondents indicated that these two types of joint activities respond to some extent to their needs. For *joint early dialogues*, a majority in all respondent categories considers that they very much or to some extent meet their needs, although dissenting views were also expressed in the categories of public administrations, healthcare providers, SMEs and "other organisations". In relation to *joint clinical assessments* (relative effectiveness assessments/REA), while most of respondents see them very useful or to some extent useful, the medical technologies industry and SMEs seem to be less interested in this type of joint activity. With regard to *joint full assessments*, most of the big commercial operators indicated that this type of joint work does not respond or respond less to their needs. Scepticism towards full HTA at EU level is also expressed by some public administrations, SMEs and payers, although the majority still supports this type of joint activity.

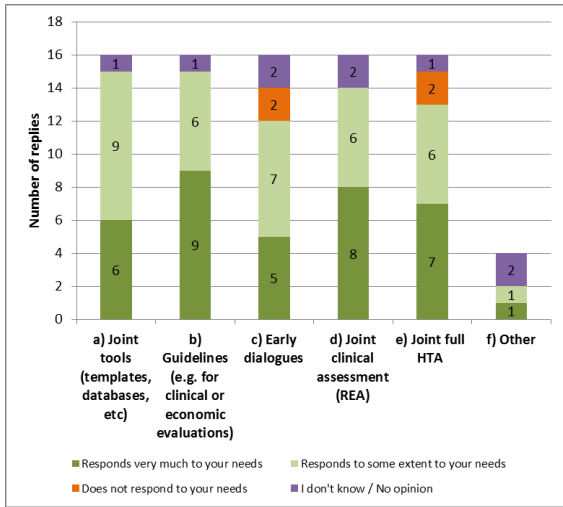
a) Public administration



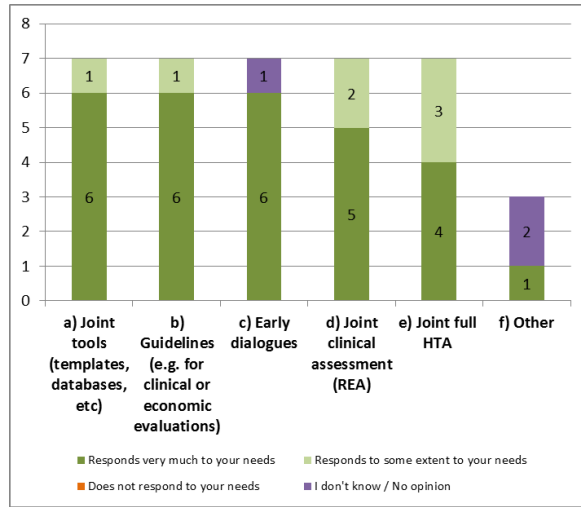
b) Patients and consumers



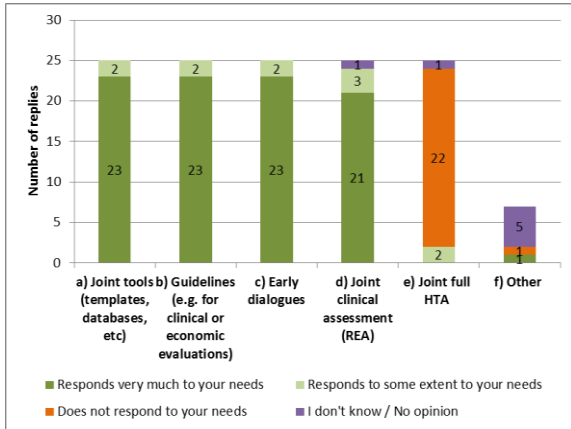
c) Healthcare providers



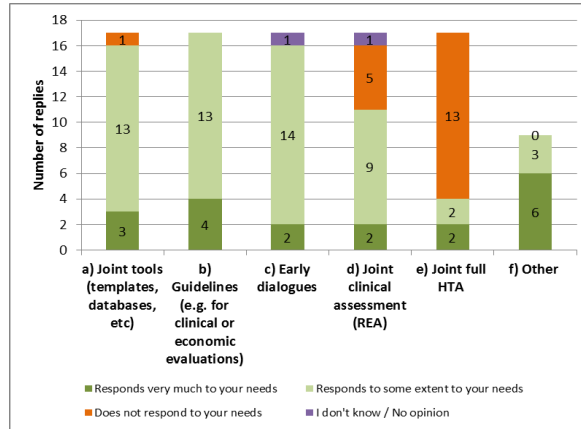
d) Academia



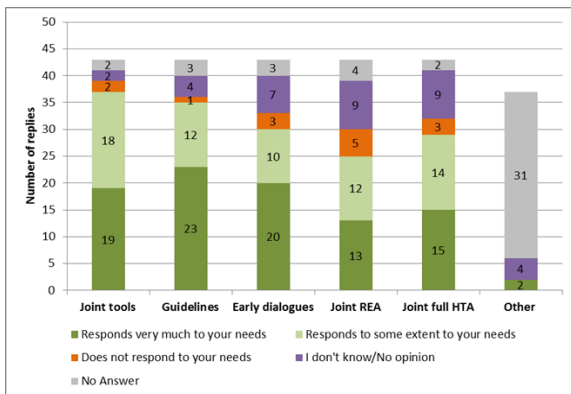
e) Pharmaceutical industry (non-SME)



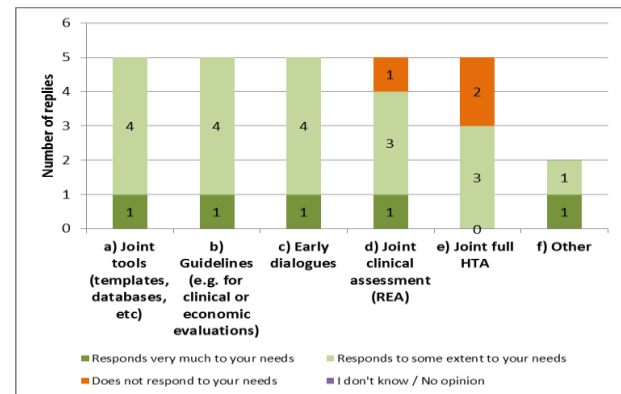
f) Medical technologies' industry (non-SME)



g) SMEs



h) Payers



f) Other organisations

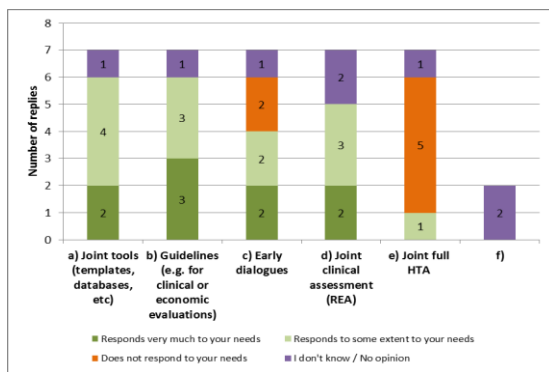


Fig.21. Overview of the answers regarding the activities relevant for EU cooperation on HTA beyond 2020 per category of respondent (Total number of respondents = 186)

With regard to the **policy options for the future EU cooperation on HTA**, respondents were asked to rank from the least preferred to the most preferred a simplified version of the policy options described in the Inception Impact Assessment. The questionnaire outlined three options with focus on the type of participation (i.e. voluntary or mandatory) and uptake by participating Member States' HTA bodies (i.e. voluntary or mandatory). As shown in Fig. 22, the "voluntary participation with mandatory uptake option" appears to be the option which is generally favoured, and at the same time the option with overall lowest opposition and the highest percentage of neutral opinions. In contrast the options voluntary participation with voluntary uptake and mandatory participation with mandatory uptake have a significantly higher opposition (50% or more) and less support.

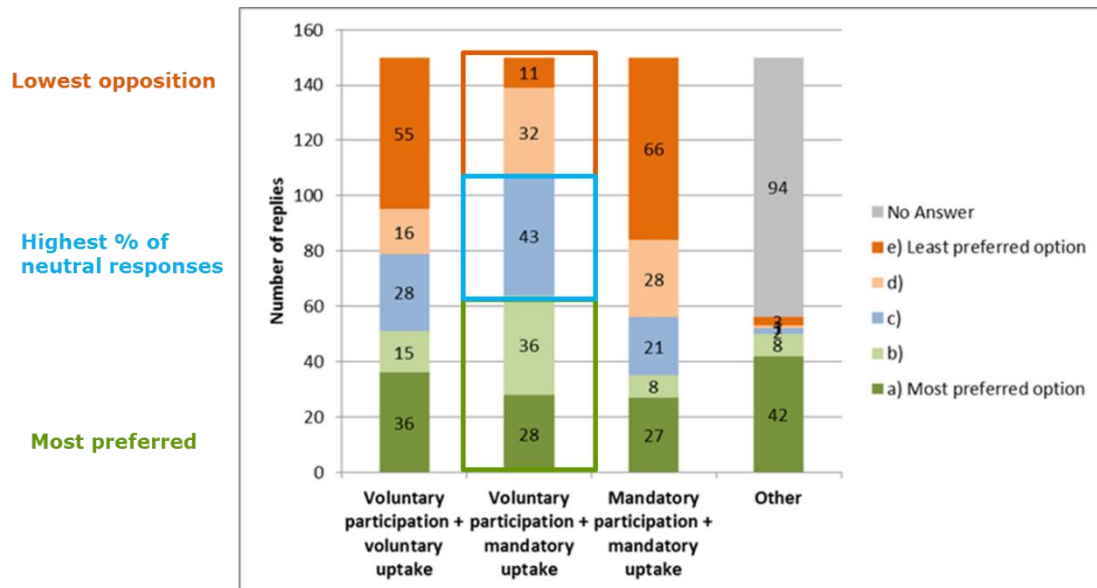


Fig.22. Overview of the opinions on the policy options for continuing EU cooperation on HTA

More than a third of the respondents provided input to the category "other policy options", as following:

- *Public administrations*. Some respondents indicate a preference for a legal framework for voluntary participation with voluntary uptake. Others underline that voluntary participation with voluntary uptake of joint work should be accompanied by mandatory compliance with agreed methods and procedures. Another option put forward is that voluntary participation

and mandatory uptake should be accompanied by the possibility to adapt joint work to the national context. Several contributors specify that voluntary participation with mandatory uptake of joint work for the participants should be limited to clinical and technical matters (i.e. REA/domains 1 to 4 of EUnetHTA HTA Core Model), whereas joint assessment of non-clinical domains (e.g. economic, legal, ethical) could be carried out by interested Member States/HTA bodies without mandatory uptake. The idea of a phase-in approach is also raised, with the option voluntary participation with mandatory uptake of joint work considered more appropriate for pharmaceuticals, while for medical devices joint work could start with the development of appropriate common tools and methodological guidelines for assessment and be extended over time. As selection criteria for technologies to be assessed jointly are proposed: public health priorities, unmet need, orphan medicinal products, important budget impact, complex products such as ATMPs and multiple technologies interventions, medical devices which according to the new Medical Devices Regulation would require evaluation of clinical performance.

- *Patients and consumers representatives* specify that more commitment from Member States is needed and confirm their support for the option mandatory participation with mandatory uptake. Several associations support the position of Eurordis, who suggests that the option mandatory participation with mandatory uptake could also foresee HTA agencies joining on a voluntary basis for developing new HTA methodologies to evaluate costs and economic aspects to be then used for price simulations based on national/local data. Other joint work to be performed on a voluntary basis could cover research (e.g. developing common methods for cost and economic aspects or for the use of real world data). The need to clarify whether the voluntary/mandatory nature applies also to stakeholders is also underlined. In this regard, it was suggested that patients' representatives should be consulted during the HTA process. As regards industry, it was suggested to introduce a mechanism providing an obligation for companies to participate to joint assessments for a selection of health technologies, including technologies for rare diseases. For the technologies not evaluated at EU level, it was proposed to introduce a mechanism of mutual recognition of assessments performed at national/regional level.

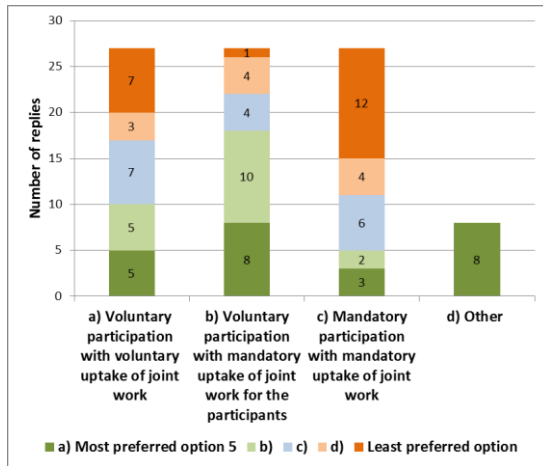
- Overall *pharmaceutical industry companies and their trade associations* support the harmonisation of European relative efficacy assessments at time of launch, accompanied by an alignment at EU level of the evidence requirements between regulators, HTA bodies and payers. Many representatives of the pharmaceutical industry advocate for voluntary participation for both Member States and manufacturers until the process of joint work has proven itself, however with mandatory uptake of joint work. It was stressed that economic assessments should remain the responsibility of Member States.

- *Medical technologies' operators and their trade associations* reiterate that their sector requires a different approach than pharmaceuticals with timing and selection of technologies to be assessed by HTA bodies and not centrally at EU level. The approach proposed by the medical technologies representatives is considered an "improved version of long-term voluntary cooperation financed by the Union", based on a centralized voluntary structure, using HTA methodologies and guidelines taking into account the particularities of this sector, with a focus on the generation of real world data in the post-launch period and including a collaborative model involving all stakeholders. It was also underlined that HTA should focus on products that are innovative and address high unmet patient needs in disease areas where appropriate clinical and economic evidence has been or can be generated (e.g. transformative in-vitro diagnostics and medical devices).

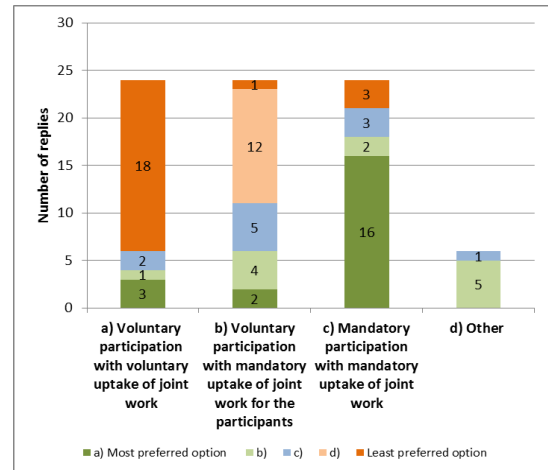
Many respondents from all categories of stakeholders emphasised the need for a clear definition of the term "uptake".

An overview of opinions on policy options per category of respondents is presented in Fig. 23.

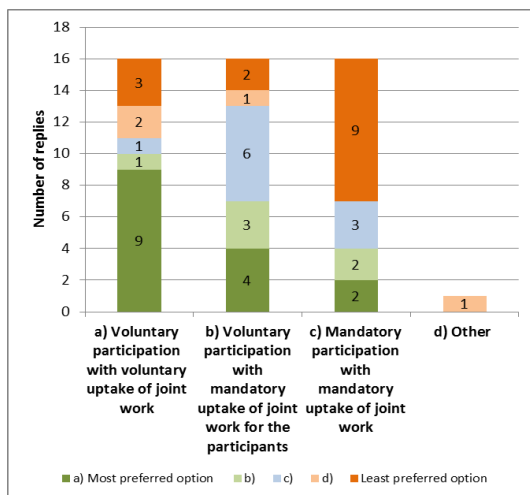
a) *Public administration*



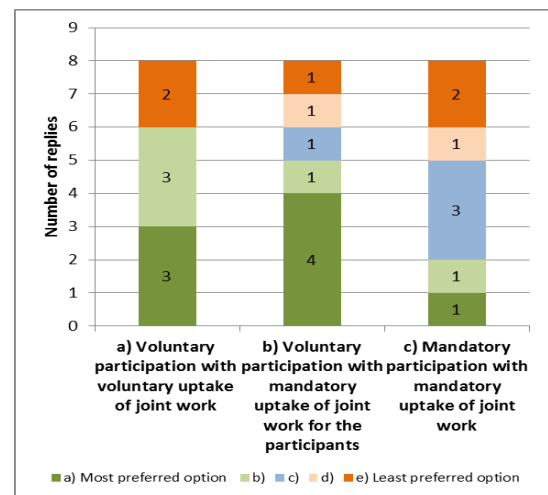
b) *Patients and consumers*



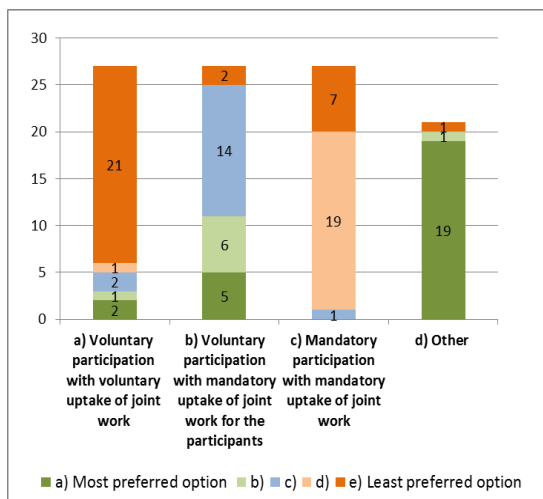
c) *Healthcare providers*



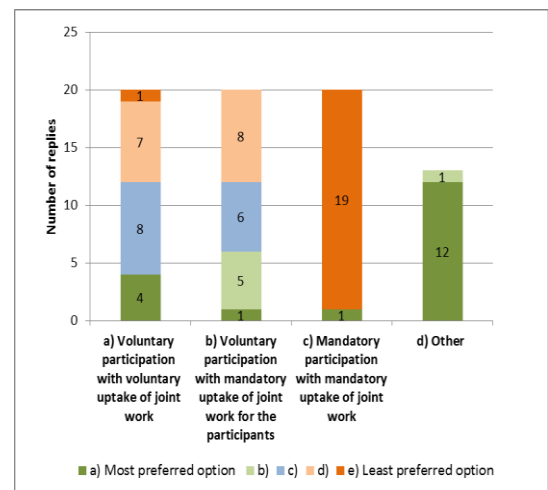
d) *Academia*



e) *Pharmaceutical industry (non-SME)*



f) *Medical technologies' industry (non-SME)*



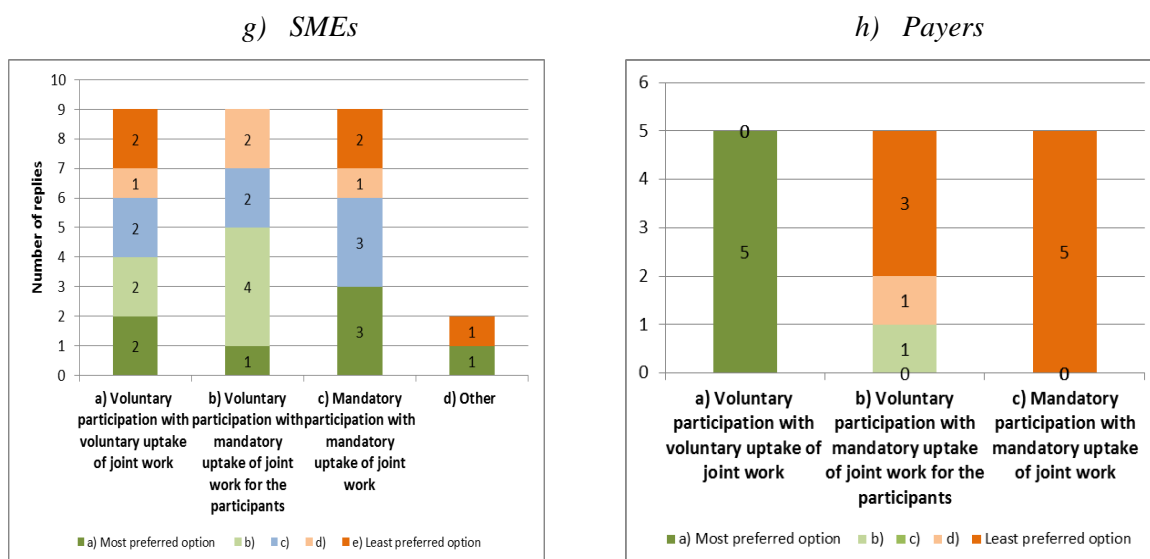


Fig.23. Opinions on policy options for continuing EU cooperation on HTA per category of respondents

In relation to the potential **funding mechanisms of the future EU cooperation on HTA**, more than half of the respondents (99 respondents, representing 53%) point towards a mix of contributions from EU budget, industry fees and Member States contributions (Fig. 24).

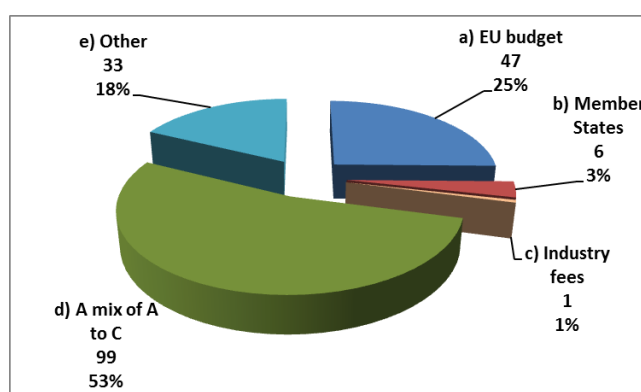


Fig.24. Overview of the opinions on the funding mechanism of the future EU cooperation on HTA

Comments provided by the different respondents in relation to the funding mechanism are presented below:

- Representatives of *public administrations* support a mixed financing system because it may allow a cost sharing between institutions and industry and therefore a high production of reports. Even though financing based on fees may be considered an opportunity, joint work should be financially independent from industry funding. However for early dialogues a "fee for service" could be considered, similar to the one received from industry for the scientific advice provided by marketing authorisation bodies. It was stressed that sustainable financing should be provided from the EU budget, which would also ensure that conflicts of interest are avoided. Participation of EU Member States was seen by most respondents as participation in kind, mainly by providing the technical expertise for the joint work.

- *Patients and consumers* representatives emphasise that financing of the EU cooperation on HTA should be based on fundamental principles of transparency, diversification, good governance and ethical conduct. The need to foresee and allocate funds to stakeholders,

patients and patient organisations and academia to ensure their meaningful involvement in the joint work is also underlined. Patients and consumers' organisations and also *healthcare providers'* associations observe that a mix of EU budget and national contributions could provide for stability and predictability while ensuring also a greater level of commitment and ownership of the Member States with respect to the EU cooperation on HTA. Many respondents warn against a funding mechanism based exclusively on industry fees, due to higher risk of conflicts of interest and influence from industry (e.g. on the choice of criteria, methodology).

- *Academia'* representatives are mostly in favour of a central funding mechanism with shared costs, in which the EU budget would provide for stability of the cooperation, covering the running costs of the coordination structure, improvement of methodologies and international cooperation. As beneficiaries of the cooperation, it was considered that Member States should contribute mainly in kind by providing the necessary expertise for the joint work. Similar to the EMA model, the possibility to charge fees was mentioned.

- *Payers'* representatives strongly advocate for ensuring the independence of HTA, and specified that in their view EU cooperation on HTA should remain publicly funded. In case fees for service from industry would be considered for joint early dialogues, fees should constitute only a minor portion of the budget and strong measures should be introduced to avoid any conflict of interest in the subsequent HTA steps.

- *Industry'* representatives and their trade associations consider that funding should be largely based on EU budget, with contributions from Member States and voluntary fee-for-service contributions from industry. It was underlined that EU cooperation on HTA should not put an additional financial burden on companies, in particular on SMEs. The *medical technologies' industry* highlight their readiness to contribute only under specific conditions such as when there is a clear link from HTA to coverage decision making, and aligned evidence generation requirements appropriate for medical technologies. Representatives of *pharmaceutical industry* indicate their openness to continue the current practice of paying a fee to receive scientific advice, provided the system to be set up is fit for purpose and responds to industry needs. Some companies also state their availability to discuss the possibility of industry fees to support joint REA provided that the joint work aligns with industry needs. Industry representatives emphasise that any fee for service system would need to be thoroughly discussed with the industry before being decided and implemented.

With respect to the **governance mechanism**, the consultation shows an overall preference towards an existing EU agency (Fig. 25).

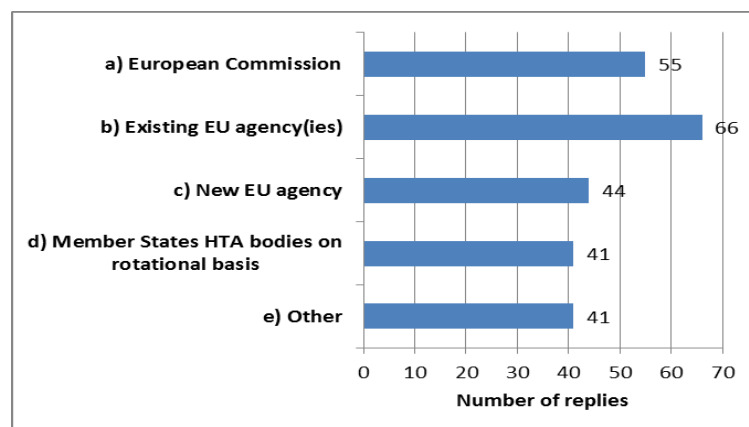


Fig.25. Overview of the opinions on the governance mechanism of the future EU cooperation on HTA

Under other potential governance solutions, respondents indicated the European Commission, a new EU agency, and Member States on rotational basis. Comments on governance mechanisms are summarised below:

- Representatives of *public administrations* emphasise the importance of separating the regulatory and HTA functions and ensuring the independence of HTA agencies. Many respondents indicate that using an existing agency by adding a structure/unit to support HTA at EU level could be seen as a practical solution, especially if EUnetHTA structures and tools (such as POP database, intranet) could be easily incorporated. While some respondents are against the creation of a new EU agency, others express their preference for this governance mechanism which would better reflect the specific needs of the HTA sector, with competencies clearly and transparently defined. However, it was also noted that this may not be an optimal solution. Several contributors note that a rotating secretariat could be assumed by the Member States, while others indicate that it may increase the risk of discontinuity and it may be challenging especially for smaller Member States due to their limited capacity. The model where the secretariat would reside in an existing EU agency, and joint work would be carried out by Member States' experts was also mentioned and recommended for future consideration.

- Many *patients and consumers associations* state that in their opinion, and based on its working model with the Member States experts, robust procedures, capacity to integrate new domains and to involve all relevant stakeholders, EMA could be entrusted to host the secretariat of the EU cooperation on HTA. It was argued that for the citizens it would be easier to understand a system in which one single agency is responsible for all aspects related to the entry on the market of medicines and the assessment of other health technologies. However there are also strong voices insisting that HTA needs to remain independent from the system of granting market authorisations (principle of division of powers), and others who underline that Member States should keep control on HTA processes to reflect their autonomy in terms of price setting, and to take into account the specificity of each healthcare system. Similar views were expressed by organisations representing "other stakeholders".

- Some *healthcare providers* state that there are no existing EU agencies suitable for hosting EU cooperation on HTA and a new agency with staff recruited from the Member States HTA agencies and closely collaborating with local HTA agencies and existing agencies such as EMA could be a "nice to have", but expensive solution. It was also noted that organisational support and coordination should be as concentrated as possible, with a rotation secretariat increasing the risk of discontinuity.

- Representatives of *academia* support a centralised model for EU cooperation on HTA, hosted by an impartial organisation, independent from industry influence. It was stressed that European REA should be conducted on the basis of accepted EUnetHTA methodological, analytical and quality standards, and the outcomes of joint work should be recognised by all Member States without any duplication from their side. Appropriate permanent mechanisms for involving relevant stakeholders including industry, clinicians and patients would be needed. It was also underlined that there is a critical need to consider the evaluation of health technologies in a full continuum from early development to post market authorisation, and cooperation with EMA would be needed to guarantee a timely and efficient joint HTA assessment of new pharmaceuticals. A dedicated unit in the Joint Research Centre of the Commission was seen as another potential option.

- Organisations representing *payers* are supportive for a coordination mechanism led by a national HTA body on a rotating basis or a small group of HTA bodies (acting as a steering group), and European Commission providing for organisational support. Additionally, some express opposition to integrating HTA activities in EMA.

- Lastly, many representatives of the *pharmaceutical industry* support EFPIA's position and noted that at this early stage it is important to clarify the principles of secretarial/organisation support, rather than determining the location of this support. A model similar to the one in EMA with a Committee composed of experts from Member States, reviewing and endorsing HTA reports prepared by national experts/rapporteurs based on agreed methodologies) was suggested.

- Representatives of the *medical technologies' industry* observe that setting up a new EU agency does not seem feasible and, while EMA is a good model for a successful agency in the field of pharmaceuticals, due to its lack of expertise would be an inappropriate host for the EU cooperation on HTA on medical technologies. In this context, an existing structure of the European Commission was seen as a potential solution for providing support from a secretarial and organisational point of view. All industry' representatives stress that any coordination mechanism should be based on highest scientific standards and should receive appropriate resources.

3.2.2.4. Additional comments

Many contributors provided additional comments, the main ones being summarised below:

- It was highlighted that in the process of shaping the future EU cooperation on HTA, consideration should be given to the following issues: distinguish between assessment and appraisal (the latter being the responsibility of national health care services and local insurance bodies), clear separation between regulatory assessment (which informs decision on marketing authorisation) and HTA (that informs decisions on added value, use of technologies and reimbursement and pricing), step-wise approach as potential key success factor, focus on selected technologies, clear and strong coordination/governance/secretarial support, extension of the scope of early dialogues to guidelines on technology development, and appropriate stakeholder involvement.

- The need for transparency, including 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, was underlined. All information should be available for review by health professionals and the public.

- The need to ensure independence of HTA bodies from industry, but also from political pressure and other interests was underlined.

- The need to invest resources for the engagement with civil society throughout the HTA process, especially of patients' organisations that can provide valuable input, was reiterated.

- Innovative medical technologies eligible for a joint clinical assessments should be clearly defined (e.g. implantable high risk products according to class IIb and III and active implants were suggested). Innovative medical technologies could be assessed as part of a medical/surgical procedure, within a treatment paradigm or diagnostic procedure.

- It was suggested that prioritisation of health technologies to be jointly assessed at EU level should be established through multi-stakeholders' involvement (including patients, providers, HTA bodies, health insurers, research institutions, industry, etc.).

- The need for a specific and comprehensive framework for HTA of vaccines and vaccination programmes was highlighted. This would require dialogue and coordination of all stakeholders including EUnetHTA, HTA bodies and National Immunization Technical Advisory Groups (NITAGs) with a view to fostering common good practice in methods and relative efficacy evaluation throughout Europe to ensure rapid implementation of effective vaccination programmes.

- Due to their important roles which include also post-market evaluation of pharmaceuticals and pharmacovigilance, community pharmacies expressed their interest to be more involved and engaged in HTA projects and actions both at national and European level.
- For medical technologies, it was reiterated that the HTA process should not affect the registration process and CE marking and HTA methodologies should be developed taking into account the particularities of this heterogeneous sector.
- The need to reshape the European pharmaceuticals' development landscape was mentioned. The added value of independent clinical studies and comparative effectiveness studies conducted with the support of the Member States, regulators and HTA was emphasised. In addition, alternative, innovative strategies for development of new treatments should be evaluated.
- In case of a review of the Transparency Directive (i.e. Directive 89/105/EEC), the insertion of the joint HTA process should be appropriately addressed.

4. Next steps

In the preparation of the Impact Assessment and the formulation of a future initiative, the Commission will duly consider the views expressed by stakeholders in this open public consultation, as well as in the other activities outlined in the consultation strategy¹². The results of the online public consultation will be complemented with the stakeholders views collected in the other consultation activities and will be summarised in the Synopsis Report which will be attached to the Impact Assessment regarding strengthening EU cooperation on HTA beyond 2020.

¹² http://ec.europa.eu/health/sites/health/files/technology_assessment/docs/20161013_strategyhta_en.pdf