EUROPEAN COMMISSION

Brussels, 31.1.2018
SEC(2018) 82 final

REGULATORY SCRUTINY BOARD OPINION

Proposal for a regulation of the European parliament and of the Council on health technology assessment and amending Directive 2011/24/EU

{COM(2018) 51}
{SWD(2018) 41}
{SWD(2018) 42}

EUROPEAN COMMISSION
Regulatory Scrutiny Board

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Brussels,
Ares(2017)

Opinion

Title: Impact Assessment / EU cooperation on Health Technology Assessment (HTA)

Overall 2nd opinion: POSITIVE WITH RESERVATIONS

(A) Context

A Health Technology Assessment (HTA) evaluates a technology and compares it to alternatives. It considers evidence and assesses the medical, social, economic and ethical characteristics of pharmaceuticals, medical devices and other technologies. Member States use HTAs to inform clinical practice and decision making on pricing and reimbursement of pharmaceuticals and medical devices. The part of an HTA that assesses the clinical effectiveness of a health technology by comparing it to existing standards is called a Relative Effectiveness Assessment (REA).

EU cooperation on HTAs started over 20 years ago. This has included joint REAs as well as collaborative projects to align tools and methodologies. This cooperation, which is currently in the form of a Joint Action, has demonstrated its potential to avoid duplication of work by national authorities. It has also revealed limitations with regard to sustainability and Member State uptake of the joint results.

The present impact assessment explores ways to strengthen EU cooperation on HTAs.

(B) Main considerations

The Board notes that the report has improved since its first opinion. It justifies better the case for considering the discontinuation of the Joint Actions as the baseline and explains the differences with the project-based cooperation option. The revised report elaborates on the proportionality of the preferred option, provides additional information on its impacts and describes the different governance models in more detail. The report clarifies the measures to improve patients' and consumers' participation in HTAs. The Board also notes the phasing and incremental nature of the preferred option.

However, the report still contains significant shortcomings that need to be addressed. As a result, the Board gives a positive opinion, on the understanding that the report shall be adjusted to integrate the Board's recommendations with respect to the following key aspects:

1) The baseline is treated as an option and not as a comparator for the options.

2) The report provides indications that the mandatory uptake of joint work would be sufficient to address many of the current shortcomings. However, it does not convincingly demonstrate that it is necessary. It is not clear what the resulting amendments to the existing Directive are.

3) The report provides insufficient indications of Member States' support for key aspects of the options.

4) The revised report insufficiently discusses the uncertainties, risks, trade-offs and implementation challenges associated with the preferred option.

(C) Further considerations and adjustment requirements

(1) Baseline and comparison of options

The report has chosen as a baseline the discontinuation of the present joint actions. This is a worst case scenario and any option which foresees some joint HTA comes better than the baseline. The report should be consistent when it compares options and make sure that the options are compared to the baseline. It should clarify in particular whether this is the case for the governance and budget figures.

(2) Proportionality of the preferred option

The report provides relevant arguments on the limitations of project-based cooperation. It should however further explain why a legal framework offering a sustainable cooperation structure could not satisfactorily address most flaws of the current system (in terms of quality and timeliness). This would result in spontaneous uptake of joint outputs, without having to introduce a legal obligation for their use by Member States. The report should, therefore, further discuss, and appropriately discard, intermediate solutions between projects and a strict legal obligation to use HTA outputs before concluding on the necessity of imposing their mandatory uptake. Furthermore, the claim that *"national rules on how HTA is actually carried out are contained in the administrative provisions of Member States' HTA bodies rather than in national legislation"* actually weakens the argument on legal hurdles limiting the use of EU REAs and the need for a legally mandatory uptake as a proportionate measure.

The report should clarify the opt-in rules (for the implementation of the regulation, for participation in the individual actions and possible derogations to the mandatory uptake). The report mentions the withdrawal of article 15 on voluntary cooperation on health technology assessment of the Directive on patients' rights. The report should indicate whether the preferred option will re-introduce some of its essential components such as the HTA Network, the good governance principle, management and transparent functioning as well as arrangement for granting aid.

(2) Member States' support

The report should better demonstrate the readiness of Member States to adopt an obligation to use outputs produced at EU level in their national procedures. **It** should provide a more thorough analysis of the willingness and capabiliy of more advanced Member States' to take a leading role in an EU framework. It should confirm the support for transparency measures such as the publication of joint REAs, considering that some Member States do not currently publicly release such results. Finally, it should further explain the choice of a Commission-hosted secretariat as an optimal scenario for Member States while the report identifies an Agency-based option as the most likely to meet the desired objectives of this initiative.

(3) Risks and uncertainties

In order to ensure well-informed policy choices, the report should address more transparently the risks associated with the initiative. Unintended consequences could, for instance, include the introduction of new requirements in some Member States to inform reimbursement or pricing decisions. Another issue could be the lack of incentives to build further capacity in low resource countries that would simply adopt the EU-level REAs, thereby perpetuating geographic imbalances in the development of EU HTAs.

The report should also explain the feasibility and risks linked to the convergence in HTA methodologies and processes given Member States' feedback on existing disparities. It should explain how a gradual approach or lessons from the joint actions could apply in this area.

On the basis of this more thorough discussion on risks and trade-offs, the presentation of the preferred option should better qualify the magnitude of expected benefits and the factors that might affect their realisation.

(4) Added value of EU REAs on new drugs

The preferred option proposes to conduct joint REAs on new (pharmaceutical) drugs subject to and immediately after central marketing authorisation procedures. Given the novelty of such drugs, and in the absence of any real world effectiveness data, the report should better explain the added value of assessing twice their efficacy using what would appear to be the same evidence.

Some more technical comments have been transmitted directly to the author DG.

(D) RSB scrutiny process

The lead DG shall ensure that the report is adjusted in accordance with the Board recommendations of the prior to launching the interservice consultation.

Full title	Impact Assessment on the initiative on strengthening EU cooperation on Health Technology Assessment
Reference number	2016/SANTE/144
Date of RSB meeting	25 October 2017

EUROPEAN COMMISSION
Regulatory Scrutiny Board

Brussels,
Ares(2017)

Opinion

Title: Impact Assessment / EU cooperation on Health Technology Assessment

(HTA) Overall opinion: NEGATIVE

(A) Context

A Health Technology Assessment (HTA) evaluates a technology and compares it to alternatives. It considers evidence and assesses the medical, social, economic and ethical characteristics of e.g. pharmaceuticals, medical devices, or other technologies. Member States use HTAs to inform clinical practice and decision making on pricing and reimbursement of pharmaceuticals and medical devices. The part of an HTA that assesses the clinical effectiveness of a health technology by comparing it to existing standards is called a Relative Effectiveness Assessment (REA).

EU cooperation on HTAs has been ongoing for over 20 years. This has included joint REAs as well as collaborative projects to align tools and methodologies. This cooperation, which is currently in the form of a Joint Action, has demonstrated its potential to avoiding duplication of work by national authorities. It has also revealed limitations with regard to sustainability and Member State uptake of the joint results.

The present impact assessment explores ways to strengthen EU cooperation on HTAs.

(B) Main considerations

The Board acknowledges efforts to conduct an analysis of Member States' structures, resources and processes in place for the development and use of HTAs. It also acknowledges the quality of the stakeholder consultation.

However, the Board gives a negative opinion, because the report contains important shortcomings that need to be addressed with respect to the following key aspects:

(1) The findings of the mid-term evaluation and the stakeholder consultation do not justify considering the continuation of the current joint actions as unsustainable.

(2) The report does not adequately justify the choice of the baseline, and the options do not adequately address all of the initiative's stated objectives.

(3) The report does not demonstrate that the initiative is a proportionate and effective response to low HTA uptake.

(4) The report does not explain what the proposed measures would imply for Member States with regard to resources or adjustments to national regulatory frameworks and practices. It does not specify the measures to improve patients' and consumers' participation in HTAs.

(5) The report does not sufficiently analyse the preferred option and the delivery mechanisms of the initiative, including related resource implications.

(C) Further considerations and adjustment requirements

(1) Baseline and options

The report should further clarify the baseline, as well as the structure, scope and content of the options.

The report should reassess and adequately justify the choice of the baseline. This should present the most likely developments in the absence of the proposed initiative. It should also provide the comparator against which the impacts of the different options and their sustainability are measured. If, despite positive feedback from stakeholders and encouraging findings of the mid-term evaluation of the 3r[d] Health Programme, the continuation of the present policy is deemed to be ineffective and/or unrealistic, this should be further substantiated. In the absence of such justifications, it is not appropriate to have a baseline that assumes EUnetHTA Joint Actions will end after 2020.

For the same reasons, the report should either discard option 2 (very similar to the continuation of the present policy) or further elaborate it and differentiate it from what is currently in place. It should then consider the possibilities to introduce incentives or conditions on the uptake of joint outputs under different grant schemes. This is the approach that the supporting study uses to assess the impacts of this option.

Overall, the analysis of options should consider a greater number and more refined credible alternatives. It should further differentiate between types of health technologies (e.g. pharmaceuticals and medical devices). It could also distinguish between new and existing technologies. This could potentially lead to mixed solutions where a project-based approach might be suitable for some cases. This exercise should result in a better tailoring of the options to the needs of Member States while addressing the different industries' concerns.

Option 4.1 (joint REA with opt-in) should better describe the relevance, advantages and risks of allowing Member States to remain out of the system indefinitely.

Option 5 (joint full HTA) raises significant issues in terms of feasibility, subsidiarity and proportionality that it should be discarded upfront.

Finally, the report should further elaborate the preferred option (4.2) by detailing and assessing its envisaged flexibility adjustments (notably the Member States-driven prioritisation mechanism and the specific support provisions for SMEs). The report should also explain how Member States would benefit from the measures in the preferred option. It should assess how these measures complement existing national and regional initiatives.

(2) Proportionality and effectiveness

The report should further demonstrate that a project-based approach cannot address the root causes for low uptake of joint work, which reportedly include issues of quality and timeliness of joint outputs, safety requirements, and reliability of evidence.

In addition, the report should draw on experience to date to convincingly explain why introducing the mandatory uptake of such joint work is necessary to address these issues. The report would need to show that this measure would be sufficient to actually achieve the goal of avoiding duplication of work on clinical assessments for health technologies.

The initiative will still allow for derogations and allow Member States to add additional national requirements. Therefore, the report should discuss whether the proposed measure might result in greater administrative burden in the process of informing reimbursement

and/or pricing decisions.

The report should also discuss how new obligations such as the mandatory uptake of joint work could be enforced. It should clarify that the new obligations would require adjustments to the existing Directive 2011/24/EU, which presently excludes measures that interfere with Member States' competences in deciding on the implementation of HTAs conclusions. Moreover, the pros and cons of using a Directive versus a Regulation should be made clearer.

Overall, to more usefully inform decision making, the report should further qualify its conclusions on the preferred option. The conclusions should be clear on the likelihood of a more efficient production and a better utilisation of HTAs. While the costs of the preferred option are clear, the report should describe the potential risks and trade-offs affecting the benefits of this option.

(3) Impacts on Member States

The report should provide more information about what the proposed measures would imply for Member States in terms of resources and necessary adjustments to national regulatory frameworks and practices. Such a description should take into account differences in Member States' HTA capacity. In that context, the report should provide assurances that steps towards harmonised tools, methodologies and standards will not be over-demanding for countries with emerging HTA practice and limited resources. It should also address the potential downsides for more advanced Member States. It should show that these countries are willing to cope with additional responsibilities to lead HTA work. It should show that the work programme at EU level allows overcoming identified bottlenecks.

The report should detail measures for the 'improved' participation of patients and consumers' representatives on one side and healthcare professionals on the other side. It should detail how best practices identified in EUnetHTA could be adapted to the different domains at stake.

(4) Governance structure

The discussion on possible governance models needs to be further elaborated. It should take into account the political constraint of not creating new agencies. It should show that the Commission has the expertise to run the secretariat internally.

Given the needs of the governance and the questions left open for the preferred option (point 1), the services could consider to add an implementation plan (Better Regulation tool #36). It should give a clear overview of the origin of the resources (Commission, new operational budget, national contributions).

(5) Presentation

The report should add information on the interface between different regulatory processes for health technologies at EU and national levels. It could draw on material in the annexes. Flowcharts or comparative tables illustrating the different requirements and timings of different procedures would help. It would be useful to include a glossary of key technical terms and concepts, including notions of efficacy and relative effectiveness.

Some more technical comments have been transmitted directly to the author DG.

(D) RSB scrutiny process	
The lead DG shall ensure that the report is revised in accordance with the above-mentioned requirements and resubmitted to the Board for its final opinion.	
Full title	Impact Assessment on the initiative on strengthening EU cooperation on Health Technology Assessment
Reference number	2016/SANTE/144
Date of RSB meeting	25 October 2017