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From:	Presidency
To:	Working Party on Pharmaceuticals and Medical devices
Subject:	Proposal of the European Parliament and of the Council on health technology assessment and amending Directive 2011/24/EC - <i>Presidency discussion paper</i>

Delegations will find in the Annex a Presidency discussion paper outlining five scenarios for how to achieve that health technology developers fully fulfil the information requests for joint clinical assessments. This document is intended for the Working Party meeting on 1 September.

Discussion paper

How relevant and useful a joint clinical assessment report can be to Member States is determined, *inter alia*, significantly by whether the information that is requested from health technology developers according to the assessment scope is actually available to assessors. Thus, the goal is to achieve full availability of all requested information. This is the absolutely necessary prerequisite for high-quality joint clinical assessments. Only when joint clinical assessments are based on complete information, do they stand the chance to meet Member States' needs in terms of HTA.

The full availability of all requested information demands a high level of compliance from health technology developers. Based on experience in national systems, this is (unfortunately) not something that can be automatically assumed to be the case – even if health technology developers are legally obliged to submit this information. Some national systems have therefore introduced sanctions in the form of consequences in their national pricing and reimbursement processes, which punish health technology developers for non-compliance by means of either restricting or delaying reimbursement or negative effects on the pricing of the concerned products. Such sort of sanction at national level appears to be a more effective incentive for health technology developers to submit full information than penalties could be.

However, such sort of sanction is not possible at EU-level. Hence, at EU-level we have to carefully scrutinize which fallback mechanisms are viable options for getting all information necessary to carry out a joint clinical assessment in case health technology developers do not abide by their obligations. In essence, the necessary information could come from either one of two sources: the national systems, or the EMA.

During previous presidencies, several scenarios have been outlined, that aim at safeguarding the process if health technology developers do not abide by their obligation to submit all requested information. Here, we outline different scenarios, including new ones (scenarios 3 to 5), aimed at achieving availability of complete information necessary to conduct joint clinical assessments. We delineate the advantages and disadvantages of the scenarios regarding their potential to achieve availability of full information, also taking into account their potential pitfalls. These assumptions are based on the premise that article 8 foresees that Member States have to take into consideration joint clinical assessment reports.

The aim of this discussion paper is to discuss at the upcoming working party what should be best integrated into the regulation. This will then constitute the basis for a concrete text. Importantly, we do not consider the outlined scenarios as static. Thus, it might make sense to combine their different elements to come up with a different combination to achieve full availability of all requested information.

Scenarios on how to achieve that health technology developers fully fulfill information requests for joint clinical assessments.

1. Scenario 1

- This scenario foresees that health technology developers (HTDs) have to submit complete information according to the scoping process at the latest 60d prior to CHMP opinion (1st request).
- If assessors deem that information is missing from the submission, this information has to be handed in upon request by assessors according to a new deadline (2nd request), which will be defined among other details of the procedure, in an implementing act.
- On the HTD side, HTDs are not allowed to submit information from the 1st and 2nd request at national level that they have submitted at EU-level, starting from the 1st submission deadline (60d prior to CHMP opinion).

- In case of violation of these obligations on the part of the HTD, consequences at both EU- and national level shall apply:
 - At EU-level, the joint clinical assessment can either be discontinued or continued based on (if available at all) the incomplete information that has been submitted by HTDs and other sources of clinical information that would complement the submitted information. In this case, the joint clinical assessment report shall contain a statement that it was conducted based on incomplete information.
 - At national level, MS shall impose penalties on the HTD.
- On the Member States' (MS) side, information that has been submitted by HTDs at EU-level, can never be requested again at MS level, starting with the time the HTD is being informed about the submission deadline (i.e., before the HTD has submitted any information).
- If MS receive information from HTDs despite the ban to submit information that has been requested at EU level, they shall share this information with the Coordination Group, if it falls within the request at EU level.

Consequences for MS

This scenario entails that, first, as long as the HTD has not submitted information at EU-level, MS do not know what they can or cannot request at national level, as HTDs might not (fully) fulfil the information request. This is because the ban to request what has been submitted starts already at the time of the request. Second, for the same reason, MS cannot request information, once requested from the HTD at EU level- even if the HTD has never submitted any information whatsoever, thus bearing the risk, that no information at all is available on a given product.

However, some HTDs may submit information to MS that commonly get information from HTDs despite the ban to submit information at national level that has been requested at EU level. This is because HTDs want to get their product into reimbursement in MS that are strategically important for them – at least if the product is good from their point of view. MS that usually do not get information at national level, or get it late, will get some information via other MS. In terms of getting a conclusive and timely joint clinical assessment report, chances are slim for MS. They will either get no joint clinical assessment report, or get a joint clinical assessment report based on incomplete information, or get a joint clinical assessment report (potentially also not fitting their requirements) at a later time point, assuming assessors will take into account information that has reached the Coordination Group via the national route. This late joint clinical assessment report would likely violate the deadline set for the publication of the joint clinical assessment report and could lead to parallel national and EU-level clinical assessments. For assessors in particular, but also the entire system of joint clinical assessments, this scenario is quite burdensome, given that assessors have to chase after information that the HTD has not submitted at EU-level.

Consequences for HTDs

In this scenario, the HTD can basically circumvent a conclusive joint clinical assessment at his own discretion. If he doesn't submit the requested information at EU-level, or only a selection of it, he can still submit at national level. The HTD has no direct incentive to submit data at EU-level. However, over time, the HTD might get under pressure to submit at least the information that he submits at national level in a selection of MS, at EU-level.

2. Scenario 2

Like scenario 1:

- This scenario foresees that HTDs have to submit complete information according to the scoping process at the latest 60d prior to CHMP opinion (1st request).
- If assessors then deem that information is missing from the submission, this information has to be handed in upon request by assessors according to a new deadline (2nd request), to be defined via an implementing act.

- In case of violation of these obligations on the part of the HTD, the joint clinical assessment will be discontinued and a statement is published that states that the HTD failed to comply with the requirements of the regulation.
- On the MS' side, information that has been submitted by HTDs at EU-level, cannot be requested again at MS level, starting with the time the HTD is being informed about the submission deadline.
- If MS receive information from HTDs at national level that falls within the request at EU-level, they shall share this information with the Coordination Group.

Different from scenario 1

- Scenario 2 encompasses an end date for the ban on MS to request at national level information that has been submitted at EU-level; the ban ends with the publication of the joint clinical assessment report.
- Scenario 2 does not foresee penalties at MS level in case HTDs violate their obligations.
- Scenario 2 entails –in case of non-compliance on the part of the HTD- the publication of a list of the information that was not provided by the HTD.
- If the requested information reaches the Coordination Group through the MS and the CG decides that it is complete, then the joint clinical assessment will be resumed under scenario 2.

Consequences for MS

Consequences for MS are similar to those delineated in scenario 1. In terms of getting a conclusive joint clinical assessment report, however, MS stand a better chance to get one, because the joint clinical assessment report will be discontinued if the HTD did not submit complete information, and only be resumed, once information is available at EU-level via the MS' route. Thus, if a joint clinical assessment report is produced, it will be based on a more comprehensive set of information than in scenario 1.

The crucial question here is how and when the Coordination Group takes the decision on whether at a certain point in time the originally requested information is now fully available through the MS. A mechanism such that the Coordination Group needs to agree by consensus on resuming the joint clinical assessment, followed by a majority vote if no consensus could be reached would probably mitigate the risk that, on the one hand, one single MS can prevent the joint clinical assessment and on the other hand, that MS do not get the information they need. The problematic issue in this scenario is the timeline, as a late joint clinical assessment report (stemming from the resumption of the assessment) would likely violate the deadline set for the publication of the joint clinical assessment report and could lead to parallel national and EU-level clinical assessments.

Consequences for HTDs

In this scenario, the HTD can get around submitting the requested information at EU-level, in a similar way as in scenario 1. However, he ultimately cannot avoid a joint clinical assessment report (at least when submitting information at national level), given that the process will be resumed once all requested information is available through MS.

Despite the legal obligation, HTDs may remain reluctant to submit complete information at EU-level. However, over time, based on the experience with high quality joint clinical assessment reports and the overall added-value of EU-HTA as a one-stop-shop, this might pose a sufficiently high incentive for HTDs to abide by the new regulation and submit at least the information that he submits at national level in a selection of MS, at EU-level.

3. Scenario 3

Like scenario 1:

- This scenario foresees that HTDs have to submit complete information according to the scoping process at the latest 60d prior to CHMP opinion (1st request).
- If assessors then deem that information is missing from the submission, this information has to be handed in upon request by assessors according to a new deadline (2nd request), to be defined via an implementing act .

- On the MS' side, information that has been submitted by HTDs at EU-level, cannot be requested again at MS level, starting with the time the HTD is being informed about the submission deadline.
- If MS receive information from HTDs at national level that falls within the request at EU-level, they shall share this information with the Coordination Group.

Like scenario 2:

- Scenario 3 encompasses an end date for the ban on MS to request at national level information that has been submitted at EU-level; the ban ends with the publication of the joint clinical assessment report.

Scenario 3 foresees that the joint clinical assessment will be discontinued if the HTD fails to comply with the requirements of the regulation, and a corresponding statement is published.

- Scenario 3 entails –in case of non-compliance on the part of the HTD- the publication of a list of the information that was not provided by the HTD.
- Scenario 3 does not foresee penalties at MS level in case HTDs violate their obligations.

Different from scenarios 1 and 2:

- If the requested information reaches the Coordination Group through the MS, this information is accessible to all MS, however the joint clinical assessment will not be resumed. Instead, there will be an automatic update of the joint clinical assessment one year later.

Consequences for MS

Consequences for MS are similar to those delineated in scenario 2, however, there is no decision on when to resume the joint clinical assessment. In terms of getting a conclusive joint clinical assessment report, however, MS will likely not get one at the time of the initial request if HTDs do not abide by their obligations. However, MS will have access to information through other MS (as is also the case in the previous scenarios). The chance of getting a conclusive assessment in the form of an update after a year is quite good, as MS will provide information as it reaches them, and, crucially, different from scenario 2 there would be no timeline problem within the regulation. There might, however, still be some national assessments that run in parallel with the EU-level assessment to comply with national pricing and reimbursement procedures. Once the update at EU level is completed, MS may reevaluate their pricing and reimbursement decisions.

Consequences for HTDs

In this scenario, the HTD can get around submitting the requested information at EU-level. However, it cannot circumvent a joint clinical assessment report, given that there will be an update after a year, when likely the bulk of originally requested information as well as information that goes beyond the original request is available through MS. Despite the legal obligation, HTDs may remain reluctant to submit complete information at EU-level. However, over time, based on the experience with high quality joint clinical assessment reports and the overall added-value of EU-HTA as a one-stop-shop, this might pose a sufficiently high incentive for HTDs to abide by the new regulation and submit at least the information that he submits at national level in a selection of MS, at EU-level. In the end, they cannot evade a joint clinical assessment because of the update.

4. Scenario 4

Like scenario 1:

- On the HTD side, HTDs are not allowed to submit information from the 1st and 2nd request at national level that has been requested at EU-level, starting from the 1st submission deadline (60d prior to CHMP opinion).

Like scenarios 1, 2 and 3:

- Scenario 4 foresees that HTDs have to submit complete information according to the scoping process at the latest 60d prior to CHMP opinion (1st request).

Like scenarios 2 and 3:

- Scenario 4 does not foresee penalties at MS level in case HTDs violate their obligations.

Different from scenarios 1, 2 and 3:

- Scenario 4 foresees a formal completeness check and as a consequence possibly a 2nd request at the beginning of the procedure (timeline to be specified in the regulation), right after the HTD has handed in information according to the first request. This serves the purpose that the information is not deemed incomplete because of formal issues, such as the HTD having forgotten to hand in a certain set of pages or transmission errors etc. The Coordination Group has to decide on the formal completeness via consensus/majority.
- Multiple (additional content-related) requests are then possible throughout the procedure. This is because assessors might realize in the process that further details are needed from the HTD in order to complete the assessment.
- MS are not allowed to request information at national level that has already been requested at EU-level, starting with the 1st request. This ban has no end date, that is, information once requested at EU-level can never be requested again at national level.
- In case the HTD does not hand in the requested information, as attested by *the formal completeness check*, consequences at EU- level shall apply:
 - The joint clinical assessment will be discontinued and a statement is published that states that the HTD failed to comply with the requirements of the regulation, alongside a list of the information that was not provided by the HTD.

Consequences for MS

On the MS' side, information that has been requested by HTDs at EU-level, can never be requested again at MS level, starting with the time of the 1st request.

There are two crucial points in this scenario:

- The first crucial point is how formal completeness of information submitted by the HTD is decided upon. If it is decided by consensus, a single MS can prevent that there will be a joint clinical assessment at all. If instead a majority vote is in place, there is a risk that some MS will never be able to request information from the HTD that they need for their national procedure (this might be the case when the request is too unspecific or unclear, or the HTD does not fulfill it, however, the Coordination Group decides by majority vote that the submitted information is “good enough”).
- The second crucial point is how MS can handle the case that the HTD decides – for whatever reason - not to submit the requested information at all. MS would have no chance to get any information at national level either. In some MS this would lead to them having to take reimbursement decisions on no information.

Chances to get a comprehensive and timely joint clinical assessment report are high in this scenario, as effectively a parallel national assessment report is being prevented by the ban on MS to request already requested information and the ban on HTDs to submit information at national level that has been requested at EU-level, so HTDs will have an incentive to submit the requested information at EU-level, and in a timely manner. This is because they will not risk to delay national processes in markets that they usually access soon after marketing authorization.

Consequences for HTDs

HTDs can effectively not submit at national level what has been requested at EU-level, and in addition MS cannot request the information needed for national assessments once it has been requested at EU-level. As a consequence, HTDs have no room to circumvent a joint clinical assessment report apart from not submitting anything at all.

This would, however, shed bad light on HTDs and their products, so it might be a rather unlikely action on the part of the HTD

5. Scenario 5

This scenario differs from all previous four scenarios in that the process for MS and the HTDs is maximally streamlined:

- There is no request by the Coordination Group and no direct submission of information by the HTD to the Coordination Group.
- Instead, the obligations regarding the HTD are limited to hand in what is being requested by the European Medicines Agency (EMA). This information would be automatically transmitted to the Coordination Group.
- On the MS side, requests for information at national level shall not duplicate what has been submitted to the EMA. There is no possibility to hand in information at a later stage. Consequently, there are no consequences foreseen, as is the case with sharing of information between MS and the Coordination Group.
- The Coordination Group then assesses the product based on the information that the HTD has handed in at the EMA. Usually, the clinical studies being evaluated for HTA are the ones that have been handed in for authorization purposes.

Consequences for MS

One advantage of this scenario is, that the administrative burden for MS is low, since there is no request and no decision about completeness.

Regarding the likelihood of getting a comprehensive and timely joint clinical assessment report, chances might vary by product. The analyses provided by the HTD to the EMA will not necessarily suit those needed for HTA, especially indirect comparisons. Moreover, such setup could evoke the impression, that information (e.g., certain analyses) beyond what EMA requests for their marketing authorization procedure, is not necessary.

There is likely no timeliness issue in this scenario, as information is submitted to the EMA by default about a year in advance of marketing authorization.

Consequences for HTDs

For HTDs, this scenario provides for a one-stop-shop that is to their advantage in terms of administrative burden. On the other hand, they will not be able to circumvent a joint clinical assessment in this scenario, as information transmission from the EMA to the Coordination Group would be automatic.