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EUCOPE's feedback to the public consultation on the draft Implementing Act on JCA of medicinal products

The European Confederation of Pharmaceutical Entrepreneurs (EUCOPE) is the European trade association for small to mid-sized innovative life science companies.

The draft Implementing Act (IA) on Joint Clinical Assessment (JCA) of medicinal products, prepared by Member States' "Comitology" Committee on Health Technology Assessment (HTA) with the support of the European Commission, has recently been published for public consultation and feedback. The draft IA will establish the detailed procedural steps and timelines of the new EU HTA procedure.

EUCOPE welcomes the Regulation (EU) 2021/2282 on health technology assessment (HTA) and its aims to provide a transparent and inclusive framework for quality HTA, to reduce duplication for national HTA authorities and industry and to facilitate business predictability and ultimately, to accelerate access of medicines to EU patients.

However, we do not believe that the draft rules in their current format will allow for these aims to be achieved due to the lack of systematic and early interaction with Health Technology Developers (HTDs) at the scoping stage and the restrictive timelines, which will put small to mid-sized companies that are less resourced under a serious threat of being unable to prepare and submit a high-quality dossier.

In the following we explain our workability concerns, and provide some proposed solutions, based on our member companies' experiences with national HTA procedures and participation in the EUnetHTA Joint Actions.

The proposed timelines are too short, risking the workability of the procedure

While JCA is intended to start at the time of filing for Marketing Authorisation, the first 140 days are spent solely on developing the scope of the assessment, without involvement of HTDs. There is no benefit in delaying finalisation of the PICO(s) until after the Day 120 clock-stop, on the contrary it risks the quality of the JCA report, by putting undue pressure on HTDs who are submitting the critical evidence and analyses for the assessment.

Given that the overall timeline for JCA is fixed, and also much shorter than the national HTA procedures, it is simply not feasible to require the HTDs to only begin developing the dossier submission after two-thirds of the overall time for JCA has passed, and to only provide 90 days for the dossier preparation.

The timeline of 90 days poses major challenges for HTDs and should be extended, with less time used on developing the scope. A further shortening of the timelines for Type II variations should be avoided, as the timeline of 90 days is already very limited time to develop a high quality dossier given the potential high number of sets of parameters in terms of PICO(s) from all Member States to be accounted for.

The scoping phase clearly needs to be made more efficient. One way to achieve this would be to also include input from HTDs on the potential scope and considering any relevant information from HTDs based on their substantial prior research into the disease area and the condition, either in writing or via a scoping meeting.

There is also no reason why the scoping preparations cannot start before the EMA submission, as HTDs could also confirm the intention to file for a Marketing Authorisation ahead of time, allowing HTA authorities to start their preparations in advance.

- The timelines described in Article 12 (3), (5), (6) and Article 14 (4) are unworkable and need to be at minimum 30 days with possibility for extension, to allow HTDs to fulfil the requirements to provide additional analyses, factual accuracy checks and justifications.

Without possibility of early interactions, especially small to mid-sized HTDs are put under serious threat of being unable to deliver on the aim of the Regulation

HTDs require sufficient information up front from the selected assessors to prepare for JCAs, and there needs to be sufficient time for companies to gather, analyse and submit requested information to the assessors. Given the relatively short envisaged timeline of three months for developing the dossier, all HTDs subject to JCA must be invited to a scoping explanation meeting, and the meeting must take place earlier to be of value.

Without the possibility for early interactions with the assessors, especially small to mid-sized HTDs that are less resourced are put at a serious disadvantage, as there is no way to receive clarity on the scope of the assessment to begin preparations for JCA. This goes against the aim of the Regulation of reducing burdens of parallel procedures and importantly risks the quality of the assessments, which are based on a submission dossier consisting of evidence and analyses provided by the companies.

In general, opportunities for dialogue between HTDs and HTA bodies reduce the risk of misunderstandings regarding what evidence can be expected to be brought forward, which methods to apply and which comparators should be used for an assessment. Such interactions are a well-established part of many national HTA procedures, and also the previous EUnetHTA Joint Actions. Especially assessments of Orphan Medicinal Products (OMPs) and Advanced Therapy Medicinal Products (ATMPs) stand to benefit from interaction between developers and assessors, so that the complexities of the disease and the technology can be appropriately captured.

There are also currently large variations in standards of care between Member States and allowing for a discussion of the proposed scope, i.e. PICO(s), during a scoping meeting allows developers to share their insights and specific considerations and to identify any possible issues with compiling the dossier, increasing the quality of submission dossiers.

Article 4 (4) of the Regulation on HTA specifies that the procedural guidance to be developed shall take into account, where appropriate, the methodology developed by EUnetHTA Joint Actions. In EUnetHTA JA3 the scope was developed with input from the developer in the form of a letter of intent, as well as an in-person scoping meeting with the assessors, before the final PICO was defined.

With the complexity of establishing the new JCA procedure, due to its novelty, the by now established practice of inviting all HTDs subject to JCA to a scoping meeting, during the scoping phase of the procedure should be maintained.

If more time cannot be found for HTDs to develop the dossier, the draft scope should as a minimum be shared with HTDs earlier in the scoping phase, and HTDs must be invited to a scoping meeting to allow for anticipation of the various different PICO scenarios. If HTDs are not able to begin their preparations earlier, the quality of the submission dossier and ultimately the JCA report is put at great risk.

- We propose to amend Article 2 so that all HTDs are invited to a scoping meeting to discuss the draft scope, and to also provide input in writing on the PICO(s). Systematic and early involvement of HTDs via a scoping meeting, as is currently guaranteed in many national HTA procedures, and the EUnetHTA Joint Actions must be maintained, especially as we understand JCA is intended to replace equivalent national procedures, reducing duplication.

- Further, Article 11 should be amended to make the assessment scope explanation meeting a mandatory step of the procedure, and for efficiency this could be merged with the assessment scope consolidation meeting of Article 10. HTDs as the key stakeholder must also be given the chance to provide oral or written comments during the meeting.

Additional comments

- Article 20 should be modified to ensure that all confidential data identified as such by the HTD are protected and not made publicly available without the HTD's prior written consent. The involvement of The JCA Subgroup as a third-party to these decisions is unwarranted. The JCA Subgroup is not adequately equipped to decide on the commercially sensitive nature of information, only the HTD possesses all the necessary background to make this judgement. Confidential data should include data from the JSC process, clinical trial data or any other HTD-proprietary data not available in the public domain. It is also not clear how companies are supposed to demonstrate the sensitive nature of the commercially sensitive data.

- The procedure for exchange of information between the EMA and the HTA Secretariat described in Article 3 (4) should be transparent, and the HTD should be informed about what information is being shared, and for what purposes. Further clarity is needed on what information can be exchanged, whether assessors can request information from the EMA at any time and whether the Marketing Authorisation Applicant will be asked to provide information.

- In order for HTDs to start their preparations for filing for national HTA procedures, it would be beneficial to receive information about which PICO(s) have been requested by which Member States as early as possible.

- Regarding the Annexes which include the submission dossier templates, HTDs are asked to include details of any substantial variations in clinical pathways, details on regulatory status/early access/compassionate use programmes and HTA reports from EEA states, Australia, Canada, UK and the U.S. While we understand the intention to gather information that could be relevant for the JCA, this should not be a requirement for all products as there can be significant differences in approaches to HTA. For the U.S. for example, the Institute for Clinical and Economic Review (ICER) is not a formal public body and should therefore not be included. Further details on the requirements for responder definitions (e.g. MID) would be welcomed, as well as specific guidance on subgroup analyses.

- We appreciate the effort for the JCA to involve patients, clinical experts and other relevant experts as well as national authorities and stakeholders in accordance with the procedural rules of the respective Member States to take part in the process. However, the process itself for this collaboration is not very detailed in the current draft and more clarity would be welcomed. Further, it is not clear how the input from different stakeholders is weighed and integrated into the final assessment scope, and further transparency would be welcomed.