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EU-HTA in 2025: Industry's Stake in the Scoping Process

Objective

The HTA landscape in Europe will dramatically transform over the next decade with the recent legal changes that initiate a stepwise shift from a segregated, national HTA to a joint EU-HTA. Starting in 2025 with oncology therapeutics and ATMPs and followed by orphan drugs in 2028, HTA of all new medicinal products will be mandatory on EU level. This requires a structure which reliably and efficiently leads the assessment authorities as well as the manufacturers through the HTA process. EUnetHTA 21 is currently developing the corresponding necessary regulations in the form of guidelines. Thereby the scoping process is of particular importance since the scientific problems to be answered in the EU-HTA are specified here. Stakeholders are supposed to be involved in the guideline development through a hearing process. However, it is unclear whether and to what extent the hereby identified critique will be addressed by the EUnetHTA 21.

Methodology

Using the scoping guideline (ID D4.2) as an example it is investigated which barriers are existing from the hearing party's perspectives with respect to the planned scoping process and whether they have been addressed by the EUnetHTA 21 in the final version.

For this purpose, at first the published comments regarding the identified main issues are consolidated. Afterwards the draft and the final version of the guideline are compared automatically in order to derive which adaptations have been performed by the EUnetHTA 21.

Results

The draft of the scoping guideline was placed for commenting from May 2nd until May 31st, 2022. The final version was published on September 12th, 2022. In total, 27 parties (associations, manufacturers, service providers) submitted their statements. However, apart from the European associations, only 4 EU member states (plus Switzerland and UK) submitted their comments. Especially the following four main issues are viewed as critical:

1. Intransparent process to determine the PICO schema which places a higher value on national interest than on scientific findings

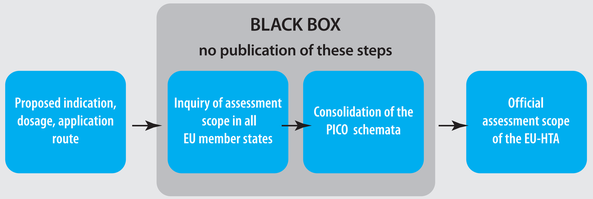

Figure 1: Procedure of the scoping process

→ For the national assessment processes which follow the EU-HTA, information about the submitted PICO schemata of the member states are inevitable.

2. Lack of binding and realistic timelines for the overall process

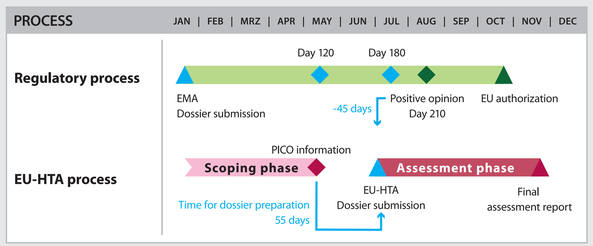

Figure 2: Timeline of the EU-HTA process

→ The date of the dossier submission cannot be concretely determined since it is depending on a future event (45 days before the positive opinion).

→ Impacts of the clock stop in the regulatory process on the EU-HTA process remain unclear.

3. No participation of the manufacturers in the scoping process

- The scoping process is based on the indication, dosage and application which act as basis for the applied marketing authorization. The manufacturer has no option to provide additional information.
- The manufacturer has no option to comment on the final, consolidated PICO schema.

4. Definition of numerous PICO schemata

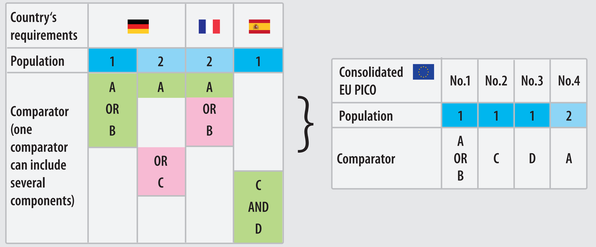

Figure 3: Example of a PICO consolidation (Comparator excluded; Comparator included)

→ The consolidation process can result in a multitude of PICO schemata.

→ Simultaneously, the minimum common denominator is supposed to be generated. Comparators which are specified as "OR links" by a member state can be eliminated during the consolidation phase.

Comparison of draft vs. final version of the scoping guideline

- A comparison of the draft and the final scoping guideline shows that only a few modifications have been made. Particularly adjustments on the linguistic level have been performed.
- The only content-related modification is focused on the consolidated PICO schema: The requirements of **each** member state have no longer to be reflected in the consolidated PICO schema. Instead, cases in which a PICO is required only by a **single** member state shall be further discussed according to the final guideline.
- The four content-related points of criticism mentioned above which had been addressed in the stakeholder's comments have not been considered.

PICO: Population, Indication, Comparator, Outcomes

Conclusion

- None of the four identified main issues of the statements have been implemented in the final guideline. From EUnetHTA 21's comments it doesn't become apparent why the implementation has been denied. The stakeholder's influence on the design of the guideline seems to be highly limited so far.
- EUnetHTA 21's answer to the multitude of comments was that the comment was "*out of scope*" of the corresponding guideline and that more detailed information would follow in the *Implementing Acts*. Due to the fact that the *Implementing Acts* are expected just shortly before launching the EU-HTA in 2025, the question how the manufacturers can prepare themselves regarding these requirements on such short notice remains open.
- The manufacturers should use the option of early consultation – also on a national level.
- Necessary organizational adjustments as well as a strategic considerations for the product portfolio should already be addressed now in order to find favorable pragmatic solutions for the upcoming EU-HTA.

References

- Regulation (EU) 2021/2282
- EUnetHTA 21 Practical Guideline D4.2 Scoping Process Version 1.0, 12.09.2022
- EUnetHTA 21 – Individual Practical Guideline Document – Practical Guideline Scoping Process Version 0.3, 02.05.2022
- EUnetHTA 21 Public Consultation - Merged comment form D4.2 – Scoping phase