OPEN HEALTH

European Health Technology Assessment Regulation:

Implications for the industry and the 5 key areas for successful implementation

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The European Health Technology Assessment Regulation (EU HTAR) mentions that Population, Intervention, Comparator, and Outcome (PICO) should not be data driven (in other words, PICO should not be based on the evidence developed around the innovation) but based on policy needs. This implies that the information provided by the Health Technology Developers (HTDs) will be considered during this scoping process. However, HTDs will be excluded from the PICO scoping process and discussions, and each Member State will be asked to draft a country-specific PICO based on country-specific policy needs.

A survey will be sent to all Member States for input on PICOs. The PICOs will then be consolidated by the coordination group. Multiple PICOs will likely be required for the JCA given that clinical practice will differ across Member States. Early scoping tests conducted by different actors show that at least 5 PICOs can be expected, with major divergencies expected on (sub)population and comparators.

Timelines will be challenging. According to the current timeline (still under discussion), the final PICOs will be known around 90 days prior to the JCA submission deadline, leaving little time for evidence generation (such as systematic literature reviews [SLRs], indirect treatment comparisons [ITCs], models) to be completed. This means that early engagement with local affiliates and key external stakeholders (payers, healthcare providers, patient organizations) will be crucial to understand differences in clinical practice between Member States that will impact the scope of work.

In short, the main concerns are:

- How will prioritization of Member States' needs and requirements be done in a fair and transparent way?
- Will the 'biggest impact across the EU' for an intervention be defined as affecting the highest number of patients or the greatest number of Member States?

Challenges include the level of complexity, short timeframes, and limited opportunity to contribute to the scoping process, in addition to the need for:

- Multiple PICOs, including off-label treatments (multiple SLRs and ITCs)
- Methodological requirements unclear due to differences between Member States, but gaining more clarity in 2024

Will the list of comparators be based on the best available alternative, or will it also include later-generation therapies in countries that do not yet use novel treatments as their standard of care?

5 Key Areas for Successful Implementation

Evidence Generation

1

Uncertainty Mapping & Metric Validation

OPEN Health is conducting a thorough investigation of uncertainties in the European health technology assessment (EU HTA) landscape through meticulous mapping and stakeholder engagement, aiming to develop and validate an uncertainty metric to inform evidence generation for various population, intervention, comparator, outcome, and study design elements.

Evidence Generation

2

Systematic Literature Review and Network Meta-Analysis within EU HTA Regulatory Timelines

OPEN Health is strategically conducting systematic literature reviews and network meta-analyses within EU HTA regulation timelines, integrating innovative artificial intelligence methodologies while exploring HTA authorities' perceptions to optimize research outcomes and stay at the forefront of technological advancements.

Stakeholder Engagement

3

PICOS Scoping With External Stakeholders

OPEN Health collaborates with external stakeholders, including patients and clinicians at national and European levels, to define and refine their role in informing PICOS scoping. Using the Delphi panel methodology, especially with European patient organizations, we aim to propose transparent and inclusive methods for scoping.

Stakeholder Engagement

4

Patient Engagement in HTA

OPEN Health is advocating for meaningful patient engagement in the EU HTA process by actively identifying opportunities for patient involvement, ensuring that their perspectives shape assessments, and establishing ongoing dialogue with advocacy groups to foster a collaborative environment that prioritizes patient input.

National Perceptions

5

National Perspectives and Expertise

OPEN Health is conducting targeted research in key EU countries, including Germany, France, Italy, and the BeNeLux region, to tailor approaches to specific national contexts and optimize its HTA strategy by collaborating with local experts and stakeholders to incorporate diverse regulatory perspectives and expertise.

For more information, please contact us to be connected with our experts.

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OPEN Health unites deep scientific knowledge with wide-ranging specialist expertise to unlock possibilities that improve health outcomes and patient wellbeing. We are a flexible global organization that solves complex healthcare challenges across consulting, HEOR and market access, scientific communications, patient engagement, and creative omnichannel communications.

Contact us