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European Collaboration on Health Technology Assessment (HTA): The New Road to Access

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Health Technology Assessment (HTA) has long played a pivotal role in shaping healthcare systems worldwide. HTA examines the implications and applications of health interventions, including medications, medical devices, diagnostic tools, and even health system organizational changes. By evaluating the social, economic, organizational, and ethical issues of a health intervention or technology, HTA helps inform policy decisions, ensuring they are effective, efficient, patient-centered, and safe.

In the European Union (EU), the desire for a more cohesive and consistent HTA framework led to the formulation of the EU HTA Regulation 2021/2282 (HTAR). The regulation was adopted in December 2021, became effective in January 2022, and has an implementation timeline extending to January 2030. With much time on paper but little to waste in preparation, here's what health technology developers (HTDs) need to know right now.

WHAT DOES THE HTAR AIM TO DO?

The EU's HTAR is fueled by a set of ambitious yet pragmatic objectives. At the forefront is the aspiration to enhance access to medicines for all patients across the member states, ensuring geography doesn't dictate the quality of healthcare. Collaboration is another cornerstone, as the regulation seeks to foster a united front among Member States, propelling a coordinated HTA approach at the European level.

The HTAR also aims to streamline processes and reduce bureaucratic redundancies by mandating that evidence for joint clinical assessments be submitted just once. This not only ensures efficiency but also harmonizes methodological standards across the board. In markets that historically grapple with minimal or even non-existent HTA infrastructure, the regulation paves the way to simplify the reimbursement process, making life-changing treatments more accessible and affordable.

MAIN AREAS OF HTAR COLLABORATION

A major component of the EU's HTAR is the introduction of Joint Clinical Assessments (JCA). These assessments focus primarily on medicinal products that have received central marketing authorization. Additionally, JCA also encompasses selected high-risk medical devices and In Vitro Diagnostics (IVDs). JCA is crucial in ensuring new treatments and tools entering the market are both safe and effective for European citizens.

Joint Scientific Consultations (JSCs), previously often referred to as "early dialogues," are another key focus area. These consultations offer invaluable scientific advice to HTDs, guiding aspects like clinical trial design and evidence generation. JSCs can occur exclusively within the realm of HTA or run parallel with regulators, emphasizing the collaborative nature of the HTAR.

As the HTAR also underscores the identification of emerging health technologies, it provides a clear path for the future of healthcare in the EU. Lastly, while not mandatory, there's an avenue for voluntary cooperation in other areas, allowing Member States to collaborate further on diverse health technologies and non-clinical HTA aspects.

A CLOSER LOOK AT JCA COLLABORATION

The essence of JCA lies in its comparative approach. By comparing a health technology's clinical evidence with that of other health technologies or existing procedures, the assessment offers a holistic understanding of its potential impact on patient care.

JCA is underpinned by collaboration, with member states pooling their expertise to address the scientific and clinical facets of HTA. Close collaboration not only fortifies the quality of the reports but also guarantees their timely dissemination for national implementation. While the assessments are joint, the responsibility of deducing its relevance remains with individual member states. Their HTA bodies continue to helm the crucial tasks of appraising the technology's added value to their respective health systems and, subsequently, making decisions on pricing and reimbursement.

HTAR JCA development is currently in the preparatory phase until January 2025, when implementation will begin. It will reach its full scope in January 2030. JSC development mirrors the same timeline. The JCA process can be nuanced and slow-moving, which is why it's important for HTDs to proactively prepare during this time.

CHALLENGES AND POTENTIAL IMPLICATIONS

The path to HTAR compliance is filled with both opportunities and intricacies, especially when it comes to the JCA process. Once the Population, Intervention, Comparator, Outcome (PICO) is received, an HTD might find themselves with a window of less

than three months to gather a comprehensive evidence dossier. Such a tight timeframe, combined with a vast scope that covers various national requirements and comparisons to multiple comparators, demands significant analytical capabilities, resource allocation, and strategic forethought.

Furthermore, HTDs, despite their expertise in the disease area, evidence base, and treatment practices, seem to be sidelined during the scoping process. They now face the intricate task of harmonizing the EU-level and national submission processes and their respective requirements. As all stakeholders navigate this, they must simultaneously cater to both national HTA and JCA processes.

JSC is anticipated to become a pivotal phase of the clinical development journey in its own right. While HTDs have the option to apply for Multi HTA or Multi HTA/EMA parallel scientific consultation on evidence generation, the selection process is based on predefined criteria due to limited availability. This scarcity of JSC opportunities is a cause of concern for HTDs.

When it comes to evidence, the gold standard remains Randomized Controlled Trials (RCTs), with single-arm trials often perceived as offering limited value. The acceptance of indirect treatment comparisons (ITCs)/Network meta-analysis (NMA) endpoints, especially those prioritizing patient-centered outcomes, will be vital. However, the acceptability of a particular outcome is at the discretion of Member States and may vary within their national decision-making procedures. Current EUnetHTA 21 guidance documents shed light on some specific considerations. Intermediate or surrogate endpoints are deemed acceptable when measuring a final outcome is not viable, provided there's strong evidence linking the surrogate to the final outcome. Additionally, data from single-arm trials can be juxtaposed with an external control arm for comparative statistical analysis.

KEY CONSIDERATIONS

As HTDs find themselves navigating the intricate maze of the HTAR, there are several proactive measures that can be taken to ensure compliance and optimize product outcomes.

Strategy and clinical development can be a great starting point. This involves reviewing the portfolio for products slated for JCA in 2025 or 2028, which will enable you to intertwine anticipated JCA outcomes within national access strategies. Embracing EU HTA guidelines in forthcoming clinical development plans is pivotal. Whenever appropriate, HTDs should aim for JSC, as this offers invaluable insights and guidance.

Success in this new environment calls for ongoing collaboration across all stakeholder channels, ranging from market access/HTA and regulatory to clinical, biostats, legal, and affiliates. It's imperative to clearly define roles and ensure alignment among stakeholders to adeptly handle critical timelines, especially during simultaneous regulatory and product launch endeavors.

Additionally, HTDs must remain vigilant in identifying potential adjustments to national submission workflows to harmonize EU HTA with national submissions. And while the future looks promising, a pragmatic approach would also involve parallel "traditional" national submissions for products that aren't immediately impacted by the new regulations.

Anticipating the intricacies of potential PICO, subgroup analyses, and multiple ITCs even before the scope is finalized can set HTDs on the path of preparedness. Aligning the timing of the necessary analyses, Systematic Literature Reviews (SLR), and HTD submission dossiers with regulatory timelines is crucial. Equally vital is ensuring ample HTA, SLR, and biostatistical expertise and capacity are at the ready.

Meanwhile, the EU has pressing priorities as well, including increasing joint scientific consultation options; allowing for integration of input from all stakeholders including HTDs along the process as well as creating opportunities for meetings (e.g., in scoping phase); developing the JCA toward a more streamlined and truly joint European assessment; and using the post implementation process review effectively for continuous refinements.

As the horizon of healthcare in the EU continues to evolve, it's clear that early engagement in the HTA space is not just advantageous but essential. Contact Certara to learn more.

About Certara

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