Strategic Implications of EU HTAR (JCA and JSC) for Global Biopharma and Biotech: Navigating Evolving Paradigms in Market Access

HTA68

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Background

- The Health Technology Assessment Regulation (HTAR) in the European Union (EU), encompassing Joint Clinical Assessment (JCA) and Joint Scientific Consultation (JSC), introduces a unified framework for evaluating the clinical value of medicinal products (1)
- All new medicines will come under the scope of the regulation in January 2030, with the process already initiated for oncology drugs and advanced therapy medicinal products (ATMPs) (1)
- A structured population, intervention, comparator, outcome (PICO) approach is central to that framework (1,2)

Objective

- The objective of this research was to define strategic approaches for Global Biopharma/Biotech to ensure compliance with HTAR requirements and enable timely patient access to innovative drugs in the EU

Methods

- A review of current JCA/JSC requirements and guidelines, coupled with secondary desk research, was conducted to assess implications for internal Biopharma/Biotech processes and cross-functional collaboration
- The potential for leveraging real-world evidence (RWE) to inform future PICO criteria and enhance stakeholder communication was also explored

Abbreviations:

ATMPs, Advanced Therapy Medicinal Products; **CDP**, Clinical Development Program; **EC**, European Commission; **EGP**, Evidence Generation Plan; **EMA**, European Medicines Agency; **EU**, European Union; **HTA**, Health Technology Assessment; **HTACG**, HTA Coordination Group; **HTAR**, HTA Regulation; **HTD**, Health Technology Developer; **ITC**, Indirect Treatment Comparison; **JCA**, Joint Clinical Assessment; **JSC**, Joint Scientific Advice; **LR**, literature review; **MAA**, Marketing Authorisation Application; **MS**, Member State; **PICO**, Population, Intervention, Comparator, Outcome; **PRO**, Patient-Reported Outcome; **RWD**, Real-World Data; **RWE**, Real-World Evidence; **SAP**, statistical analyses plan; **SBU**, Swedish Agency for Health Technology Assessment and Assessment of Social Services; **SLR**, Systematic Literature Review; **TLR**, Targeted Literature Review; **TLV**, The Swedish Dental and Pharmaceutical Benefits Agency

Results

Joint scientific consultation (JSC)

- JSC, a **voluntary process** under the HTAR, that encourages early dialogue between health technology developers (HTDs) and Member States (MS), could play an essential role in shaping clinical study design that meets JCA evidence requirements (3,4)
- **Early engagement—ideally before pivotal trial** (Phase II/III)—is strongly recommended to align on key clinical aspects (PICO) and ensure pivotal trial generates robust data, particularly in areas where there is no established precedent in the field. Input on economic aspects to support national HTA submissions is also possible (4,5)
- However, **capacity for JSCs remains constrained**, with only around 10 expected in 2025—5 to 7 for medicinal products and 1 to 3 for medical devices (6)
- Because only a few products are expected to be accepted for JSCs in the early years, companies should establish a **fallback plan** – such as seeking **national HTA scientific advice** – for instance, TLV and the SBU offer guidance on designing clinical trials to meet JCA and reimbursement requirements (7) – or conducting "mock" or "proxy" JSCs (e.g., via payer advisory boards) to anticipate likely research questions and PICOs

Joint clinical assessment (JCA)

Early anticipation of PICO scoping is a critical step in aligning evidence generation for JCA with strategic objectives requiring a streamlined internal process **(Figure 1).**

- Health Technology Developer (HTD) will have **only 100 days after final PICO scoping** to submit the JCA dossier (60 days for accelerated procedure or type II variation) (8) and **must justify any deviations from final PICOs** (2)
- **PICO simulation** should guide the design of an **optimal clinical development plan**:
  - PICO simulation should reflect the perspectives of key markets that are **representative of the EU-27 Member States (MS)**, encompass major payer archetypes, and hold significant influence in the JCA process
  - PICO should be prioritized based on their relevance to strategic objectives and the feasibility of addressing them, considering technical, timeline, and resource constraints
- Anticipating PICO scoping is essential to identify potential evidence gaps early and proactively plan for tailored evidence generation (**Table 1**)
- However, PICO should serve to refine – not dictate – the development strategy. Begin by defining an aspirational value proposition and exploring key scenarios to maximize overall product value

Figure 1. Illustrative streamlined EU HTA process to ensure optimal JCA

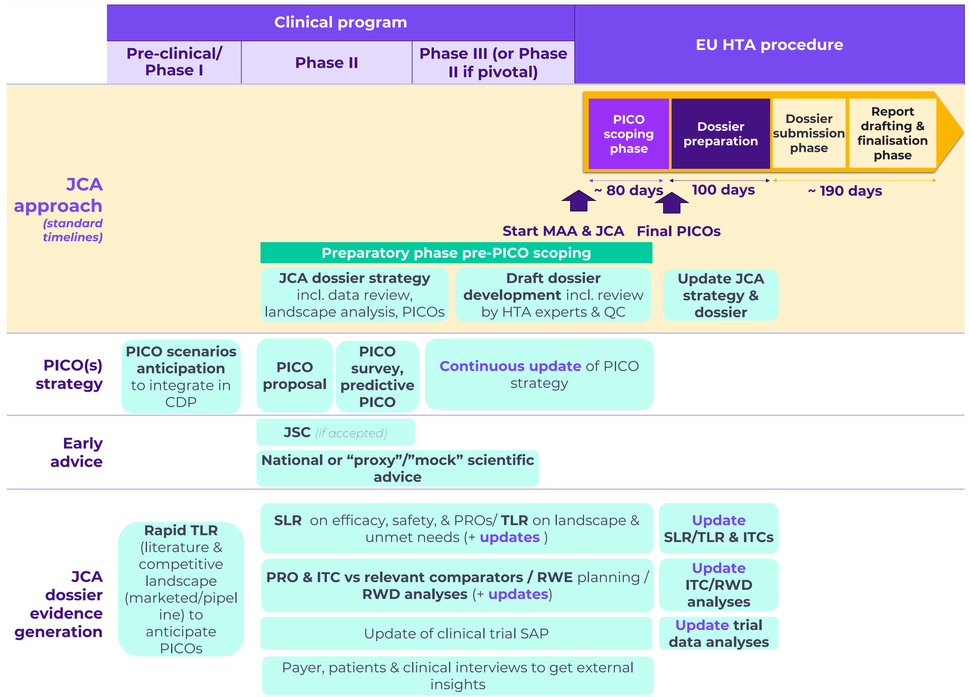

Table 1. Evidence considerations: Strategy and planning components to meet JCA requirements

Type of evidence	Relevance
Systematic/target literature review (SLR/TLR)	• Develop a comprehensive, adaptable TLR (covering the disease and competitive landscape) and SLR (encompassing clinical efficacy, safety, and patient-reported outcomes) to enable quick post-scope adjustments (if required)
Patient-reported outcomes (PROs)	• Include PROs in early-phase CDPs to capture patient-centric benefits in pivotal trial, since included within JCA scope (9), to generate robust data aligned with expected PICO scenarios
Indirect treatment comparisons (ITC)	• Plan ITCs against relevant comparators early—even for broader scopes—to align with multiple national HTA requirements and to allow for swift post-scope adjustments • This approach also ensures that work done for JCA submission can be effectively leveraged in national submissions
Real-world data/evidence (RWD/RWE)	• Identify relevant RWD sources early to accommodate multiple potential PICOs, with early data analysis cleaning • Gain understanding of local populations, treatment patterns (e.g., comparators in use), and evolving market needs • Where trial data are limited, consider cautiously leveraging RWE to construct external comparator arms within ITCs
Expert input	• Engage early with HTA experts, clinicians, and patients to inform evidence generation and PICO selection

Conclusions

- While not binding, JCA reports will potentially shape national HTA decisions, especially in less mature systems. While MS retain authority over final benefit evaluations, JCAs are expected to increasingly shape local evidence expectations and, ideally, reduce duplication.
- It is crucial that companies leverage insights from upcoming evaluations to support internal teams and prepare for EU HTA submissions.
- To prepare for JCA and meet demanding timelines and enable optimal patient access to innovative drugs, early cross-functional alignment is essential. Global biopharma/biotech should facilitate harmonization across regulatory, clinical, HEOR/RWE, and market access strategies while adapting internal team structures, roles, and planning processes.

References:

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