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Implementation of the New European Health Technology Assessment Regulation (HTAR)

The main challenges in the preparation of the new European Health Technology Assessment Regulation (HTAR) and how ProPharma can help you succeed with your product launch in Europe.

In 2021, the adoption of the legislation for joint European Health Technology Assessment (EU HTA) initiated a new era for HTA assessment at a European level and is potentially the biggest change in regulating European market access in the last ten years. This European Health Technology Assessment Regulation (HTAR) has already come into force in January 2025 for some of the health technologies.

Background & Timeline for Implementation

The HTAR aims to enable a joint perspective among EU member states on clinical HTA aspects in the development of medicinal products, in vitro diagnostics (IVDs), and high-risk medical devices. The HTAR incorporates Joint Clinical Assessments (JCA), Joint Scientific Consultations (JSC), horizon scanning activities, and voluntary cooperation. The horizon scanning activities involves work performed by the European Commission (EC) to allow the early identification of emerging health technologies that are likely to have a major impact on patients, public health, and healthcare systems. The voluntary cooperation is a collaboration between the EU member states in areas such as the development and implementation of vaccination programs and the capacity building of national HTA systems. Both these activities are outside the scope of the mandatory aspects of the HTAR. Therefore, this white paper will focus on the JCA and JSC, which are crucial components for all companies who plan to submit their Market Authorization Application (MAA) to the European Medicines Agency (EMA).

As of 2025, companies with products indicated for the treatment of cancer and/or an Advanced Therapy Medicinal Product (ATMP) are now required to prepare and submit a JCA dossier.

The HTAR has made it a requirement for companies to submit a JCA to the EC in parallel to their MAA filing to EMA. The JCA consists of a clinical, epidemiological, and safety section, and will be published by the EC. Subsequently, the JCA will be part of the HTA decision-making at the national level.

The Time to Begin Preparing for HTAR Implementation Is Now

The implementation of the HTAR will be a staggered process. As of January 2025, companies with products in the setting of cancer and/or as an Advanced Therapy Medicinal Product (ATMP) will be required to prepare and submit a JCA dossier. Next in line will be orphan drugs, planned for 2028, and all remaining drugs, as of 2030.

Currently there are significant concerns remaining around the process. The major worry among companies is that the increased time and resource needs will hinder European patients from gaining access to new health technologies.

Potential Challenges & Solutions Associated with HTAR Compliance

Concerns Around the EU HTA Process

While companies are aware that the EU HTA process is changing, significant concerns remain surrounding the process for placing innovative health technologies on the market in the EU. For example, approximately two months after the submission of an MAA to the EMA, each member state will define their PICO (Population, Intervention, Comparator(s) and clinical Outcome(s)) framework for the JCA. In this regard, it is still not specified how the (number of) target populations and comparators from the different member states (MSs) will be determined for the JCA preparation. Therefore, it is also challenging to estimate the amount of additional work the HTAR will require from the companies.

The EU HTA Coordination Group is comprised of four different Subgroups. These include: the JCA Subgroup, the JSC Subgroup, the Methodology Subgroup, and the Identification of emerging health technologies Subgroup. The JCA Subgroup consists of representatives from the member states and is directly responsible for the delivery of the JCA reports. The assigned assessor and co-assessor of the JCA subgroup will compile all the different PICO frameworks and present it in a consolidated scoping study that will be shared with the rest of the members of the JCA Subgroup. The consolidated scoping study with the different PICO frameworks are then presented to the companies and the companies will have 100 days to develop their JCA dossier.

In summary, the JCA steps from the Implementing Act include:

- **Start:** JCA process starts when the MAA dossier is submitted to EMA
- EU HTA Coordination Group appoints a Subgroup
- Subgroup appoints assessor and co-assessor from 2 different member states
- PICO frameworks are outlined with involvement of 27 member states
- A consolidated scoping study with the final PICO frameworks are presented to the company
- JCA dossier preparation by the company (100 days)
- JCA dossier submitted by the company 45 days before CHMP opinion
- JCA dossier evaluated by the assessors
- Input stakeholder network (optional and based on availability)
- EU HTA Coordination Group approves the JCA dossier
- Report to the EU HTA Coordination Group
- **End:** Publication by the European Commission < 30 days since the European Product Assessment Report (EPAR) has been approved by EMA

More details on the timelines for this process are available in Figure 1, below.

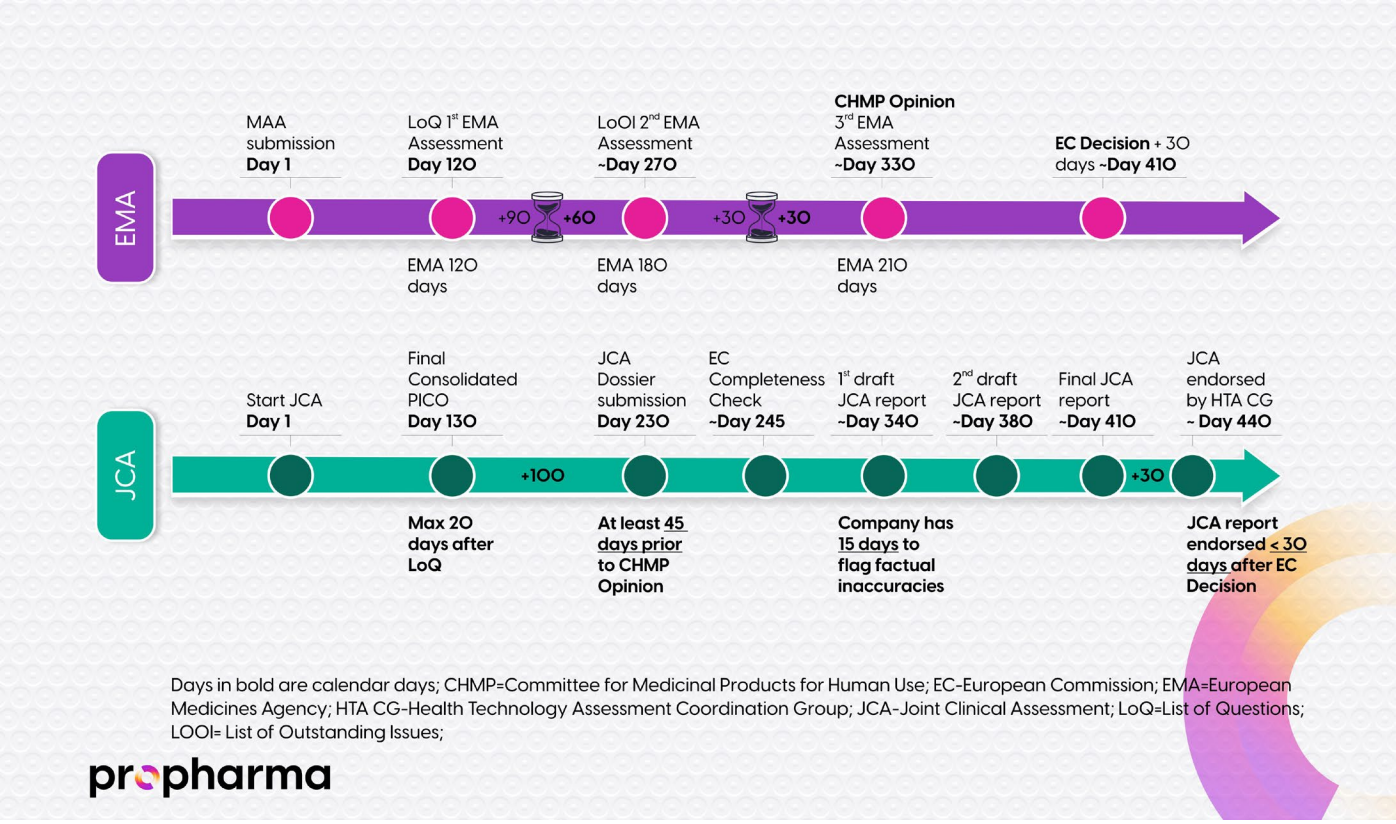

Figure 1: Timelines for the parallel JCA and EMA process

Concerns of an Increase in Time and Resources Rather Than Faster Patient Access

The aim of a JCA is to enable a joint perspective on clinical aspects of the development of health technologies, accelerating the process for patient access, and ensuring consistency in assessing new health technologies. However, many predict the HTAR process will be more time and resource consuming due to the early timing of the JCA relative to the MAA, the very challenging procedural timelines, and the opportunity for the JCA Subgroup to request that additional information is provided at almost any time during the process.

Complex Requirements and Short Timelines for Compliance

After the consolidated scoping study with the PICO frameworks are presented to the companies, organizations will have only 100 days to develop their JCA dossier by incorporating safety, clinical, and epidemiological data through performance of systematic literature reviews (SLRs) and indirect treatment comparisons (ITCs) following each PICO framework. The published template of the JCA report is extensive. Although the national specific HTA sections, such as health economic modeling and relative costs-effectiveness, are not covered by the JCA, the JCA preparation still requires complex evidence synthesis and comprehensive SLRs which are time consuming and not something that is normally achieved within a 100-day timeframe.

In addition to these proposed tight deadlines, the consolidated scoping study with PICOs might be subject to change by the JCA Subgroup's appointed assessor with the co-assistance of the co-assessor. For example, this can occur when there is a change to the therapeutic indication(s) initially submitted to the EMA. It is up to the assessors to decide whether that change affects the scope and then inform the JCA Subgroup. These factors add to the complexity of the process and may be expected to substantially impact the progress of the procedure.

Price setting, health economics, and full reimbursement in each market is still performed at a national level and usually commences following the Committee for Medicinal Products for Human Use's (CHMP) opinion. For transparency, each MS is obligated to report how the JCA was used in national decision making and is not allowed to redo/require analyses already evaluated in the JCA.

Here there is still a risk that data from the JCA are not detailed or accurate enough to allow for national decision-making due to further country-specific HTA requirements on each national level. Some examples include national guidelines that will impact selected target populations and comparators, which may vary from country to country based on factors such as demographic differences. Moreover, clinical care may be very different among the EU member states depending on national choices for reimbursement.

Furthermore, indirect treatment comparisons on subgroups will still need to be tailored to the specifics of the MSs that have different requirements for the health economic aspect of national HTA decision-making. The scoping study with PICO frameworks that represent all European healthcare systems are therefore a main challenge. There might still be countries which will aim to maintain their own assessments where additional national HTA dossiers with country-specific demands will be expected.

To overcome these issues, ProPharma has a multidisciplinary scientific team of European regulatory and HTA consultants available with strong scientific skills and extensive experience in each national clinical and health economic requirements. As such, we are uniquely positioned to guide you through the process and support you with the strategic planning and preparatory steps for the regulatory MAA submission to EMA jointly with the JCA. These steps are based on what the HTAR requires from the companies in the new procedure and will help you create a successful market access plan in Europe.

Path to Successful European Product Launch

Although the HTAR implementation is already underway for some health technologies, we have observed that only a small number of companies have started to consider the organizational changes that may be needed at an EU or global level to meet the demands of the JCA.

ProPharma can help ensure your company is ready for the requirements by offering you the following four step approach to a successful product launch. These four steps are based on what the HTAR requires from the companies in the JCA procedure.

Step 1: Introductory Training on HTAR Rules & Implications

To begin the process, an introductory training on the HTAR process and its implications is strongly recommended. The trainer will be fully versed and has a complete understanding of the entire process as well as the consequences of noncompliance.

Step 2: Conduct a Technology-Specific Scoping Study

As part of the HTAR process, a scoping study will be conducted by the JCA Subgroup with input from all the EU Member States. As timelines will be short, it is important to be well prepared for the step following receipt of the scoping study by conducting well ahead a technology-specific scoping study for your product yourself and, based on that, outline a plan for the JCA, including defining the PICOs, before the clock starts.

Step 3: Workshop Preparation to Define Optimal PICO(s)

The finalization of the technology-specific scoping study with PICOs and plan for the JCA dossier development will be arranged in more detail as part of this workshop and next steps will be discussed and agreed.

Step 4: Implementation of the Plan

For the final step in the JCA development, the PICO frameworks, as defined by the technology-specific scoping study and the workshop, will be applied, resources added as needed and timelines will be adhered as outlined in Steps 2 and 3.

We support our clients by offering our unique strength in combining our regulatory and market access expertise to optimize a fast and efficient product launch and patient access. Our staff of experienced subject matter experts, including former regulatory and HTA agency employees, are well-informed on the latest evolutions of market access and HTA regulations.

The Value of Joint Scientific Consultations

Early and coordinated advice can enhance the chances of obtaining favorable pricing and reimbursement outcomes, facilitating quicker market access. Joint scientific consultations are an opportunity to engage with HTA bodies (HTAb) representatives either alone or jointly with EMA representatives; this has been implemented alongside the JCA in January 2025. By providing integrated feedback from both EMA and HTAb will help HTDs align their development plans with the requirements for marketing authorization and reimbursement.

To qualify for a JSC, the medical product must be likely to undergo a JCA, the request for JSCs must be submitted during designated periods specified in the Annual Work Program of the HTA Coordination Group (HTACG), and the request must include all necessary information and evidence to support the consultation, ensuring it aligns with the requirements for a subsequent JCA. Eligibility criteria and selection criteria are due to number of slots and resources. The JSC Subgroup then selects the JSC and decides.

ProPharma's Regulatory Sciences team is comprised of experts who can support you with the preparation and participation in the JSCs. Joint scientific consultations are strongly recommended to discuss any issues of concerns from EMA and HTAb to ensure that company's PICO and trial designs are accurate to facilitate the development plans.

Expert European Regulatory Consultants with Extensive HTAR Knowledge

Apart from our immediate capability to support you with the preparation steps for the HTAR implementation, we can also help you with anything from early regulatory and HTA interactions, to full MAA support to EMA, JCA preparations to the EC, and national HTA preparations and submissions. Our unique ability to support your company throughout the full product life cycle enables us to help you achieve a successful product launch across the EU and beyond. Contact us today to learn more.