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The EU Regulation on HTA: Developing an Adapted Market Access Framework for Pharmaceutical Assets

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INTRODUCTION

The EU Regulation on Health Technology Assessment (HTA) entered into force in January 2022 and applies in 2025

- The new framework covers joint clinical assessments (JCA) across Member States (MS) to avoid duplicative effort. JCAs are non-binding, compilation of clinical evidence that must be given due consideration by Member States (MS) and should be part of the documentation that supports national HTA activities
- JCAs will start with a small number of health technologies and will be progressively expanded
  - January 2025 – medicinal products with new active substances for the treatment of cancer, and those regulated as advanced therapy medicinal products (ATMP)
  - January 2028 – orphan medicinal products
  - January 2030 – all other medicinal products
- EUnetHTA has published deliverables under the Third EU Health Programme under the mandate from the European Commission. The deliverables serve as a basis for the development of guidance documents to be adopted by the HTA coordination group (HTACG) and/or drafting of implementing legislation by the European Commission

OBJECTIVE

Although the operating procedures are still being developed by the HTACG, this study aims to analyze the regulation and its presumable impact on manufacturers' preparation, and present an adapted Market Access framework including core activities to ensure commercial success considering price and reimbursement decisions remain under the remit of MS

METHODS

- We analyzed the current provisions and gaps of the EU HTA regulation including the deliverable documents published by EUnetHTA21, press releases and grey literature, to assess the implications on Market Access activities from a manufacturer's perspective
- The analysis focused on new chemical entities and did not take into consideration new indications of already approved drugs (Type II variations) and cases of accelerated approvals
- The conclusions drawn are based on the authors experience in advising manufacturers on pricing, reimbursement in Europe

RESULTS AND DISCUSSION

Gaps still exist in the procedure:

- Formal operating procedures of the Joint Scientific Consultations (JSC) process, and if and how an early population, intervention, comparator, outcomes (PICO) scoping process can be integrated in these early consultations
- Clear timeframe of JCA PICO scoping process and how it may be impacted if deviations between the claimed indication and the approved indication exist. As the scoping process happens prior to EMA approval, the final approved indication may influence PICO question(s)
- How potential deviations from the standard EMA timelines e.g., when clock stop durations are longer than planned, may impact the JCA process and how to proceed if this happens
- Clear understanding of the potential number of PICOs that may be required, and how it may impact the manufacturer's ability to prepare the dossier within tight timeframes, and how potential delays may spillover to MS HTA processes
- The formal template of the JCA as endorsed by the HTACG
- Timeframes of the publication of the final report
- Integration of JCA to MS HTA processes

When mapping the process with informed assumptions, below is an adapted Market Access framework including core activities per clinical development phase:

Phase 2 (Figure 1)

- A critical analysis of the landscape informs the early Market Access Strategy and the potential PICOs relevant in key markets. This feeds the design of the pivotal Ph 3 trial
- Manufacturers should potentially request a JSC at Ph 2, parallel to an Early Scientific Advice at the national level if needed, to validate PICO scope at both EU and national levels. Insights help finalise the Ph 3 trial design and the JCA process down the line
- Early economic model for early analysis of initial pricing scenarios and may support go and no-go decision for Ph 3

Phase 3 (Figure 1)

- While Ph 3 is ongoing, RWE generation to gain insight on specific research questions and business issues, support the value proposition, and feed the economic model and the Global Value Dossier
- Health economic models to support pricing and reimbursement negotiations with payers

1 year before Marketing Authorisation (Figure 1)

- The year following EMA regulatory submission is dense with activities
- Once Ph 3 trial results become available, launch preparations accelerate. In addition to EMA submission preparations, an updated landscape analysis assesses any changes that may impact the Market Access strategy and enables finalization of the pricing and global launch sequence strategies. Decisions at this point may have PICO implications
- Per EUnetHTA estimates, JCA procedure will start 2 months after EMA submission and PICO scoping process lasts 3 months
  - A scoping process will be conducted by a Coordination Group where it identifies PICO parameters reflecting needs across MS. Based on this exercise, the manufacturer will be informed of the PICO scope thus the data needed for the dossier
- Although the regulation states that the deadline for dossier submission is at the latest 45 days prior to the envisaged date of CHMP opinion, the manufacturer realistically will only have ~3 months to finalize their dossier which is tight
- An additional 3 months after submission is foreseen to assess the completeness of the dossier and additional data may be required from the manufacturer
- Per regulations, the JCA draft by will be published by the Coordination Group no later than 30 days following EMA marketing authorisation approval (MAA)
- Parallel to the whole JCA process, finalization of the value communication tools and preparation of national HTA dossiers, where possible, must run in the same period after regulatory submission
- Timelines may become shorter as the new EU Pharma Strategy also proposes the reduction of authorisation time of new medicines

Post- Marketing Authorisation Approval

- Much is unknown following the publication of the JCA draft following EMA Marketing Authorisation Approval (MAA)
- The timeframes for the review of the draft by stakeholders, the needed revisions, finalization, and publication of the final report by the European Commission is currently unknown
- As such the submission process and timeframes for national HTA dossiers based on the launch sequence strategy is unclear as the process of integration of JCA to national HTA processes are not yet formalized

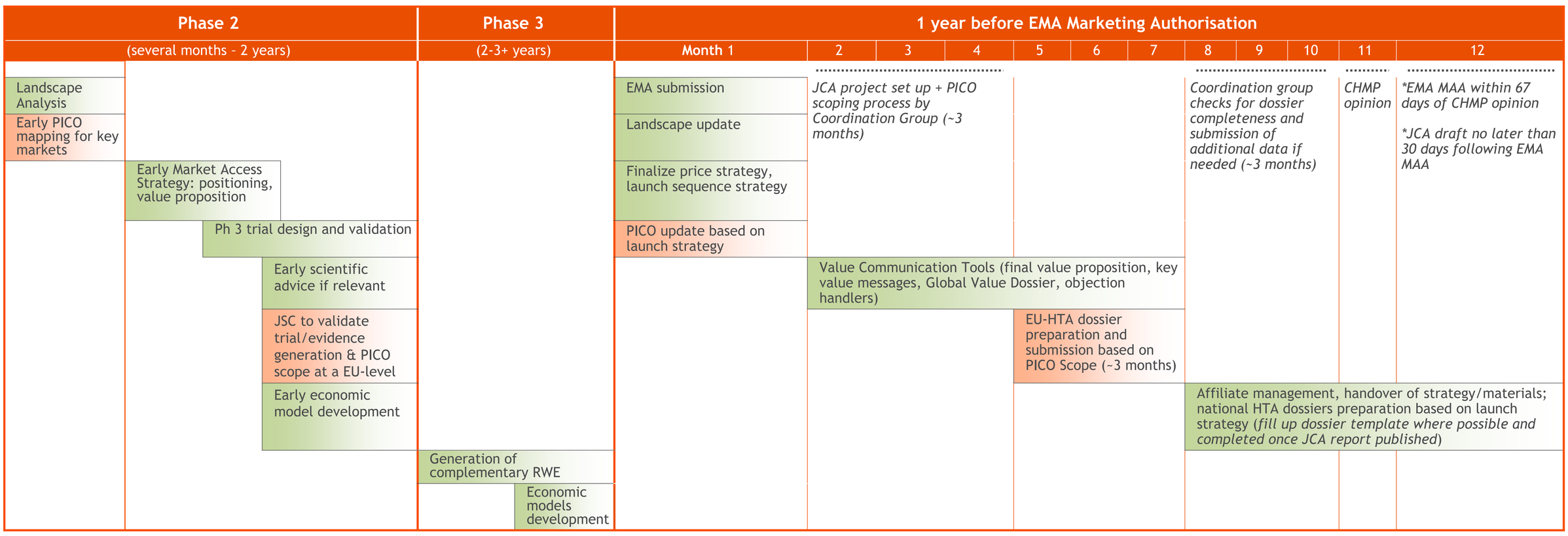

Figure 1. Phase 2 to Phase 3 Market Access activities and Market Access activities one year prior to MAA

CONCLUSIONS

- The EU-HTA regulation condenses core activities in a shorter timeframe and additional activities are imperative for pharmaceutical launch in EU. Our analysis presents an initial adapted Market Access framework pending more guidance being released in the coming months. Timelines may become shorter as the new EU Pharma Strategy also proposes the reduction of authorisation time of new medicines
- To avoid challenges and delays in the assessment, an early coordination with the HTA coordination group through the JSC may provide an opportunity to validate PICO scope at an early clinical development stage. ATMP manufacturers must also ensure that they have the necessary resources to compile the required evidence in a short amount of time. Filling the JCA dossier template and national HTA dossiers as early as possible e.g., disease and product-specific information, will save time. Adaptation and completion may follow once PICO specifications and the final JCA report are provided, respectively

References

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