# WORKSHOP SYNOPSIS

## Facilitating joint clinical assessment (JCA) implementation, utilisation and timely patient access

*Considerations and measures to assess efficiency and effectiveness of the process to enable iterative learnings among stakeholders*

**14th June 2024**
**Barceló Sevilla Renacimiento,**
**Seville, Spain**

CIRS Centre for Innovation in Regulatory Science

# INFOGRAPHIC SUMMARY

**CIRS brought together HTA agencies, pharmaceutical companies, payers and patient organisations** to discuss preparations for the **HTA Regulation** and to make recommendations on ensuring efficient and effective implementation of **joint clinical assessment (JCA)** at the national level.

## Recommendations to ensure efficiency of the process from JCA to national HTA decision making:

- Develop a **metrics** framework to evaluate efficiency
- Establish **training** programmes to support Member States
- Facilitate **information sharing** and learning amongst Member States
- Develop **open-source tools** to reduce duplication across industry

## Recommendations to ensure effectiveness of the JCA:

- Conduct **research** on how agencies will use the JCA report in decision making
- Enhance **communication** between stakeholders
- Identify **metrics** on the value of joint scientific consultation to agencies
- Develop a product-based **scorecard** to capture stakeholder perceptions on the JCA assessment process and quality of the company submission

## Background

The Regulation (EU) 2021/2282 on health technology assessment (HTAR) reflects a significant step towards harmonising the clinical assessment in HTA decision making across EU Member States. It aims to improve the availability of innovative health technologies for EU patients by strengthening the quality of HTA across the EU and reducing duplication of effort for national HTA agencies and industry. The new HTAR framework covers joint clinical assessment (JCA), joint scientific consultation (JSC), the identification of emerging health technologies and voluntary cooperation.

In preparation for the application of HTAR from January 2025, an HTA Coordination Group and four subgroups have been established to develop methodological and procedural guidance, based on the work of EUnetHTA21. The first implementing act - adopted in May 2024 - sets out rules and templates for JCA of medicinal products for human use. However, there are still questions and concerns regarding the practical execution of the JCA report in national reimbursement decision making. It is also key that success measures or indicators are identified to allow for continuous learning and improvement as the JCA process evolves.

**In this workshop, CIRS brought together senior representatives from HTA agencies, pharmaceutical companies, payers and patient organisations to discuss their readiness for the EU HTA Regulation (HTAR) being applied from January 2025. The aim was to make recommendations on how to ensure efficient and effective implementation of the JCA at the national level and to identify metrics that will enable iterative learning among stakeholders.**

## Workshop sessions

This multi-stakeholder workshop consisted of a series of presentation sessions (see programme), a panel session and two parallel breakout discussions. The presentations provided agency and industry perspectives on preparations for the HTAR, as well as future considerations for involvement in JCA and national implementation at the Member State level.

The breakout groups were asked to discuss and develop recommendations on two topics:

- **Efficiency** of the process from JCA to national HTA decision making: Ensuring timely company submissions and efficient agency coordination
- **Effectiveness** of the JCA: Ensuring the value of JCA outputs to support national HTA decision making

# Key points from presentations and open-floor discussions

## Agencies are adapting but must remain flexible

HTA agencies are conducting various activities to prepare for JCA implementation, such as horizon scanning, workshops and training for assessors, and restructuring internal working environments e.g. IT platforms. They are planning to implement and maintain two parallel processes (JCA and non-JCA) from 2025 to 2030, due to the rolling plan for implementing the HTAR. Flexibility is key to seeing how changes informed by continuous learning can be implemented into JCA.

## Smaller HTA agencies are already benefitting

Being a member of the EU HTA Coordination Group and its subgroups has helped small HTA agencies, e.g. those in Eastern Europe, to build internal capacity and capability. For example, participating in joint PICO exercises has increased knowledge, skills and experience within some agencies. The impact of JCA on national assessment processes may not be as significant for maturing HTA agencies as for more mature agencies with very established procedures and methods. However, maturing agencies may face different challenges to implementing JCA, for example, fragmentation in their national reimbursement environment and barriers in legal frameworks.

## Short-term efforts for long-term gains

Capacity for JCA implementation is a key issue for both companies and agencies. For many agencies, resources are already limited and there can be difficulties recruiting the right experts. Companies are concerned about the additional internal workload, as evidence to satisfy all EU HTA processes is needed earlier, with an additional dossier developed in parallel to the regulatory filing. Teams such as Biostatistics/Data Science and Market Access are anticipated to be impacted the most. Nevertheless, there is shared hope that in the long run, JCA will reduce duplication and save resources for both companies and agencies, and ultimately accelerate access to innovative therapies for EU patients.

## Companies feel relatively ready but are concerned with uncertainty around JCA

Before the workshop, CIRS surveyed its member companies to gain collective insights into company readiness for the HTAR and help inform the workshop discussions. No companies indicated that they were completely unprepared for HTAR, yet none were fully ready either; most companies positioned themselves just over the midpoint of readiness scoring. Various actions related to processes, resources, policy/advocacy and pilots have been taken within companies to prepare for the HTAR. For example, most respondents had conducted assessments of pipeline products anticipated to undergo JCA in 2025 (92%), participated in HTA-related conferences, workshops and training (92%), and established an internal task force dedicated to HTAR (85%).

When asked about the impact of HTAR on regulatory strategy, all companies said EMA submission is likely to proceed as scheduled. Nevertheless, there were concerns about uncertainty in the JCA process and timelines, as well as timely delivery of the JCA report.

## Stakeholder communication is key

Proactive communication and involvement of stakeholders, such as industry associations, healthcare providers, patient organisations and payers, have been key to agencies' preparations for HTAR. However, not all stakeholders understand the decision-making relevance of the JCA report; the fact that the JCA report will have a different impact on different decisions in different countries needs to be better communicated to manage expectations. It would be valuable for stakeholders to come together in 2025 to have a 'safe harbour' discussion on early experiences of JCA and to share learnings.

## Uncertainty over patient involvement

There is uncertainty around when and how patients and patient organisations will be involved in the JCA process. This needs clarifying to ensure predictability and representative, meaningful patient contributions. National patient organisations vary greatly in terms of staffing, resources, knowledge and experience, so this needs to be addressed if the JCA requires scientifically based input from a patient perspective, for example, patient experience data to inform PICOs. Clear communication, transparency and continuous evaluation are key to shaping patient involvement in JCAs.

## What does success look like?

The definition of success of the HTAR varies and so may require greater alignment across stakeholders. The CIRS pre-workshop survey of international pharmaceutical companies showed that most companies believe that the success of the HTAR in the short term will be reflected by gaining positive recommendations in EU jurisdictions. In the long term, the focus shifts to achieving greater alignment in HTA methodologies and evidence requirements across the EU.

For HTA agencies, success can be viewed on a national and European level: nationally, it may mean gaining as much as possible from the JCA in order to reduce activities within the agency post-JCA. On a European level, success can be seen as establishing good HTA practices and providing consistency and predictability for companies on what evidence is needed from the HTA perspective.

From a patient perspective, short-term success of the HTAR is the recognition of patients as equal and trusted partners in the JCA. Increased quality of care and access to therapies for European patients could be long-term success measures.

## A learning journey, supported by metrics

All stakeholders support a collective 'learning-by-doing' approach towards the implementation of JCA; it is just as important to learn from mistakes as it is to learn from advances. While agencies are already learning from each other through the HTA Coordination Group and joint working, companies are also discussing the development of joint open-source tools and algorithms to facilitate information sharing and establish a common framework for analyses. This would be helpful to the assessors in HTA agencies as it would provide some confidence in the similarity of analyses.

Identifying metrics to assess the efficiency and effectiveness of the JCA is an essential step on the learning journey that is the HTAR. These metrics will allow for continuous learning and improvement as the JCA process evolves and will help to define success of the HTAR.

# Recommendations from breakout discussions

## Efficiency of the process from JCA to national HTA decision making

Recommendations for further work to enable timely company submissions and efficient agency coordination:

- Develop **metrics framework/scorecard** to evaluate efficiency and inform continuous improvement. The following measures should be considered:
  - Timing of the HTA submission: are the JCA and national agency reviewing the same relevant clinical package?
  - Dossier completeness: How many dossiers were considered incomplete, and how important was the missing information?
  - Appraisal time: Reduction in appraisal time, all covered in JCA, does not need additional effort locally
  - PICO consistency: Number of PICOs presented for each class of product
  - Patient involvement measures
  - Comparison of the national decision-making process for JCA vs non-JCA submissions
  - Extent of consideration of JCA at the national level
  - How has the dossier been assessed in terms of methodology and what evidence has been considered e.g. indirect comparisons
- Establish **training** programmes to support capability and capacity building for Member States.
- Facilitate **open dialogue** amongst Member States to share technology-specific learnings; what is needed through the JCA process and what are the considerations locally across Member States?
- Develop **open-source tools** to reduce duplication and build capacity across health technology developers e.g. statistical tools.

## Effectiveness of the JCA

Recommendations for further work to ensure the value of the JCA output and its effective utilisation in national decision making:

- Conduct **research** to understand what agencies expect from the JCA and how they will use the JCA report in their decision making. Agencies could be grouped by archetype to identify trends.
- Enhance **communication** between stakeholders: more frequent and earlier communication. This must help to set expectations on what the JCA is and is not.
- Identify **metrics** that can help HTA agencies to understand the value of JSC and enable iterative improvement.
- Develop a product-based **scorecard** to rate each submission from different stakeholder perspectives (industry, agency, patient etc). Did the submission include the information each stakeholder needed and how did they rate the process?

# Workshop Programme

**Workshop Chair: Dr Brian O'Rourke**, CIRS HTA Steering Committee

| **Session 1: Preparing for the EU HTA Regulation: Insights from agency perspectives** | **Session 2: Preparing for the EU HTA Regulation: Insights from company perspectives** |
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| **Dr Wim Goettsch**, Professor HTA, Utrecht University and Special Advisor HTA, National Health Care Institute (ZIN), The Netherlands | **Dr Tina Wang**, Senior Manager, HTA Programme and Strategic Partnerships, CIRS |
| **Dr Kamila Malinowska**, Director of the President's Office, Agency for Health Technology Assessment and Tariff System (AOMiT), Poland | **Dr Antonia Morga**, Senior Director, Global HEOR and HTA Strategy Lead, Astellas, UK |
| **Boryana Ivanova**, Head of HTA Department, National Council on Prices and Reimbursement of Medicinal Products, Bulgaria | **Dr Lara Wolfson**, Associate Vice President and Head of HTA Statistics, MSD, Switzerland |
| **Mihaela Popescu**, Physician in Health Technologies Department, National Agency for Medicines and Medical Devices, Romania | **Dr Aikaterini Fameli**, Global Head of Oncology and HTA Policy, GlaxoSmithKline, UK |

| **Session 3: Considerations and measures to assess efficiency and effectiveness** | |
|---|---|
| **Dr Neil McAuslane**, Director, CIRS | **Pedro Carrascal Rueda**, Executive Director, Patient Organizations Platform (POP), Spain |
| **Breakout A) Efficiency of the process from JCA to national HTA decision making**<br><br>**Chair: Dr Michael Berntgen**, Head of Scientific Evidence Generation Department, European Medicines Agency (EMA)<br><br>**Rapporteur:** Alison Davie, Senior Director HTA Oncology, Eli Lilly, UK | **Belén Torres**, Member of Joint Clinical Assessments and Identification of Emerging Health Technologies HTAR Subgroups, Agencia Española de Medicamentos y Productos Sanitarios (AEMPS), Spain |
| | **Dr Michael Ermisch**, Head of Department AMNOG G-BA, GKV-Spitzenverband, Germany |
| **Breakout B) Effectiveness of the JCA**<br><br>**Chair: Dr Anja Schiel,** Senior Assessor / Statistician, Norwegian Medical Products Agency (NOMA), Norway<br><br>**Rapporteur: Dr Thomas Butt**, Executive Director, Head of Global Health Economics & Outcomes Research, BioMarin, UK | **Dr Marc Van de Casteele**, Coordinator of the Pharmaceutical Experts, Department of Pharmaceutical Reimbursement, Belgian Health Care Institute RIZIV-INAMI, Belgium |
| | **Dr Vanessa Schaub**, Head of Global/EU HTA Strategy, Roche, Switzerland |
| | **Valentina Strammiello**, Director of Programmes, European Patients' Forum, Belgium |


**About CIRS**

The Centre for Innovation in Regulatory Science is a neutral, independent UK-based subsidiary of Clarivate plc. Its mission is to maintain a leadership role in identifying and applying scientific principles for the purpose of advancing regulatory and health technology assessment (HTA) policies and processes. CIRS provides an international forum for industry, regulators, HTA bodies and other healthcare stakeholders to meet, debate and develop regulatory and reimbursement policy through the innovative application of regulatory science. It is governed and operated by Clarivate for the sole support of its members' activities. The organisation has its own dedicated management and advisory boards, and its funding is derived from membership dues, related activities, and grants.

## Workshop organised by

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