WORKSHOP REPORT

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

Section 1: Executive Summary

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 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 objectives

- **Identify current process and procedures** of HTA agencies in Member States, and companies' local submission approach: What needs to be changed or in place to move from concept to practical implementation of JCA?
- **Discuss critical challenges and potential solutions** for implementing JCA in the national decision-making process: What is necessary to allow agencies to focus on value-added activities in order to provide timely patient availability building on the JCA?
- **Make recommendations on assessing the efficiency and effectiveness** of JCA: What research is required to assess the short- and long-term goals of JCA? What indicators are required to enable iterative learning among stakeholders?

Workshop format

This multi-stakeholder workshop consisted of a series of presentation sessions, a panel session and two parallel breakout discussions (see programme). 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, such as 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.

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, ultimately accelerating 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 company indicated that it was 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 considered 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 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 evaluate 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?

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

Workshop Programme

Please note, affiliations are stated as they were at the time of the meeting.

14th June 2024

Session 1: Preparing for the EU HTA Regulation: Insights from agency perspectives	
09:00	**Chair's welcome and introduction** **Dr Brian O'Rourke**, Chair, CIRS HTA Steering Committee
09:10	**Enhancing JCA preparedness: Internal exercises and shared learning from a national HTA agency** **Dr Wim Goettsch**, Professor HTA, Utrecht University and Special Advisor HTA, National Health Care Institute (ZIN), The Netherlands
09:25	**Enhancing capacity for JCA implementation in the context of national resource settings** **Dr Kamila Malinowska**, Director of the President's Office, Agency for Health Technology Assessment and Tariff System (AOMiT), Poland **Boryana Ivanova**, Head of HTA Department, National Council on Prices and Reimbursement of Medicinal Products, Bulgaria **Mihaela Popescu,** Physician in Health Technologies Department, National Agency for Medicines and Medical Devices, Romania
10:10	**Moderated discussion**
Session 2: Preparing for the EU HTA Regulation - Insights from company perspectives	
10:20	**Readiness of companies preparing for HTA regulation** CIRS pre-workshop survey feedback **Dr Tina Wang**, Senior Manager, HTA programme and Strategic Partnership, CIRS
10:35	**What does readiness look like for companies? How are companies adapting to the JCA What are the opportunities and key challenges faced internally? What is the pathway for national submission when JCA and national HTA submissions co/exists? What are the building blocks that companies need to consider for the transition?** 5 minutes reflection followed by discussion. **Dr Antonia Morga,** Senior Director, Global HEOR and HTA Strategy Lead, Astellas, UK **Dr Lara Wolfson**, Associate Vice President and Head of HTA Statistics, MSD, Switzerland **Dr Aikaterini Fameli,** Global Head of Oncology and HTA Policy, GlaxoSmithKline, UK
10.55	**Moderated discussion**
11.10	**Coffee break**
Session 3: Considerations and measures to assess efficiency and effectiveness	
11:40	**Overview of CIRS mapping and metrics projects and introduction to breakout discussions** **Dr Neil McAuslane,** Director, CIRS
11:55	**Breakout A: Focusing on efficiency** **Implementing JCA procedure within national process: Ensuring timely company submissions and efficient agency coordination** **Chair: Dr Michael Berntgen,** Head of Scientific Evidence Generation Department, EMA **Rapporteur: Alison Davie,** Senior Director HTA Oncology, Eli Lilly, UK

	Breakout B: Focusing on effectiveness **Ensuring the value of JCA output and optimising resource from agencies and companies: Practical strategies for effective utilisation within jurisdictional decision making** **Syndicate Chair: Dr Anja Schiel,** Senior Assessor / Statistician, NOMA, Norway **Rapporteur: Dr Thomas Butt**, Executive Director, Head of Global Health Economics & Outcomes Research, BioMarin, UK
13:00	**Lunch break**
14:00	**Continue breakout discussions and agree recommendations**
15:00	**Break**
15:30	**Feedback of breakout discussions and participants' viewpoint** **Breakout A rapporteur: Alison Davie,** Senior Director HTA Oncology, Eli Lilly, UK **Breakout B rapporteur: Dr Thomas Butt**, Executive Director, Head of Global Health Economics & Outcomes Research, BioMarin, UK
15:55	**Reflections on what should be the future considerations for involvement in JCA and national implementation at the Member State level:** • How should the local expert be involved (clinical, patient representatives) during the scoping process to maximise the value and fit of the JCA into the national context? • What are the challenges to maintain a balance in representatives of patients and clinicians across different member states? • How can patient groups and clinicians maximise opportunities to enhance the implementation of JCA and ensure effective utilisation of the outcomes in national decision making? **Pedro Carrascal Rueda,** Executive Director, Patient Organizations Platform (POP), Spain
16:10	**Panel discussion – Measures and metrics to enable iterative improvement and continued learning among stakeholders** 5 minutes reflection on the following questions: • Measuring the efficiency of the JCA process - How can metrics be defined for short and long-term goals? • Measuring the effectiveness of JCA – How well is it utilised at the national level? How does JCA affect national decision making? • Beyond time metrics, discuss elements contributing to the success of HTAR, including improvement of the quality of national decision making and the augmentation of national capacity. • Enhancing consistency in approaches to JCA and national HTA-informed decision making. • Possibility of establishing good HTA practices as an integral component of HTAR across all EU HTA agencies. • Cooperation with stakeholders (clinicians, patients and industry). **HTA Viewpoint – Belén Torres,** Member of Joint Clinical Assessments and identification of Emerging Health Technologies Subgroups, Agencia Española de Medicamentos y Productos Sanitarios (AEMPS), Spain **Payer Viewpoint- Dr Michael Ermisch,** Head of Department AMNOG G-BA, GKV-Spitzenverband, Germany **Payer Viewpoint - Dr Marc Van de Casteele,** Coordinator of the Pharmaceutical Experts, Department of Pharmaceutical Reimbursement, Belgian Health Care Institute RIZIV-INAMI, Belgium **Company Viewpoint – Dr Vanessa Schaub,** Head of Global/EU HTA Strategy, Roche, Switzerland **Patient Viewpoint - Valentina Strammiello,** Director of Programmes, European Patients' Forum, Belgium
17:30	**Next steps and close of meeting**
19:00	**Drinks reception followed by workshop dinner**

Section 2: Presentations

Please note that the following presentation summaries represent the views of the individual presenters and do not necessarily represent the position of the organisation they are affiliated with.

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Session 1: Preparing for the EU HTA Regulation: Insights from agency perspectives

Enhancing JCA preparedness and gap analysis: Internal exercises and shared learning from a national HTA agency

Experiences with implementing the EU HTAR within the National Health Care Institute

Prof Wim Goettsch, Professor HTA, Utrecht University and Special Advisor HTA, National Health Care Institute (ZIN), The Netherlands

ZIN is an independent governmental body that manages the basic health care package in the Netherlands. Therapeutic value assessment, economic evaluation and appraisal steps are all performed within ZIN. ZIN is considered a medium-sized agency with about 50-80 people working on HTA, and a smaller number dedicated to HTA for pharmaceuticals.

What does the HTAR mean for ZIN?

The Regulation on Health Technology Assessment (EU) 2021/2282 (HTAR) provides several opportunities, such as allowing countries to join forces to carry out assessments, saving industry time and costs by submitting documents centrally at the EU level, and taking HTA to a higher level within the EU as a whole. For ZIN, advantages of the HTAR include avoiding duplication of work, exploring how the joint clinical assessment (JCA) reports may be used within the Beneluxa Initiative (a collaboration between Belgium, the Netherlands, Austria, Luxembourg and Ireland) and giving ZIN assessors a new European dimension to their work. ZIN is also interested in how the HTAR can support voluntary cooperation and HTA agencies working together beyond JCA e.g. on additional data collection.

Important steps to prepare for the implementation of HTAR

ZIN has carried out a number of activities internally to prepare for the implementation of HTAR (see slide below). The first step was to decide on the Dutch delegation for the HTA Coordination Group (CG) and its subgroups, including putting forward a Co-Chair for the JCA Subgroup. Following a discussion with its Board of Directors, ZIN decided that it would aim to participate three times as an assessor or co-assessor in JCAs that were to be conducted in 2025. ZIN has also carried out horizon scanning to anticipate medicinal products that will undergo JCA, workshops and training for its assessors, meetings with the Ministry of Health and internal restructuring of its IT environment to make it more collaborative in the JCA.

HTAR implementation project

An internal HTA implementation project was set up to ensure that ZIN:

- Applies the rules of the EU HTAR correctly from 1 January 2025 onwards.
- Participates in the EU HTAR, in line with ZIN's ambitions.
- Uses the advantages that the EU HTAR has to offer ZIN and Beneluxa.
- Connects with stakeholders concerning the modification of ZIN's process, receiving input from them and supporting them in taking the steps they need to take to prepare.

The project features a project group, steering committee and sounding board group, with representation from different parts of the organisation, for example, assessors, IT, communication, legal affairs etc. The basis of the project is to perform gap analyses in which EU and ZIN templates, procedures and methods are compared, and the outcomes are used to decide ZIN's next steps in HTAR preparation. The initial focus of the project is on medical products but will soon be expanded to medical devices too.

ZIN has begun making adjustments to its assessment process, for example, it will start earlier with the PICO formulation and have this discussion with stakeholders such as industry associations, healthcare providers, patient associations and payers. The agency is also making adjustments to its templates to ensure that it does not request information that was already requested on the EU level and to facilitate the incorporation of the JCA report into the national report.

Summary

For the Dutch HTA agency, ZIN, advantages of the HTAR include avoiding duplication of work and giving assessors a new European dimension to their work. Important steps in preparation for the implementation of HTAR include deciding the Dutch delegation in the HTA Coordination Group and its subgroups, horizon scanning, discussing capacity with the Ministry of Health, workshops and training for assessors, and restructuring ZIN's internal IT environment. Gap analyses are being conducted to compare EU and ZIN templates, procedures and methods in order to make adjustments to the national assessment process. It is essential that HTA agencies involve stakeholders in their preparations for HTAR and are willing to make compromises on a national level to support the European system to evolve over time.

Enhancing capacity for JCA implementation in the context of national resource settings

Polish agency perspective

Dr Kamila Malinowska, Director of the President's Office, Agency for Health Technology Assessment and Tariff System (AOTMiT), Poland

From the Polish agency's perspective, the main considerations for determining capacity for JCA implementation are the national legal framework, HTA methodology and communication with stakeholders. These are described in more detail below.

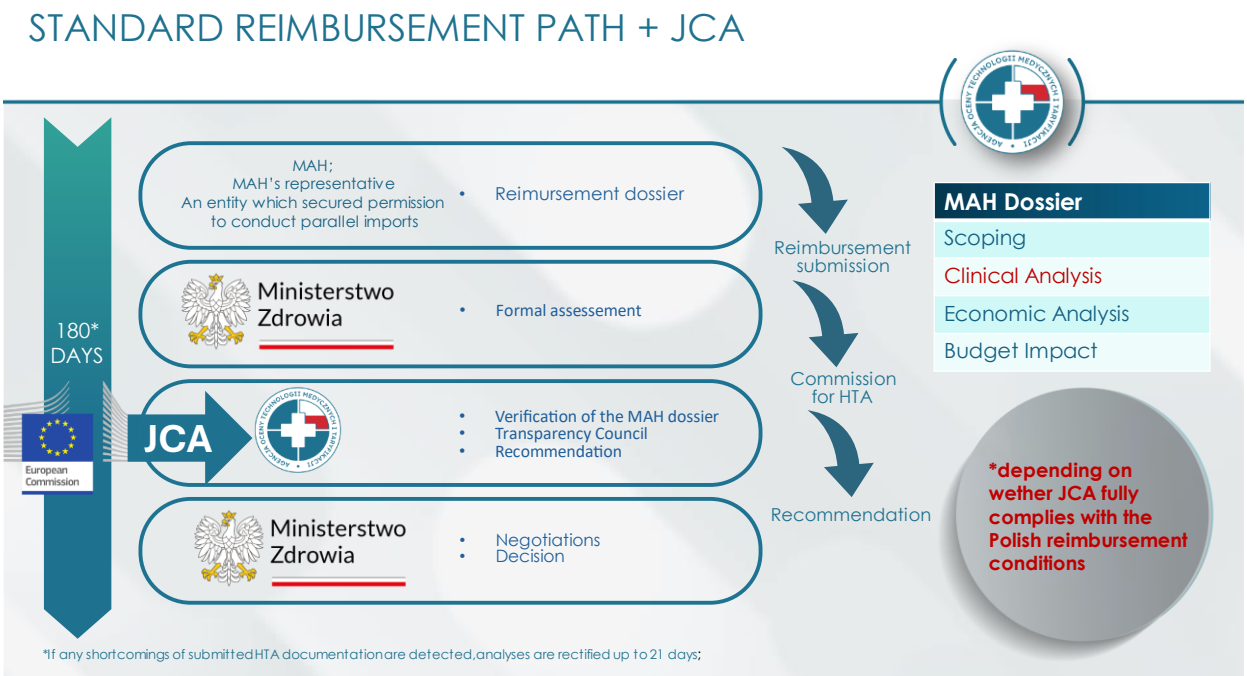
Legal framework

The Act of 12 May 2011 on the Reimbursement of Medicinal Products, Foodstuffs Intended for Particular Nutritional Uses and Medical Devices is the main legal act that regulates the rules for submitting reimbursement applications in Poland. AOTMiT has proposed a list of 30 amendments to this Act in relation to the implementation of JCA in Poland, which are anticipated to be relatively simple to integrate into AOTMiT's standard reimbursement process.

HTA methodology

Standard reimbursement process

Currently for Poland's standard reimbursement process, the Marketing Authorisation Holder (MAH) provides the clinical analysis to the Ministry of Health, and then AOTMiT receives it once it is formally accepted by the Ministry. For the proposed process including JCA, AOTMiT must download JCA reports from the HTA Coordination Group portal and verify compliance of the JCA report with the MAH submission (see slide below). The agency will use the JCA report in the assessment and provide information on how it was used or explain the reason for non-use in the reimbursement process. If any shortcomings of the JCA report are detected, AOTMiT can request the MAH to rectify the submission. Article 36a. of the Reimbursement Act states that analyses are valid for one year from the date of acceptance of the MAH submission, but no longer than three years from the date of their preparation.



List of highly innovative technologies

There are concerns that the evaluation of highly innovative technologies in Poland could be delayed by the introduction of JCA. This is because the listing of highly innovative technologies in Poland is conducted annually, so JCA reports would have to be available before 15th March each year to be considered for that year's listing.

Stakeholder communication

Dialogue and consultation with stakeholders including industry, patients, lawmakers and clinicians is key to agencies' preparation for JCA implementation. Multi-stakeholder communication should also be maintained in the implementation stage. For Poland, the implementation stage will involve a survey on clinical practice to support the preparation of national PICOs.

Summary

The national legal framework, methodology and stakeholder dialogue are key considerations for determining agency capacity for JCA implementation. To facilitate the implementation of JCA in Poland, AOTMiT has proposed a list of 30 amendments to the main legal act that regulates the submission of reimbursement applications. While the standard reimbursement path in Poland is not expected to be significantly impacted by JCA, there could be delays in the evaluation of highly innovative technologies. The next stage for JCA implementation in Poland involves a survey on clinical practice to help inform national PICOs.

Enhancing capacity for JCA implementation in the context of national resource settings

Bulgarian agency perspective

Boryana Ivanova, Head of HTA Department, National Council on Prices and Reimbursement of Medicinal Products (NCPR), Bulgaria

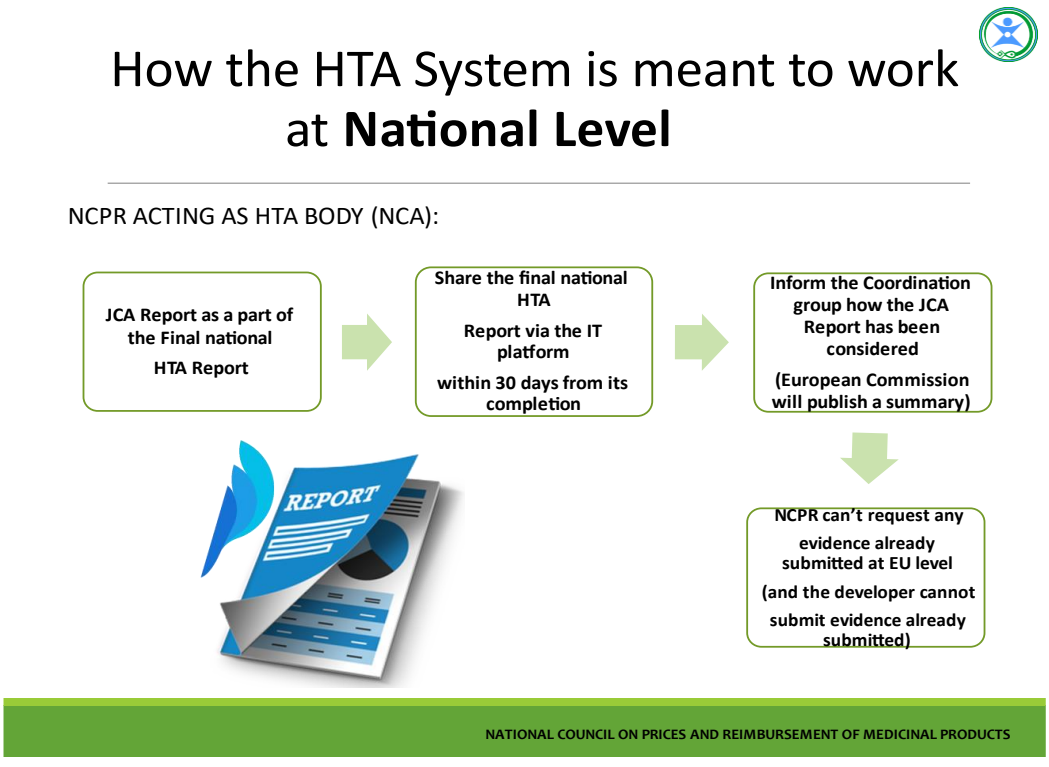
The main responsibility of the NCPR is maintaining the Positive Drug List in Bulgaria. HTA has been included in NCPR's processes since 2019, so is relatively new. The agency is in the process of building its capacity for national HTA by working closely with academics and other external experts.

NCPR is a member of both the HTA Coordination Group and its four subgroups; it has appointed representatives with relevant expertise to participate in meetings and review draft documents, guidelines, templates and implementing acts. NCPR is trying to participate as much as possible in the joint actions and PICO exercises to gain knowledge, skills and experience.

Implementing JCA at the national level

The NCPR is currently preparing to implement and maintain two parallel processes (JCA and non-JCA) while facing restricted time and resources. The agency is adjusting and matching its national procedures and documents to those that have been prepared for the HTA Regulation. To prevent potential delays in assessment, NCPR plans to involve its expert assessors in defining the assessment scope as early as possible, as well as in the process of reviewing the consolidated PICO.

The JCA report will become part of the national HTA report, which once finalised will be shared with other Member States on the joint IT platform. The next step will be for NCPR to inform the HTA Coordination Group about how the joint report has been considered in the national process. NCPR will remain responsible for drawing conclusions on added value of the assessed technology in terms of the national health system, as well as making decisions on pricing and reimbursement.



Homework for Bulgaria

Legislative amendments needed for the implementation of the HTAR are in process. Once these are finalised, the relevant procedural amendments will be made. Harmonisation of national legislation is only possible after all implementing acts are adopted and publicly available.

Proactive communication with industry, academia, patients, clinical and other relevant experts is key for HTAR preparations and for stakeholders to understand the benefits of HTAR. For example, NCPR helped to organise a regional information event held in Athens in September 2023.

Summary

Being a member of the HTA Coordination Group and subgroups has helped the Bulgarian NCPR to build capacity and capability on the national level. A review of national legislation in Bulgaria is underway and the NCPR is adjusting and matching its national procedures and documents to those that have been prepared in terms of the HTAR. Proactive communication with industry, academia, patients, clinical and other relevant experts is key to sharing the benefits of the HTAR.

Enhancing capacity for JCA implementation in the context of national resource settings

Romanian agency perspective

Mihaela Popescu, Physician in Health Technologies Department, National Agency for Medicines and Medical Devices (NAMMDR), Romania

A fragmented HTA system

The institutional arrangement for HTA within NAMMDR is fragmented. Prices are set prior to the evaluation by the Ministry of Health, then the assessment is conducted by the agency with minimum involvement from the ministry. The National Health Insurance House is the single payer in the social insurance system. It is not involved at any stage in the reimbursement decision making but is involved in negotiations for management entry agreements. The draft of the prescription guideline is split between the ministry, the consultative commissions from the ministry, the National Health Insurance House and NAMMDR.

Reforming HTA methodology

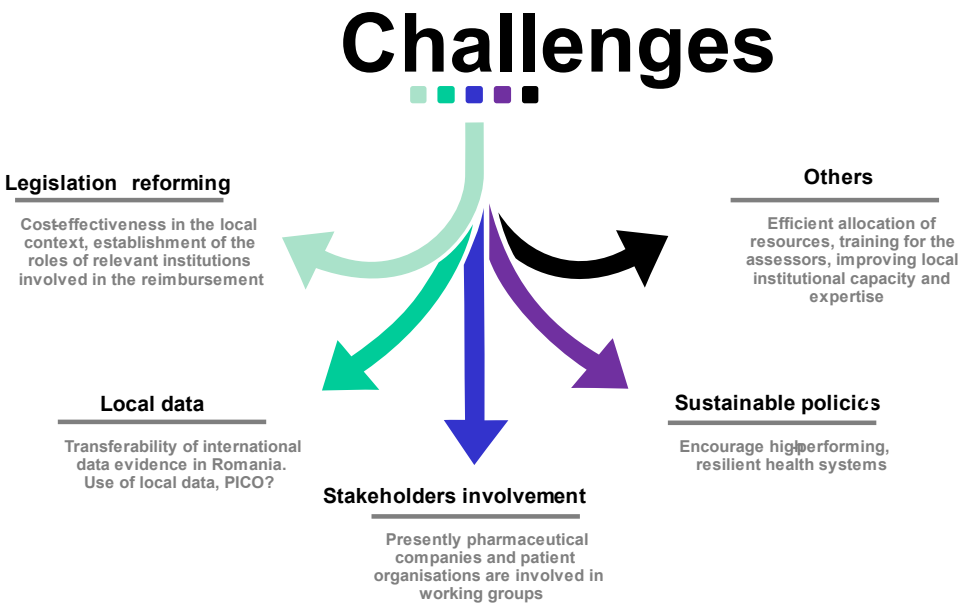
Since 2014, HTA in Romania has been based on a points-based system that considers the results of clinical and economic evaluations of France, Germany and the UK, as well as the number of EU countries where the medicine is reimbursed. The scorecard is then completed with a minimum budget impact analysis. This HTA methodology does not take into account the local environment in Romania. A collaborative project with the World Bank is underway to change Romania's HTA methodology; any future changes must link with the provision of the HTAR.

Challenges for HTAR

Several challenges have been identified in relation to implementing JCA in Romania (see slide below). These relate to national legislation, establishing the roles of the relevant institutions, the use of local data, improving local institutional capacity and expertise, ensuring sustainable policies and stakeholder involvement.

How the JCA report will be included in the national report is still work in progress for Romania. The NAMMDR is working with experts in the Consultative Committees of the Ministry of Health on the national PICO.

National Agency for Medicines and Medical Devices of Romania



Summary

HTA practice in Romania is undergoing reformation, as the existing reimbursement environment is fragmented and the HTA methodology is a point-based system not based on local context. Further changes need to be made to align with the provision of the HTAR. There are several challenges regarding the national implementation of JCA, such as the need to reform legislation, use local data, involve stakeholders, and improve local institutional capacity and expertise.

Session 2: Preparing for the EU HTA Regulation - Insights from company perspectives

Pharmaceutical companies' preparedness and organisational strategy for the HTA Regulation

Outcome of the pre-workshop survey

Dr Tina Wang, Senior Manager, HTA Programme and Strategic Partnerships, CIRS

In May 2024, CIRS surveyed its member companies to gain collective insights into company readiness for the HTAR and help inform the workshop discussions. 15 out of 20 companies responded to the survey invitation, with 13 providing detailed responses (collective results described here).

Internal preparations

When asked about their readiness for the HTAR, no companies indicated that they were completely unprepared, 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 had been taken by the surveyed companies to prepare for the HTAR. For example, most respondents had conducted assessments of pipeline products anticipated to undergo JCA in 2025 (92% of respondents), participated in HTA-related conferences, workshops and training (92%), and established an internal task force dedicated to HTAR (85%). Only 38% of respondents had participated in EUnetHTA rapid effectiveness assessment pilots.

Strategic considerations

When asked about the impact of HTAR on EU 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. Joint strategic planning and decision-making processes for evidence generation plans, closer cross-team interaction and mutual awareness of HTAR were highlighted as important internal considerations for the development strategy of products undergoing JCA.

The survey highlighted that company approaches to global regulatory strategy and JCA submission are likely to be asset dependent. Internal utilisation of the JCA report is also expected to vary depending on the specific jurisdictions being submitted to. As internal discussions are ongoing, there was recognition that companies need to stay flexible and evolve their strategies with practical experience.

Measuring success

There were diverging viewpoints among the surveyed companies as to how best to measure success of the HTAR. When presented with a list of potential indicators, most companies selected positive reimbursement decisions in EU jurisdictions (62% of respondents), jurisdictional HTA review not being duplicative of the JCA report (54%) and enhanced predictability and transparency in HTA processes (54%) as being the most important short-term indicators. No company selected faster rollout from EMA submission to HTA recommendation or more aligned timelines for HTA decisions across jurisdictions. When considering long-term success of the HTAR, greater alignment of HTA methodologies and criteria across Member States (92% of respondents) and opportunity for the adoption of more progressive evidence requirements (such as RWE) by EU HTA agencies (92%), were thought to be the most important indicators.

Challenges and potential solutions

The survey highlighted several internal and external challenges for companies to ensure a timely rollout of JCA reports to local HTA decision making. Key internal challenges were coordination difficulties between EU-level JCA processes and local HTA submission timelines (92% of respondents) and complexity in aligning JCA findings with specific local requirements (92%). To navigate these challenges, it was felt that there must be a good quality JCA submission underpinned by strong methodologies; many companies indicated that a lot can be learned from their German and French affiliates' experience. Companies must also embrace a learning-by-doing approach, implement an iterative feedback mechanism and plan early during development to identify assets and evidence requirements, utilising JSC for advice.

External challenges that were highlighted included varying acceptance of JCA reports among HTA agencies (92% of respondents), duplicative processes and requirements for submission to local HTA agencies (92%), agency resource constraints (77%) and lack of established HTA in certain jurisdictions (77%). Suggested solutions were to ensure transparency and quality in JCA and national decision making, agency engagement with companies during the JCA process and optimising JCA through iterative learning.

Summary of CIRS survey results

Adaptive Thinking and Flexible Approach
•Companies are increasingly prepared to embrace adaptive thinking and maintain a flexible approach to navigate changing environments

Learning by Doing
•The key is to evaluate experiences continuously and establish a quick feedback loop, ensuring rapid learning and improvement.

Early and Ongoing Engagement
• Engage with HTAb early and throughout the process. Enable quality, transparent decision-making process at the national level.

Measures of Success
•In the short term, success is reflected by gaining positive recommendations.
•In the long term, the focus shifts to achieving greater alignment in methodologies and evidence requirements

How are companies adapting to the JCA - What are the opportunities and key challenges faced internally?

Dr Antonia Morga, Global HEOR Product and HTA Strategy Lead, Medical Affairs, Astellas Pharma Europe

Internal preparations

Since the adoption of the HTAR in December 2021, Astellas has established an EU HTA project team under the guidance of a steering committee. A crucial step in this process has been the creation of an affiliates network, which is playing a key role in supporting the readiness of local affiliates and national launch plans. Ongoing impact assessments are being conducted to evaluate how the HTAR will affect the company's operations and pipelines.

A commitment model

Astellas has developed an internal commitment model to streamline support from global functions to local affiliates in preparation for the JSC and JCA processes. The company is currently presenting this model across all primary focus areas, including clinical development and regulatory affairs. One of the most significantly impacted areas is the biostatistics/data science function, which will play a critical role in completing the analyses required for the JCA. The overall goal is to foster a collaborative, cross-functional approach that empowers product teams to develop a robust JCA strategy, execute the necessary analyses, and draft a high-quality dossier.

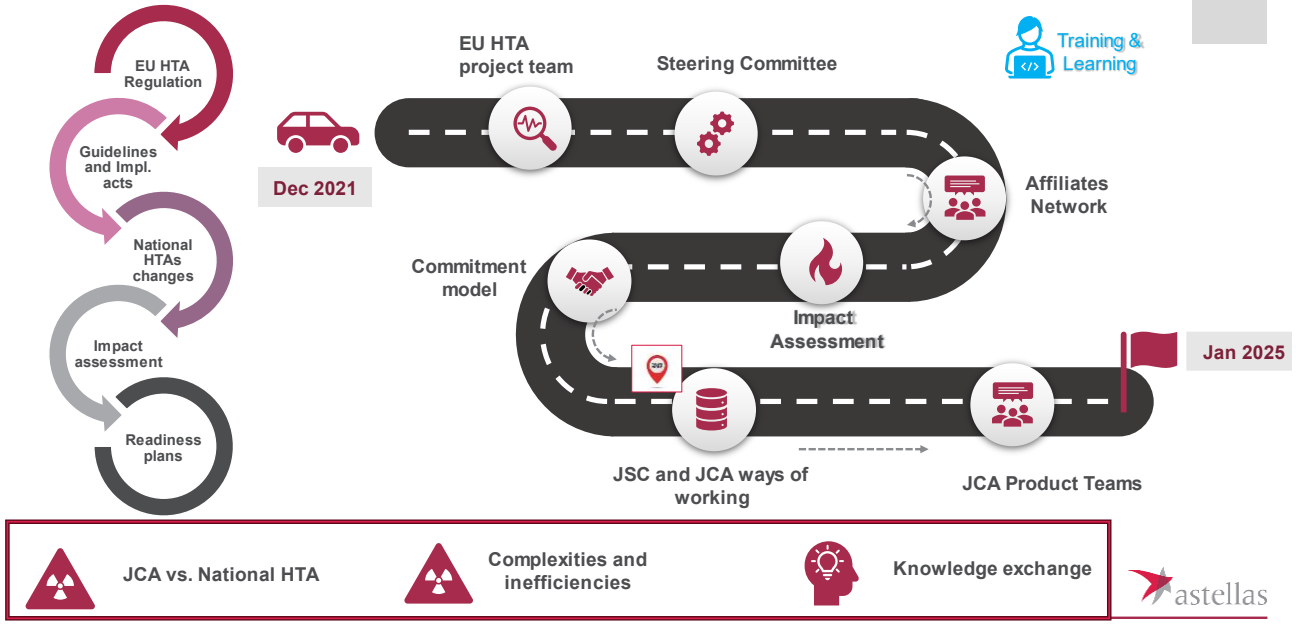
Challenges for HTAR

Open dialogue and knowledge exchange among stakeholders are crucial for the success of the HTAR. However, there have been limited opportunities for companies to contribute meaningfully to the configuration of the JCA process. Concerns have been raised that the JCA is not being fully integrated into national HTA processes. Additionally, the complexity of managing multiple PICOs and the lack of transparency regarding their consolidation pose significant challenges. Companies may need to begin working on the dossier 18 to 24 months before submission, which can strain resources, especially given the limited guidance available.

Summary

To prepare for the implementation of the HTAR, Astellas has established an internal project team, steering committee, and affiliate network. The impact on resources remains a concern for both companies and HTA agencies. There is a clear need for more opportunities for open dialogue between companies and agencies, allowing for a proactive, learning-focused review of JCA processes.

EU HTA REGULATION: A ROADMAP OF PEOPLE AND PROCESSES



Astellas Confidential and Proprietary Information

How are companies adapting to the JCA - What are the opportunities and key challenges faced internally?

Dr Lara Wolfson, Associate Vice President and Head of HTA Statistics, MSD, Switzerland

A bumpy journey

While the HTAR has the potential to make the process of bringing medicines to patients much smoother, there are going to be bumps in the journey. The amount of effort companies and agencies will have to put in is likely to be significant for 5 to 10 years, but then will hopefully reduce as harmonisation occurs. A key question is: how can companies, HTA agencies, the EU HTA Coordination Group and Stakeholder Network work together to shorten that timeline and lessen the height of that bump of increased resources?

Need for dialogue

Multi-stakeholder dialogue on the JCA process has been limited so far. There is a need for companies and HTA agencies to come together to discuss the real decision problem in terms of what are the right PICOs and what data is available to answer a particular clinical question. Companies are conducting a huge amount of analysis to predict different PICO scenarios, and there is the risk that some of this may not be relevant. The biggest concern for companies is conflicting PICOs; what happens if there are two PICOs that cannot both be answered in a development programme? There needs to be mutual understanding as to how evidence-based decisions are going to be made based on the PICO framework.

An opportunity in joint tools

Although there are lots of challenges, there is also an opportunity for companies and the HTA Coordination Group to come together to jointly develop open-source tools and algorithms to facilitate information sharing and establish a common framework for analyses. Such tools would not only be helpful for companies by reducing duplication, but would also help the assessors in HTA agencies as they would provide some confidence in the similarity of analyses.

Summary

The HTAR gives opportunities for companies and HTA agencies to collaborate, but there is a need to build trust by having an open dialogue. This will help to address the workload concerns of both stakeholders.

How are companies adapting to the JCA - What are the opportunities and key challenges faced internally?

Dr Aikaterini Fameli, Global Head of Oncology and HTA Policy, GSK

From 2025 the EU launch process will change, meaning companies will need to develop an additional dossier in parallel to regulatory filing. The evidence to satisfy all EU market HTA processes will be needed ahead of regulatory approval, so companies cannot stagger evidence generation in the way being done now.

Opportunities and challenges

The impending arrival of JSC and JCA has required companies to map their risks and opportunities, considering incremental activities versus efficiencies. A key opportunity is the acceleration of patient access in all EU markets by making HTA evidence available earlier, especially in less mature markets. Companies hope that eventually, local dossier development will be more efficient, given more uniform standards and reduced duplication. However, there are several challenges to overcome, including the unpredictability of the new process, the additional resources needed and how to adapt ways of working.

Adapting operationally

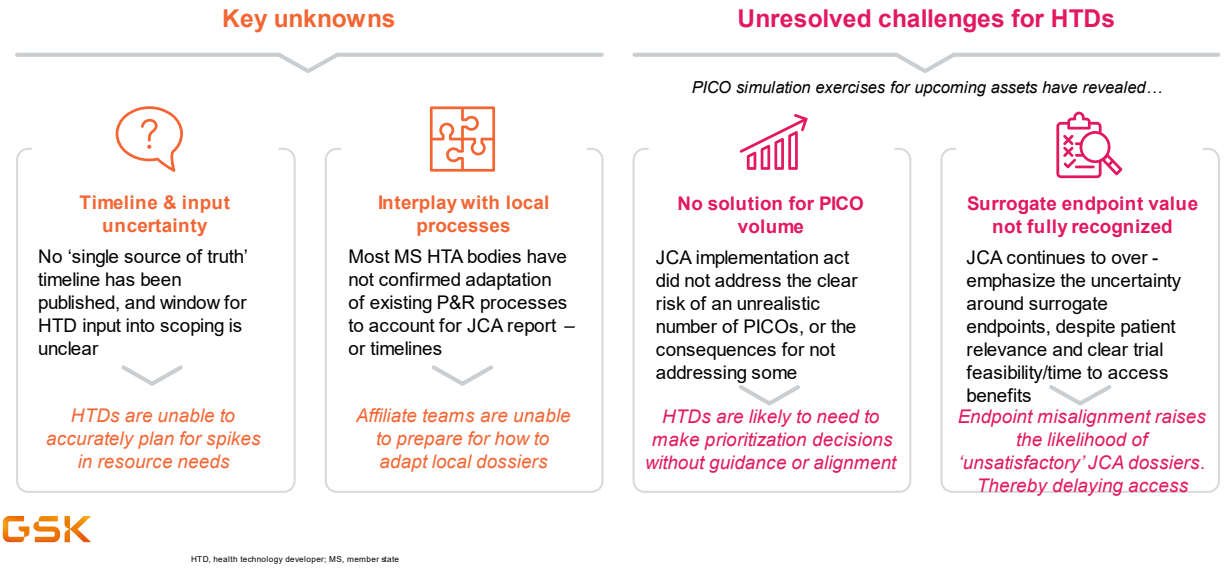
Operational processes across nearly all key functions within companies will need to adapt, collaborating earlier to align on submission requirements for all EU Members States. For example, there will be an impact on regulatory as well as market access teams because regulatory documents will be needed earlier. There is also the need to formalise coordination roles, establish new matrix models, enable collaborative technology platforms and support continuous training as HTAR processes evolve.

What is still unknown?

Efforts to prepare for JCA have revealed key unknowns and unresolved challenges for companies (see slide below). For example, there is still a lack of understanding of the interplay of JCA with local processes; which countries are going to fully adopt the new JCA process? What if a country does not want to run two processes (JCA and non-JCA) in parallel?

Efforts to prepare for JCA have revealed remaining unknowns

What is still unknown or unresolved?



GSK

HTD, health technology developer; MS, member state

3

Summary

The EU HTAR cannot become an additional, bureaucratic barrier to patient access. Companies need a clear, workable and predictable framework that must be fully functional by the time the first products are assessed. There must also be early and standardised involvement of companies, patients and clinicians throughout the JCA process. Adequate resources and early availability of EU-level HTA outputs are needed to ensure the acceleration of national processes and faster patient access.

Session 3: Considerations and measures to assess efficiency and effectiveness

Breakout discussions

Workshop participants were assigned to a breakout group and provided with a background document developed by CIRS, containing information and questions for discussion. The Chairs and Rapporteurs of each breakout were asked to facilitate and document the discussion, respectively. The Rapporteurs then fed back to all workshop participants in the main plenary session.

Breakout A: Efficiency of the process from JCA to national HTA decision making

Chair: Dr Michael Berntgen, Head of Scientific Evidence Generation Department, EMA
Rapporteur: Alison Davie, Senior Director, HTA Oncology, Eli Lilly, UK

The breakout group considered the following questions. Key points from the discussions are summarised below.

*Q1) What **measures** should be integrated into the system to evaluate the efficiency of the process from JCA to national decision making?*

- 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

*Q2) What **factors** will influence the efficiency of national implementation after JCA production, and what barriers and enablers might affect the process?*

- Collaboration – experience varies across Member States (MS), so the interpretation of some data in the JCA report may be challenging for some MS. Sharing knowledge and learnings among MS will be key to supporting those with less mature HTA systems.
- Transparency and trust – to ensure efficient uptake of JCA, there must be trust in the JCA process. This can be enabled through transparency e.g. ensuring documents are available to understand decision making.
- Alignment (across companies and across MS) - Stakeholders must remember that they are not looking at this process in isolation. There must be alignment of national PICOs with perspectives on JCA.
- Continuous learning and improvement – practice will be key to influencing efficiency.

*Q3) Besides timeliness, what other **quantitative or qualitative measures** should be considered to support process efficiency? E.g. Quality, transparency, predictability*

- Influence of lifecycle approach and feedback loop – can companies take the learnings back to development teams to inform clinical programmes at the early phase? The ultimate goal for companies is speed to market; internally this needs to be thought of from an HTA viewpoint in addition to regulatory.
- Transparency – could be measured via a database of outcomes, reporting on the decisions made and how they were made. This will be beneficial for MS learning but also for companies, informing their national submissions.
- Added value of JCA – how can this be measured?
- Trust and acceptability – measure MS perceptions of JCA (including its influence at the local level) through a survey.
- Patient engagement – what was the impact at JCA and national level? How did that translate into decision making?
- Capacity and capability building - training and knowledge sharing will help to address the demand from JCA and national dossiers.

*Q4) How will **communication channels** between EMA and HTA bodies, as well as between national HTA bodies, support JCA process/national implementation efficiency? How should this be measured for iterative improvement and learnings?*

- A voluntary channel of communication across MS is important – if the JCA IT platform is used for communication, it may be possible to measure how useful this is.
- An EU-level HTA agency sitting beside EMA would aid communication and support efficiency.
- Can the European Public Assessment Report (EPAR) have more context on the decision-making framework, which may aid assessors and decision making across MS?
- Communication between other stakeholders is also important to facilitate continuous learning throughout the lifecycle.

*Q5) **Recommend** further work to enable timely company submissions and efficient agency coordination.*

Recommendation	**Timescale**	**Who should be implementing?**
Develop **metrics framework/scorecard** to evaluate efficiency and inform continuous improvement – could use the measures suggested in Q1.	Short and long term	CIRS
Establish **training** programmes to support capability and capacity building for Member States.	Short and long term	All stakeholders
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?	Short term	HTA Coordination Group and companies
Develop **open-source tools** to reduce duplication and build capacity across health technology developers e.g. statistical tools.	Mid term	Companies

Breakout B: Effectiveness of the JCA

Chair: Dr Anja Schiel, Senior Adviser; Lead Methodologist in Regulatory and Pharmacoeconomic Statistics, Norwegian Medicines Agency (NOMA), Norway
Rapporteur: Thomas Butt, Executive Director, Head of Global Health Economics and Outcomes Research, Biomarin, UK

The breakout group considered the following questions. Key points from the discussions are summarised below.

*Q1) What are the **essential components** necessary to support the effectiveness of JCA?*

- Communication – stakeholders need to have a mutual understanding of the purpose of JCA and the impact it will have on each country's national HTA decision. Communication must be early, pragmatic and tailored to different stakeholders e.g. what does JCA mean for clinicians, patients, HTA agencies outside the EU?
- Resources – this is an issue but need clarity on the processes to have a better understanding of the resources required.
- Joint scientific consultation (JSC) – there is an opportunity to use JSC to build a better evidence base and improve decision making in the long term. However, some HTA agencies are not used to early advice and may not anticipate prioritising resources for JSC, as the benefits may not be seen for 5-10 years.

*Q2) What are the **barriers and enablers** for integrating JCA reports into national decision-making?*

The group agreed that a key barrier is the unclear decision-making relevance of JCA. There is a need for mutual understanding and communication of the fact that the JCA will have different weight in different decisions in different countries. Including additional evidence in the JCA (e.g. extrapolating data) could perhaps enhance the value of the JCA to some countries, however, the value would need to outweigh the resource implication.

The group also highlighted the challenge of providing the right information at the time of the JCA. What is the consequence if that information isn't available? There could be a situation where a requested PICO is not anticipated and so cannot be answered in time; justification may have to be enough.

*Q3) What **measures** are needed to understand the added value of JCA report in supporting national HTA assessment? What measures need to be in place to provide feedback for iterative learning and improvement?*

- Impact of JCA on decision making:
  - Time/speed to patient access (and change over time)
  - Time/speed to start of national submission process – time to validate a submission (and change over time)
- Impact of JCA on resourcing:
  - Time and resources spent on JCA by agencies and companies (does this change over time?)
- Impact of JCA on access to innovation:
  - Measure to be decided.
  - Although intended to equalise access, JCA could be a potential barrier to some companies coming to Europe.
- Impact of JCA on stakeholders:
  - Inclusion and transparency with patients
  - Inclusion and transparency with healthcare providers.

*Q4) **Recommend** further work to ensure the value of the JCA output and its effective utilisation in national decision making.*

Recommendation	**Who should be implementing?**
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.	CIRS
Enhance **communication** between stakeholders: more frequent and earlier communication. This must help to set expectations on what the JCA is and is not.	All stakeholders
Identify **metrics** that can help HTA agencies to understand the value of JSC and enable iterative improvement.	CIRS
Develop a **product-based scorecard** to evaluate 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?	CIRS

What should be the future considerations for involvement in JCA and national implementation at the Member State level?

Patient group perspective

Pedro Carrascal Rueda, Executive Director, Patient Organisations Platform (POP), Spain

The mission of POP is to promote the effective participation of patients in the health and social system. POP is an umbrella organisation representing approximately 1800 patient associations supporting patients with chronic diseases in Spain. POP is a member of the European Patients Forum (EPF), an umbrella organisation of patient organisations across Europe and across disease areas.

How should local experts be involved?

Local experts such as patient representatives and clinical experts should be engaged early in the JCA process. Collaborative scoping workshops involving local clinical experts, patient representatives, and JCA members should be used for discussing national priorities, specific health challenges, and the feasibility of implementing JCA recommendations within the national context. It is also important to provide training for local experts to familiarise them with the JCA processes and methodologies. This can enhance their ability to contribute effectively and ensure that their involvement adds significant value to the scoping process.

What are the challenges?

An overarching challenge at both the European and Member State levels is a lack of clear regulation about the role of patient organisations in the health system and what is expected of them. This can make collaboration between patient organisations and health system partners such as national HTA agencies more difficult.

There are several challenges to maintaining a balance of patient and clinician representatives across different Member States. These include variability in the strength of patient advocacy, resource and funding disparities and language/cultural differences that can create communication challenges, hindering effective collaboration and balanced representation. Potential solutions are capacity building and training, particularly for under-represented or resource-limited Member States, and ensuring equitable allocation of funding and resources, such as travel grants, stipends and logistical support.

How can the implementation of JCA be enhanced?

Active participation of national bodies of experts such as patient organisations in the JCA process is important to ensuring effective utilisation of JCA outcomes at the national level. Patient organisations have a key role in capacity building and education of patients about JCA, and may use JCA documentation as a tool for local advocacy. Making use of pilot programmes will be important to test the feasibility and impact of JCA recommendations in the national context; the results of such pilots can be used to refine and scale up successful initiatives.

EPF recently published ten recommendations aimed at improving patient involvement in JCAs under the HTAR (see slide below).

10 Key Recommendations from Patient Organisations on Joint Clinical Assessments under the EU HTA Regulation

1. Establish a predictable framework for patient involvement in JCAs
2. Include input from patients, carers and patient organisations
3. Include patient experience data in JCAs
4. Streamline patient involvement throughout the process
5. Provide plain language summaries of technologies
6. Broaden the pool of patients and specify selection criteria
7. Provide support to patients
8. Make JCA and summary reports available in all EU languages
9. Provide feedback to patients
10. Adopt a constructive approach to confidentiality and conflict of interest

Summary

There is currently a lack of clarity regarding how local experts, including patients and patient organisations, will be involved in the JCA process. Early involvement is key, but to ensure a balance of experts from different Member States, there needs to be capacity building and equitable allocation of resources. Learning from experience will be critical to strengthening future patient involvement in JCA.

Panel discussion – Measures and metrics to enable iterative improvement and continued learnings among stakeholders

Panellists were asked to consider and discuss the following:

- Measuring the efficiency of the JCA process - How can metrics be defined for short-term and long-term goals?
- Measuring the effectiveness of JCA – How well is it utilised at the national level? How does JCA affect national decision making?
- Beyond time metrics, discuss elements contributing to the success of HTAR, including improvement of the quality of national decision making and the augmentation of national capacity.
- Enhancing consistency in approaches to JCA and national HTA-informed decision making.
- Possibility of establishing good HTA practices as an integral component of HTAR across all EU HTA agencies.
- Cooperation with stakeholders (clinicians, patients and industry).

Key points from each panellist's presentation are summarised below.

Spanish agency perspective

Belén Torres, Member of Joint Clinical Assessments and identification of Emerging Health Technologies Subgroups, Spanish Agency of Medicines and Medicinal Products (AEMPS)

- AEMPS is a public body attached to the Spanish Ministry of Health that guarantees the quality, safety, efficacy and correct information about medicines and medical devices to society. It carries out a wide range of activities including medicine assessment and authorisation, clinical trial authorisation and HTA of medicines.
- The Ministry of Health is responsible for the state functions on price and reimbursement in Spain.
- AEMPS is actively participating in the four subgroups of the Coordination Group on HTA and has a Co-Chair role on the JSC Subgroup.
- AEMPS has gained support from the EU Technical Support Instrument scheme to help reorganise and strengthen the AEMPS HTA system to support national implementation of the HTAR.
- Success of the HTAR would be to conduct JSC and JCA in a sustainable way that will contribute to making agile decisions and provide predictability at the European and national levels. Stakeholders must continue to work together to achieve this.

German payer perspective

Dr Michael Ermisch, Head of Department AMNOG G-BA, GKV-Spitzenverband, Germany

- The German AMNOG procedure consists of an early benefit assessment of new drugs against a comparator treatment and a subsequent negotiation of the reimbursement price based on the early benefit assessment.
- The initial price of a drug in Germany is set by the manufacturer, who is required to submit a value dossier no later than the time of launching the drug in Germany.
- The timing of the availability of the JCA report could be a potential issue in Germany. This is because companies can choose to launch on the German market immediately after marketing authorisation, but the JCA report may not be available this quickly; it has to be published within 30 days of marketing authorisation.
- Success for HTA agencies would be to gain as much as possible from JCA in order to reduce what has to be done nationally. In the short term, countries with more mature HTA systems such as Germany may have to bear more of the workload of JCA, but in the long term, it is hoped that all countries will benefit from greater efficiency.

Belgian payer perspective

Dr Marc Van de Casteele, Coordinator of the Pharmaceutical Experts, Department of Pharmaceutical Reimbursement, Belgian Health Care Institute RIZIV-INAMI, Belgium

- Discussion with the Belgian pharmaceutical industry has started to shape the Belgian national HTA report, which will be in multiple languages. Some parts will be taken from the JCA report (in English) and others from national Belgian reports (in Dutch or French).
- To facilitate a smooth transition to JCA, Belgium legislation is being revised and an explanatory meeting on the Belgian PICO is planned to take place after the PICO discussion has been closed by the HTAR subgroup.
- A key metric for agencies will be how many staff hours are spent on European HTA, including the activities of the HTA Coordination Group and subgroups, as well as the products undergoing JCAs. Knowing that some of these products will not enter the Belgian market, or will enter at a very late stage, is a concern, as it could mean wasted or duplicated efforts (for example if the JCA report becomes out of date after two or three years).
- Another important metric will be how efficient the European Commission IT platform is – how user friendly is it? How do agencies use it?
- Beyond metrics for JCA, an important milestone for Belgium is the time to a positive or negative reimbursement decision.
- The HTA Coordination Group and its subgroups may have a major role in establishing good HTA practices throughout Europe.
- While collaboration is important, expectations must be realistic about such a large group of stakeholders working together. A high degree of absenteeism should be avoided, as that is not the aim of JCA.
- There needs to be more discussion on how to achieve a smooth transition from European HTA to national HTA, rather than focusing on PICO listings.

Company perspective

Dr Vanessa Schaub, Head of Global/EU HTA Strategy, Roche, Switzerland

- Roche fully supports the objectives of the EU HTA Regulation to reduce duplication, overcome fragmentation by harmonising methods and evidence requirements and allow for timely and high quality HTA throughout Europe, accelerating patient access to health innovation.
- A more consolidated view of what evidence requirements should look like at the European level will also help to put Europe in a stronger position when it comes to company decision making on clinical trial designs and evidence generation planning.
- As a company with a strong oncology portfolio, Roche has identified two products that will likely be going through the EU HTA process in 2025.
- Roche has been intensively preparing for this by proactively engaging externally and advocating for successful implementation of the EU HTA Regulation, as well as preparing internally for JCA submissions in 2025.
- This preparation started over two years ago with a cross-functional team including colleagues from all EU Roche affiliates. The internal change process included adapting our internal operating model, revising processes and creating new job profiles such as e.g. EU HTA Submission Leads and HTA Statistical Programmers to address the high number of anticipated PICOs.
- A successful internal change process also requires a mindset shift: companies must consider HTA agencies an equally important external stakeholder as EMA.
- To measure the overall success of the EU HTA Regulation to the above-mentioned objectives, EFPIA is implementing an EU HTA Tracking tool in 2025 to track KPIs and insights of products going through the EU HTA process to allow for meaningful process recommendations and adaptations along the progressive implementation of the regulation.

Patient organisation perspective

Valentina Strammiello, Director of Programmes, European Patients' Forum (EPF), Belgium

- 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 considered if the JCA requires scientifically based input from a patient perspective, for example, patient experience data to inform PICOs.
- To measure the efficiency and effectiveness of patient involvement in JCAs, there needs to be a process for gathering this data while maintaining the confidentiality of patients.
- EPF is considering running a study evaluating patient involvement in the first year of JCA e.g. how did patient involvement take place? Was it effective? Were there measures to train patients so they could provide valuable input?
- EPF is trying to fill a gap not mentioned in the HTAR: to coordinate European-level patient organisations and national umbrella patient organisations so that the right patients can be involved at the right time. To sustain these efforts, there needs to be capacity building and more financial support for patient organisations.
- Short-term success of the HTAR will be 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.

Appendix: Workshop attendees

Affiliations are stated as they were at the time of the meeting.

HTA and payer organisations		
Dr Nick Crabb	Interim Director, Science, Evidence and Analytics	**National Institute for Health and Care Excellence (NICE), UK**
Dr Michael Ermisch	Head, AMNOG G-BA department	**GKV-Spitzenverband, Germany**
Dr Wim Goettsch	Professor HTA of Pharmaceuticals and Special Advisor HTA	**Utrecht University and the National Health Care Institute (ZIN), The Netherlands**
Boryana Ivanova	Head of HTA Department	**National Council on Prices and Reimbursement of Medicinal Products, Bulgaria**
Kamila Malinowska	Director of the President's Office	**Agency for Health Technology Assessment and Tariff System (AOMiT), Poland**
Octavian Matei	Pharmacist, Health Technologies Department	**National Agency for Medicines and Medical Devices, Romania**
Dr Nicole Mittmann	Chief Scientist and Vice-President, Scientific Evidence, Methodologies and Resources	**Canadian Agency for Drugs and Technologies in Health (CADTH), Canada**
Ralitsa Panayotova	Chief expert in HTA Department	**National Council on Prices and Reimbursement of Medicinal Products, Bulgaria**
Mihaela Popescu	Physician in Health Technologies Department	**National Agency for Medicines and Medical Devices, Romania**
Belén Torres Garrido	Member of Joint Clinical Assessments and identification of Emerging Health Technologies Subgroups, Therapeutic Positioning Report and HTA Area	**Spanish Agency of Medicines and Medical Products (AEMPS), Spain**
Dr Anja Schiel	Senior Assessor / Statistician	**Norwegian Medical Products Agency (NOMA), Norway**
Dr Marc Van de Casteele	Coordinator pharmaceutical expertise	**RIZIV-INAMI, Belgium**
Pharmaceutical companies		
Kevyn Adler	Senior Director Market Access	**Regeneron, USA**
Dr Lianne Barnieh	Associate Director	**Beigene, France**
Thomas Butt	Executive Director, Head of Global Health Economics & Outcomes Research	**BioMarin, UK**
Juan Luis López-Belmonte Claver	HEOR & Pricing and Contracting Lead	**Sanofi, Spain**
Alison Davie	Senior Director HTA Oncology	**Eli Lilly, UK**
Dr Aikaterini Fameli	Global Head of Oncology and HTA Policy	**GlaxoSmithKline, UK**
Pascale Frey Le Jan	International Senior Director, Pricing Reimbursement Access, Oncology	**Eli Lilly, France**

Dr Ramiro Gilardino	HTA Policy Lead	**MSD, Switzerland**
Adrian Griffin	Vice President, HTA and Market Access Policy	**Janssen, UK**
Adam Heathfield	Senior Director, Global Access and Value	**Pfizer, UK**
Dr Mohit Jain	VP, Global Value, Access & Strategic Pricing – Global Head	**BioMarin, UK**
David Javierre	Public Affairs	**Sanofi**
Dr Marisca Marian	Market Access Strategy Leader, Oncology	**Bayer, Switzerland**
Laetitia Mariani	HTA Collaborations Director, International Market Access & Pricing	**AbbVie, Switzerland**
Dr Antonia Morga	Senior Director, Global Health Economics and Outcomes Research (HEOR) and HTA Strategy Lead	**Astellas, UK**
Carolina Pereira	Public Affairs	**Sanofi, Portugal**
Sophie Rabanel	Director, Market Access	**Regeneron, France**
James Ryan	Director, Global HTA Policy, HTA and Modelling Science	**AstraZeneca, UK**
Dr Cathrin Schäfer	Head of Market Access Europe	**Beigene, Switzerland**
Dr Vanessa Schaub	Head Global and EU HTA Strategy	**F. Hoffmann La Roche, Switzerland**
Dr Lara Wolfson	AVP & Head, HTA Statistics	**MSD, Switzerland**
Regulatory agencies		
Dr Michael Berntgen	Head of Scientific Evidence Generation Department	**European Medicines Agency (EMA)**
Academic organisations		
Martina Garau	Director	**Office of Health Economics, UK**
Prof Finn Børlum Kristensen	Professor of Health Services Research	**University of Southern Denmark**
Patient organisations		
Pedro Carrascal Rueda	Director General	**Plataforma de Organizaciones de Pacientes (POP), Spain**
Valentina Strammiello	Director of Programmes	**European Patients' Forum (EPF), Belgium**
Centre for Innovation in Regulatory Science (CIRS)		
Penelope Cervelo	Analyst	**CIRS**
Dr Neil McAuslane	Director	**CIRS**
Dr Brian O'Rourke	Chair, HTA Steering Committee	**CIRS**
Dr Jenny Sharpe	Communications Manager	**CIRS**
Dr Belen Sola	Research Analyst	**CIRS**
Dr Tina Wang	Senior Manager- HTA programme and Strategic Partnership	**CIRS**

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.

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