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EAA Convention Proceedings

Cross-Functional Interface & Approaching the Revision of the EU HTA Regulation

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Convention Agenda

* The EAA Spring Convention 2026 takes place on April 16/17th at RIZIV/ INAMI and Vlerick Business School, Brussels, Belgium.
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Thursday, April 16th, 2026:

Public Session

15:30 Welcome Message from the Host & Setting the Scene (Francis Arickx, RIZIV/ INAMI)

16:00 Plenary Session 1 – EU HTA Regulation: Interface with other EU-Initiatives

(moderated by Antonella Cardone, CPE & Marc Van de Casteele, RIZIV/ INAMI)

- Political Perspective (Tomislav Sokol, MEP)
- Topline Overview: Status and Interface EU HTA Regulation (Valentina Barbuto, DG Santé)
- Touch Points EU HTA with Pharma Strategy/ other Legislation (Fabio D'Atri, DG Santé)
- Industry Perspective (Alexander Natz, EUCOPE)

followed by Plenary Discussion

17:15 Coffee Break & Group Photo

17:45 Plenary Session 2 – Early Insights & Ideas for the 2027/28 Revision

(moderated by Antonella Cardone, CPE & Robin Doeswijk, EHA)

- JCA Subgroup Perspective (Anne Willemsen, JCA SG & ZIN, virtual)
- Patient Needs/ Considerations (Solène Jouan, EPF)
- Clinical Needs/ Considerations (Étienne Lengliné, Hôpital St Louis & HAS & EHA)
- MS Perspective, host-country Belgium (Marc Van de Casteele, RIZIV/ INAMI)
- Health Technology Developers' Perspective (James Ryan, efpia & AstraZeneca)

18:50 Summary & Wrap Up (Fabrizio Gianfrate, Ferrara University; Jörg Ruof, EAA)

19:00 End of Public Session

Friday, April 17th, 2026:

EAA Working Session

08:30 Welcome (Walter Van Dyck, Vlerick Business School; Marc Van de Casteele, RIZIV/ INAMI)

08:35 EU HTA Progress Updates

- Sharing Insights from IHSI (Marcus Guardian, IHSI & BCCH)
- JSC & JCA – Legal Considerations (Dominik Roters, Dierks+Company)
- Snapshot from the CORE-RWE Adjunct Session (Walter Van Dyck)

09:15 Introduction to Break-Outs (Elaine Julian, EAA)

09:30 Break-Out Sessions: Developing Initial Proposals for the 2027/28 Revision of the EU HTA Regulation

1) Patient Perspective (A. Cardone/ M. Otto / M. Racovita)
2) Clinician Perspective (R. Bernardini / R. Casado-Arroyo/ R. Doeswijk)
3) HTD Perspective (E. den Breejen/ S. Capri/ M. Rotaru)
4) Member State Perspective (M. Cossito/ A. Willemsen/ B. Dajka)

Coffee Break during Break-Out: available from 10:30

11:15 Report from Break-Out Groups (Facilitators)

12:00 Final Panel Discussion (Antonella Cardone, Robin Doeswijk)

12:25 Outlook Fall Convention Lisbon (Jörg Ruof, EAA)

12:30 End of Convention

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Editorial

Cross-Functional Interface & Approaching the Revision of the EU HTA Regulation

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The website of the European Commission includes a general description of 'What the European Health Union is'. Accordingly, all EU Member States prepare and respond together to health crises, medical supplies are available, affordable and innovative, and countries work together to improve prevention, treatment and aftercare for diseases such as cancer[1]. Within that framework the EU HTA Regulation (EU HTA R)[2] plays a critical role with regard to availability of affordable and innovative medicines and as recital 3 of the regulation stipulates, HTA contributes to the promotion of innovation, which offers the best outcomes for patients and society as a whole. The EAA strongly supports this statement and as such, two facets of the EU HTA R will be in focus at the EAA Spring Convention 2026:

- The EU HTA R is not a stand-alone initiative. Instead, it is embedded in a wide array of legislative activities and initiatives that together shape the EU Health Union. An in-depth review of Europe's Beating Cancer plan and its link to EU HTA activities was the topic of the inaugural EAA Convention in May 2022 at Copenhagen University. Other ongoing and recent legislative activities – e.g., the Reform of the EU pharmaceutical legislation[3], the European Biotech Act[4], or the Critical Medicines Act[5] – all are of direct relevance for the underlying aims of the EU HTA R. This opens up multiple options for cross-fertilization. Review of such interfaces, identification of opportunities for mutually reinforcing benefits, and the stepwise optimization of access to health care for patients and of value for society will be the scope of the public session at the EAA Spring Convention 2026 in Brussels.
- While the first JCA assessment reports have not yet been published, initial considerations and planning on how to orchestrate the revision of the EU HTA R are already ongoing. Article 31 of the EU HTA Regulation[2] indicates that no later than Jan 13th, 2028, the Commission shall present a report to the European Parliament and to the Council on the application of the regulation. The article also mandates that in January 2027 Member States shall report to the Commission on their consideration of the joint work. The EU HTA Stakeholder Network has already established a dedicated working group. In line with these timelines, and building on previous work by the EAA developing a framework of Key Performance Indicators for EU HTA[6], the upcoming spring convention in Brussels will also focus on generating initial ideas for the revision of the EU HTA R. In the second part of the plenary session key stakeholders will share early insights and ideas in a roundtable discussion, which will be refined by working groups on day 2 of the event.

For the second time, an EAA convention will take place in Brussels, this time at the premises of the Belgian HTA/Reimbursement Institute RIZIV-INAMI and at Vlerick Business School. An adjunct session to the EAA Spring Convention will address challenges regarding the utilization of RWD for HTA, the focus of the CORE-RWE (Causal Observational Research for Effectiveness using Real World Evidence) Methods Research Project which is being set up at Vlerick Business School in collaboration with KU Leuven.

Cancer Patients Europe (CPE) and the European Hematology Association (EHA) are co-hosting the convention, reflecting a strong patient and clinician voice within EAA's multi-stakeholder initiative.

We are very much looking forward to a successful EAA Spring Convention 2026 in Brussels. As frequently reiterated, EAA's overarching aim is to support the successful implementation of EU HTA. In an increasingly competitive international environment, there is no good alternative to developing an effective mechanism for joint HTA, in the interest of Europe's patients and health systems. Therefore, seamless cross-fertilization between the various legislative activities and initiatives of the EU Health Union, as well as optimizing the implementation of the EU HTA R through the evaluation and revision foreseen for 2027/2028 are key goals to which the EAA – uniquely positioned at the heart of the stakeholder field – is full committed.

1) https://commission.europa.eu/topics/public-health/european-health-union_en ; 2) https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX:32021R2282 ; 3) https://health.ec.europa.eu/medicinal-products/legal-framework-governing-medicinal-products-human-use-eu/reform-eu-pharmaceutical-legislation_en; 4) https://ec.europa.eu/commission/presscorner/api/files/attachment/882089/2025%2012%2001%20 FACTSHEET%20-%20BIOTECH%20Final%20(1).pdf; 5) https://health.ec.europa.eu/publications/factsheet-critical-medicines-act-improving-availability-and-securing-supply-critical-medicines-eu_en; 6) Julian E, Xander N, Boumaki K et al. Development of a Performance Measurement Framework for EU HTA. JMAHP 2026

EAA Convention Speaker Abstracts

One year of EU HTA - Learnings from ongoing JCAs

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We have now passed the year-mark for the implementation of the Regulation (EU) 2021/2282 on health technology assessment and while everyone is still eagerly awaiting the first JCA report, constructive exchange around some of the Initial experiences from the first year already have already helped to improve the functioning of the system.

We are pleased to see that there is a gradual increase in the capacity for Joint Scientific Consultations with an increase in the number of slots for advice meetings in 2026. The doubling of the number of request periods throughout the year is also very welcome, since it will make it easier for companies to apply for a slot that matches their development timeline.

However, the overall number of advice meetings is still too low to meet the high demand from companies - and while some EUCOPE members have been able to secure an advice meeting for their product, others will enter into the JCA without having had this opportunity.

14 JCAs have been initiated at the time of writing, and eight of these products are being developed and launched by small or mid-sized companies, and in many cases by smaller biotechs that originate outside the EU.

During the 2025 EAA fall pre-convention, the EMA SME office presented the various ways that they offer to support smaller companies in preparing for the Marketing Authorisation Application by offering advice, guidance and assistance. This support includes the opportunity for companies to request briefing meetings, which consists of early dialogue and discussion of regulatory strategy or product development with the opportunity for follow-up guidance.

One of the initial learnings that we are seeing is that there is a need for additional opportunities for early interactions between the developers and HTA bodies, both while trials are still at the planning stage, and at the start of the JCA, to reduce the risk of misunderstandings regarding what evidence can be expected to be brought forward, which methods to apply and which comparators should be used for an assessment.

The JCA timeline is constructed with short turnaround times, and if misunderstandings are not resolved at the earliest possible stage, there is a higher risk of assessments becoming delayed, discontinued or of poor results.

To reduce this uncertainty, and to avoid poor quality assessments and market launch failures, additional points of interaction should be introduced in the JCA.

EU HTA R – Early Insights and Ideas for the 2027/28 Revision – JCA SG Perspective

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First experiences of the Joint Clinical Assessments

In March 2025, the first Joint Clinical Assessments (JCA) of a medicinal products started. One year later, we have 13 JCA on medicinal products ongoing and in total there are 16 different HTA organisations acting as assessor or co-assessor for these JCAs. The majority of these JCAs have now finalized the assessment scoping phase and are either awaiting a submission dossier from the Health Technology Developer (HTD) or are in the confirmation check of the dossier. Although there are no JCAs publicly available yet, can we already explore procedural learnings of these first JCAs? We will explore the first experiences on the parallel running of JCAs and the ongoing EMA Marketing Authorization Applications as well as early insights into general dossier expectations. These early insights not only relevant to support the upcoming JCAs, but are also particularly relevant given the upcoming evaluation of the HTA Regulation, and the Member States report on the added value of the joint work.

EU HTA R – Early Insights and Ideas for the 2027/28 Revision – Patient Perspective

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Strengthening patient involvement in the EU HTA Regulation: From implementation to evaluation and revision

One year after the start of the implementation of the EU HTAR, it is already clear that this marks both an important milestone and a significant learning process for all involved. At the European Patients' Forum (EPF), we are proud to be part of this process and, through our daily work alongside other patient organisations, to help ensure that the HTAR delivers on its promise: greater harmonisation of HTA across the EU and real progress towards health equity, including better access to treatments for all patients in Europe.

One important lesson from this first year is that we are still in a learning phase. As with any new governance framework, practical issues have emerged that were not fully anticipated during the legislative negotiations, such as short deadlines for patient organisations to identify patient representatives and limited information provided to unsuccessful applicants. While not all of these issues have yet been resolved, some improvements have been made thanks to constructive dialogue with the EU HTA Secretariat.

This first year has also highlighted important lessons for the future and identified several elements that should be considered in the upcoming evaluation of the Regulation and in the future revision. As the first Joint Clinical Assessment reports are expected to be published soon, it will be crucial to assess how patient involvement is taking place in practice and how patient input is reflected in the drafting of those reports. Meaningful patient involvement cannot be reduced to consultation alone. It requires that patients' contributions, perspectives and lived experiences are genuinely taken into account in the assessment process and have a visible impact on outcomes. If the evaluation shows that this is not the case, a revision of the framework should be absolutely considered in order to address these shortcomings and provide more effective solutions.

In particular, we would need to know exactly:

- How patients are effectively involved at the different stages of the HTA process, including the clarity of their role and the timing of their contributions;
- How patient input is considered and integrated into the final reports, and whether this contribution is visible and transparent in the outcomes of the assessments;
- How diversity among patients is ensured, particularly in light of the current English-language requirements and the large volume of technical documentation patients are asked to review. EPF has long advocated for accessible materials in lay language, shared sufficiently in advance, as this would greatly support meaningful participation and ensure broader representation of patient perspectives across Europe;
- The role of patient organisations throughout the process. While the legislation allows for the participation of patient organisations, this potential has not yet been fully realised in practice. Further reflection is needed on how this role can be strengthened and better embedded in the HTA framework;
- How Joint Clinical Assessment reports are used at national level, and whether they contribute to faster and more efficient decision-making processes. While it may be too early during the 2027–2028 evaluation to fully assess whether the Regulation improves access to treatments,

analysing how these reports are used by national authorities will be essential;

- How the EU HTA Regulation is influencing national HTA processes. At EPF, we have begun exploring this question through the first EPF Barometer on Patient Involvement. This Barometer, which will be published in June 2026, analyses patient participation across various national policies and regulatory processes, including HTA, using data collected from EPF's national coalitions. Our findings already suggest some improvements in countries where patients were previously not involved in HTA processes, but improvements are not enough. National reforms taking place in the context of the HTAR provide a unique opportunity to embed best practices from the outset, ensuring that meaningful patient engagement becomes a core and permanent feature of all national HTA processes.

Another important point that should form part of any future revision is the recognition of the added value of patient organisations and the need to secure their sustainability. Their contribution to implementation should be explicitly recognized and publicly funded by the European Union, particularly at a time when many patient organisations face growing financial pressure (with the absence of operating grants under the 2025 EU4Health Programme, and the possibility that these may also be lacking in 2026) and when public resources for training and capacity-building in HTA remain limited or absent. Without such support, meaningful patient involvement in these processes will remain difficult to sustain. Expecting patient organisations to take on growing responsibilities, to train their patients and members, without the necessary resources is neither credible nor sustainable.

Patient organisations are not involved in the implementation of the HTA Regulation simply because this is a "nice to have". We are engaged because we firmly believe that greater harmonisation of HTA practices, combined with meaningful patient involvement, can lead to better access to treatments across Europe, and stronger public health outcomes. At a time when the value of European cooperation in health, and in other policy areas, is being questioned, the HTAR has the potential to demonstrate the concrete benefits of working together at EU level and of strengthening cooperation and harmonisation across Member States. Ensuring that this Regulation succeeds will therefore be essential and this can be achieved if patients remain genuinely at the centre of the process.

EU HTA R – Early Insights and Ideas for the 2027/28 Revision – Clinical Perspective

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It is difficult to make predictions in a period marked by substantial scientific and political uncertainty. Nevertheless, a preliminary assessment of the implementation of the new European regulation on health technology assessment suggests that the process established by the EU HTA Regulation (EU-HTA-R), which entered into force in January 2022, will likely generate a combination of both opportunities and challenges. First, the European harmonization of dossier assessments should promote a more consistent interpretation of submissions by clarifying shared and consensual expectations among European medical communities and the various agencies involved in evaluation. In addition, the implementation of early scientific consultations (Joint Scientific Consultations, JSC) provides an opportunity to clarify clinicians' expectations regarding the data required to rigorously evaluate the additional clinical benefit of innovative therapies. Although the full potential of JSC has not yet been realized, the development of JSC should be prioritized so that the most relevant clinical questions can be addressed early in the drug development process. The parallel submission to the European Medicines Agency (EMA) and the EU-HTA-R secretariat also allows earlier initiation of the evaluation process. In principle, this should make clinical assessments available close to the time of marketing authorization, potentially accelerating procedures that are currently slow and heterogeneous across countries. However, such parallel processes require extensive and timely information exchange between regulatory and HTA evaluations, particularly when additional data or analyses are requested or when the initially claimed target population is modified.

The first Joint Clinical Assessment (JCA) dossiers were largely developed following early scientific dialogue with the EMA alone and therefore contain data primarily generated to meet regulatory standards aimed at determining the absolute benefit–risk balance of a new product. Consequently, defining PICO elements for already-submitted dossiers may appear somewhat constrained by the fact that pivotal trial design and available data are generally already established.

From a clinician's perspective, however, HTA-type questions remain highly relevant. Clinicians require sufficiently robust evidence to determine the place of a therapy within current treatment strategies and to assess the additional benefit of innovative products based on clinically meaningful outcomes such as overall survival, quality of life, organizational impact on healthcare systems, or broader public health benefits. In this context, defining PICO elements is particularly important:

1. Eligible patient populations: Treatment benefit may differ substantially depending on the line of therapy. A drug used in late-line treatment may demonstrate a different magnitude of benefit than when used earlier in the treatment sequence. Moreover, specific subpopulations defined by age, comorbidities, oncogenic drivers, MSI status, PD-1/PD-L1 expression, or other biomarkers may have distinct medical needs and appropriate comparators. These analyses require high-level, collegial, and independent clinical expertise.

2. Comparative evidence: Demonstrating robust additional benefit compared with standard treatments remains

challenging. Due to heterogeneity in clinical practice and access to comparators across Europe, discrepancies between Member States may arise. Potential methodological approaches include trials comparing against investigator's choice therapy, multiple comparator arms, or comparisons with external cohorts. This also highlights the importance of developing well-annotated European external cohorts with detailed clinical and biological individual-level data.

3. Outcomes: Endpoints should reflect patients' priorities and be based on pragmatic consensus approaches synthesized by European scientific societies for clearly defined clinical situations. For example, a recent study published in *JAMA Oncology* (doi:10.1001/jamaoncol.2026.0072) reported that among more than 600 patients over the age of 70 with incurable cancer, fewer than 10% preferred a treatment strategy that prolongs survival at the expense of quality of life.

These issues require robust clinical expertise that remains distributed across multiple stakeholders and could benefit from greater coordination and transparency as well as interactions between agencies, patient representatives, and scientific societies, and the process of harmonizing PICO definitions. Finally, beyond clinical evaluation, access to innovative treatments depends largely on pricing, funding mechanisms, and payer willingness. Greater coordination between health systems could promote more equitable access to innovation and contribute to better alignment with the objectives of the harmonized HTA-R framework.

EU HTA R – Early Insights and Ideas for the 2027/28 Revision – MS Perspective – host country Belgium

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Regulation EU 2021/2282 of the European Parliament and of the Council of 15 December 21 on health technology assessment and amending Directive 2011/24/EU. Art 31: Evaluation and reporting

Belgium participates in the following structures established under the European HTA Regulation: the Coordination Group, the Comitology Group and all four joint HTA subgroups, as well as the Brussels Centre for Collaboration in Health. Belgium is represented by two national institutions: the Federal Knowledge Centre for Health Care (KCE) and the National Institute for Health and Disability Insurance (RIZIV-INAMI).

Article 31 provides EU Member States with the opportunity to evaluate all joint HTA work carried out so far. The deadline for this evaluation is 13 January 2027. This process is expected to stimulate debate among Member States about which elements align with their national interests and expectations—and which do not. By 13 January 2028 at the latest, the European Commission is to present a report to the European Parliament and the Council based on these evaluations. Within the Coordination Group, Member States are already working toward a uniform reporting format for 2027 so that their positions can be expressed more effectively in the final discussions in 2027.

The text hereunder focuses exclusively on pharmaceutical HTA.

Regarding JCAs, Belgium has established the legal basis needed to integrate JCA reports into national assessments[1]. The Reimbursement Commission for Medicines will adopt bilingual national HTA reports in either English–Dutch or English–French. The European component of each report will be in English, while the national sections will appear in Dutch or French, depending on the pharmaceutical company's preference.

The national submission dossier has been redesigned to avoid duplication of evidence already submitted at the European level. The aim is for national submissions to follow soon after publication of the JCA report, since relying on outdated JCA findings would undermine the purpose of the reform.

Pharmaceutical companies will be allowed to get informed on the Belgian PICO on one occasion. This occurs after the European PICO has been finalized.

In the national assessment and appraisal, particular attention will be given to the added clinical value of a new product—an aspect not addressed in the JCA. Other national HTA components will include, among others, Belgian clinical pathways and guidelines, budget impact, and the pharmacoeconomic model. Beneluxa Member States are working on how interactions are feasible on the European HTA work they do. The new Belgian HTA legislation mentions the use of national reports (if available) from the Zorginstituut Nederland and the Haute Autorité de Santé in France[2]. The aim is to feed the appraisal stage. National reports from other Member States can be included in the future.

Finally, as RIZIV-INAMI already highlighted at the European Access Academy Convention in Brussels in October 2022, securing sufficient human resources to address all European HTA activities remains a major challenge. This item is likely to require further debate among Member States in 2027.

1) Royal Decree of May 25th 2025. Official Journal of Belgium June 13th 2025, pp 53195-53198;
2) In Dutch: https://www.riziv.fgov.be/nl/thema-s/verzorging-kosten-en-terugbetaling/wat-het-ziekenfonds-terugbetaalt/geneesmiddelen/farmaceutische-industrie/geneesmiddelenevaluatie-hta-samen-met-andere-europese-landen. In French: https://www.inami.fgov.be/fr/themes/soins-de-sante-cout-et-remboursement/les-prestations-de-sante-que-vous-rembourse-votre-mutualite/medicaments/industrie-pharmaceutique/evaluation-des-medicaments-hta-en-collaboration-avec-d-autres-pays-europeens.

EU HTA R – Early Insights and Ideas for the 2027/28 Revision – HTD Perspective

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Four years after the finalisation of the EU HTA Regulation, we are rapidly approaching the publication of the first JCA report, with several more to follow this year. Although there will be much to learn in the future, now is the right time to reflect on what has been achieved whilst keeping focussed on how we can all improve the process looking forward.

Currently we have a working system, but is it working for all in an effective way to make it a success? The IT platform is operational, methods and process guidance for assessors are published, and HTDs have made submissions based on the published template. The Coordination Group has been supported by many years of voluntary cooperation under EUnetHTA, although some positive learnings do not seem to have been incorporated into the JCA process. New HTA bodies have been established, and national processes are slowly adapting. But the current system is not an optimal system that works for all stakeholders, nor a system that makes Europe an attractive place for innovative technologies. We must therefore all continue to learn and adapt.

From a HTD perspective we are sharing insights and learnings. This not only helps us to ensure we are supporting each other on delivering the needs of the JCA but also identify common challenges and areas for potential improvement. We have seen increasing standardisation and information at the start of the procedure which is greatly welcomed. We also value the Q&A documents provided by the Coordination Group and Commission responding to emerging themes and ongoing dialogues regarding the process. However, the next critical stage of a JCA is the confirmation check of the dossier, and this has begun to identify where the system may require adjustment. We recognise the challenge of timelines that the assessors have to deliver the JCA report, but those challenges are also relevant for the HTDs who have a greater burden given the volume of analyses requested and tight timelines to deliver "missing" requests. In fact, the time to undertake the confirmation check is longer than the time HTDs are given to deliver the additional requests.

Our joint learning has revealed confusion whether the confirmation check is a "pre assessment" or a confirmation that the HTD has completed the dossier in full. These two need to remain separate, as envisaged by the Regulation. We also observe that many "missing" requests refer to the template and guidance documents - and that assessors and HTDs may have a different interpretation of what is being required. We should recognise that the dossier template and accompanying guidance were released without consultation and engagement from HTDs who will need to deliver on it. With no opportunity for a proper dialogue between the assessors and the HTD, which could resolve divergences and ensure the submission meets expectations, the current process is denying the Regulation a significant opportunity to succeed. We must therefore be careful not to blame HTDs when issues may be subjective and caused by differences in interpretation, requiring further clarity from the Coordination Group. Talk of penalties is premature and fails to acknowledge there may be other underlying causes that are not HTD related; they would, ultimately, make the European region less competitive to invest in.

Every one of us is learning – from HTDs that have never launched in Europe to those with considerable experience, as well as the individuals at HTDs and national HTA bodies working on different JCAs. This is going to take several years. As noted, many issues could be resolved with dossier preparation meetings and dialogue beyond exchange of letters on an IT platform that introduce delay - something industry has called for during the last few years and is a feature of many established HTA processes.

Let's make a system that works for all and involves all – we have joint responsibility and therefore together we are part of the solution. JCA is a considerable investment to HTDs that has a real opportunity cost; if there are no demonstrable improvements in patient access, JCA becomes an additional hurdle in Europe. This will mean the ambition of the Regulation will have failed. Unpredictability to HTDs and increasing requests for more data with no obvious benefit to downstream decision-making makes Europe less attractive as a region. We must keep focussed on the objectives of this Member State driven Regulation – facilitating patient access across the EU, reducing duplication, improving business predictability and supporting high quality, transparent decision-making. These must remain centre stage for the 2028 review and beyond - focussing purely on measurable process metrics at an EU level will undermine the potential of what the Regulation can achieve for European patients.

Sharing Insights on the International Horizon Scanning Initiative

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Under Article 22 of Regulation (EU) 2021/2282, the HTA Coordination Group (HTACG) is mandated to identify emerging health technologies expected to fall within the scope of Joint Clinical Assessments (JCAs). To fulfil this mandate, the HTACG's Subgroup for the Identification of Emerging Health Technologies relies on horizon scanning intelligence from the European Medicines Agency (EMA), the International Horizon Scanning Initiative (IHSI) and others.

IHSI's contribution to the EAA Spring Convention will describe IHSI's methodology for identifying pharmaceutical products likely to enter the JCA procedure. Drawing on publicly available data, including clinical trial registries, regulatory signals, and structured outreach to Health Technology Developers, IHSI maintains a forward-looking database of medicines expected to reach the European market. Products are systematically assessed against the scientific specifications agreed by the HTACG to determine their potential JCA eligibility, currently covering oncology medicines and advanced therapy medicinal products, with scope extending to orphan medicinal products from 2028.

JSC & JCA – Legal Considerations

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The Regulation (EU) 2021/2282 on Health Technology Assessment (EU HTA Regulation) explicitly states that Member States may draw their own conclusions on the relative effectiveness of health technologies. However, this national decision-making prerogative regarding the substantive and formal consideration of the Joint Clinical Assessment (JCA) is subject to limits: since EU HTA reports consist essentially of scientific factual analyses, accurate findings — such as those on effect sizes and endpoints — are factually binding on national HTA institutions. Their formal right to deviate from the data-based scientific conclusions of the JCA report may only be exercised on the basis of demonstrable errors, more recent data, or binding national legal requirements; absent such justification, any deviation may constitute a justiciable violation of the obligation to take due account of the JCA report.

A duty of support can be derived from Art. 4(3) TEU, requiring Member States to actively promote the objectives of the European HTA framework. Member States are therefore obliged in particular to notify PICOs that enable them to adopt the findings of the JCA as comprehensively as possible. This raises, among other things, the question of whether a Member State is bound by its PICO submission — and in particular by its chosen comparator therapy — or whether it may disregard these in national proceedings by invoking subsequent developments.

Furthermore, the provisions governing Joint Scientific Consultations (JSC) warrant critical scrutiny:

- Does the budgetary cap on consultation slots give rise to a heightened duty to state reasons for rejections, along with a transparent selection process based on the prescribed criteria?
- Can the undue disadvantage faced by companies that were not granted a consultation be mitigated through publication of consultation outcomes?
- Under what conditions may Member States depart from consultation outcomes?

Finally, the question is examined of which breaches of obligation are legally challengeable and thus capable of grounding judicially enforceable claims for the affected undertakings.

The CORE-RWE Methods Research Project

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As precision medicine and real-world evidence (RWE) reach a critical juncture, we at Vlerick HMC, in collaboration with KU Leuven, are launching the CORE-RWE (Causal Observational Research for Effectiveness using Real World Evidence) Methods Research project.

CORE-RWE will build the methodological foundation for using real-world evidence in health technology assessment and outcome-based payment decisions. By integrating Causal Machine Learning with Target Trial Emulation, it aims to deliver credible, comparable, and policy-relevant effectiveness estimation methods for high-impact medical innovations.

CORE-RWE's goal is to leverage current sentiment across Europe to develop a unified approach to assessing, evaluating, and implementing outcome-based payments for medical therapy technologies based on Real-World Evidence (RWE). From a geoeconomic perspective, this initiative will serve as a consortium for methodological research partnerships among biopharmaceutical and MedTech companies, in collaboration with European Health Technology Assessment (HTA) bodies. Our broader aim is to enhance Europe's competitiveness in accessing biopharmaceuticals and medical technologies by reliably and sustainably validating health innovations through RWE.

CORE-RWE aims to uncover the real-world effectiveness of drug or device-based therapies while designing innovative payment models based on their claimed performance through risk-sharing agreements. It addresses important policy questions, such as how much real-world evidence is sufficient for treatment access and whether and how causal machine learning can enhance the credibility of comparative estimates for cell and gene therapies, managed chronic precision therapies, and medical device interventions that involve structural and information-gathering components, especially when direct randomised controlled trials (RCTs) are impractical or provide unreliable initial pre-market therapy outcome data.

CORE-RWE will conduct research to validate the use of Causal Machine Learning (CML) within a Target Trial Emulation (TTE) framework. This study will apply the Bayesian Comparative Effectiveness method and utilise synthetic population-based disease models to evaluate two types of therapies: a single-administration curative therapy and a complex adaptive longitudinal therapy in both the biopharmaceutical and MedTech fields.

The EAA Spring Convention's adjunct session "How to leverage RWD for HTA – conceptual overview & case studies" will focus on the challenges and opportunities that RWE presents for conducting outcome assessments. We will emphasise the importance of designing a robust evaluation methodology that fosters trust among all parties involved. To illustrate these points, we will provide examples from both the biopharma and MedTech sectors.

European HTA and the three Pillars of Evidence-based Medicine – What needs to be considered?

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The European Health Technology Assessment (HTA) regulation covers Joint Scientific Consultations and Joint Clinical Assessments, which are based on evidence-based medicine (EBM). EBM rests on three pillars: the quality of the evidence, the patient's perspective and then, the clinician's perspective. Evaluating these pillars is not straightforward, because they are not equal – their respective influence varies depending on the context. Importantly, with their influence differing in practice, it is not possible to pre-specify exact weights and they need to be balanced appropriately for every clinical situation.

- Meningococcal meningitis: Here, the quality of evidence is overwhelming: amoxicillin 1.2g/day is clearly indicated. Patient or clinician preferences have little or no role, except in cases of amoxicillin allergy. The pillars are therefore very unequal in prescribing decisions.
- Dyspepsia or irritable bowel syndrome: Evidence is weaker, while patient and prescriber perspectives are more relevant. In these cases, the pillars are more balanced, with clinical and patient input carrying significant weight.
- Migraine prevention and non-hormonal treatment of vasomotor symptoms: In these examples, the discrepancy between the modest effects observed in trials and high patient-reported satisfaction creates tension and a balance tipped towards patient (and clinician's perspective) with less weight on the data. The difference itself is troubling and can make the final evaluation uneasy, though this does not mean patient satisfaction should automatically outweigh trial results.
- Early-stage prostate cancer: Here, the evidence is robust, but the choice between different treatments (watchful waiting, surgery, radiotherapy, or drugs) depends heavily on patient preferences and clinician advice. The pillars are complementary and inform each other.
- Late-stage cancers: In these cases, decision-making follows a temporal sequence: the process begins with scientific evidence, which is then considered in light of the patient's condition, medical history, and therapeutic journey. Clinicians integrate these factors to guide the final decision. The pillars are utilized sequentially.

The key to a good HTA evaluation is the appropriate consideration of all three pillars, with each participant – assessor, patient, and clinician – understanding the contributions of the others. Burden of disease and unmet medical needs alone are not sufficient reasons for access without a thorough evaluation of evidence, patient, and clinician perspectives.

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Outlook EAA Fall Convention 2026 in Lisbon, Portugal

A multi-stakeholder Glance into the Initial EU HTA Assessments

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The EU HTA Regulation (EU HTA R)[1] covers two key elements – Joint Scientific Consultation (JSC) and Joint Clinical Assessment (JCA) – with JSCs remaining confidential and JCA reports to be available in the public domain. First JCA procedures for medicinal products in oncology and advanced therapy medicinal products (ATMPs) have commenced in January 12th, 2025. The list of ongoing medicinal product JCAs is available online[2]. The reports for the first JCAs covering disease areas such as melanoma, paediatric glioma, bladder cancer, spinal muscular atrophy, or small cell lung cancer are expected to be published in May/ June 2026. At the time of the EAA Fall Convention in Lisbon (i.e., November 19/20th) a number of reports will be in the public domain – so this will be excellent timing to exchange procedural insights and share initial learnings from the outcomes.

As part of the public plenary session an overview of assessments and key learnings will be provided including the patient, regulatory, statistical/ methodological, clinical and health technology developer perspectives. Subsequently, an analysis of the unmet medical need in each of the disease areas, an overview of respective clinical guideline recommendations, outcomes of the applied consolidated scoping schemes, and a review of the methodological approaches will be the basis for an in-depth discussion of available assessments.

Reflecting the multi-stakeholder dimension of the evidence-based medicine triad (data & evidence; patient perspective; clinical perspective) the aim of those discussions is to broaden the scope beyond the purely technical assessments of available evidence and including the humanistic components of patients/ patient associations as well as clinicians/ clinical guideline committee representatives and other stakeholders and collaborators involved in the process.

Article 31 of the EU HTA R stipulates that no later than Jan 13th, 2027, Member States shall report to the commission on their consideration of the joint work. In light of this upcoming milestone, EAA's Fall Convention 2026 will summarize procedural learnings and also put a particular emphasis on Member State reflection on the initial assessments.

The EAA Fall Convention 2026 will be held at the premises of and be co-hosted by the Portuguese HTA body 'INFARMED'. A unique feature of INFARMED is that it combines both, regulatory and HTA competency within one umbrella organisation. Such integrative perspective is matching the multi-stakeholder scope of EAA's overall approach. Furthermore, EAA's fall convention will be co-hosted by the European Patients' Forum (EPF) and preparations will be supported by the European Society of Medical Oncology (ESMO) and their respective guideline committee representatives to ensure scientific excellence in an increasingly competitive global environment.

We are very much looking forward to a successful EAA Fall Convention 2026 in Lisbon.

1) https://eur-lex.europa.eu/eli/reg/2021/2282/oj;
2) https://health.ec.europa.eu/health-technology-assessment/implementation-regulation-health-technology-assessment/joint-clinical-assessments_en

Contributors & Imprint

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