European Access Academy®

EPF European Patients Forum



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EAA Convention Proceedings

A Multi-Stakeholder Glance into the Initial EU HTA Assessments

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Convention Agenda

* The EAA Fall Convention 2026 takes place on November 19/20th at INFARMED, Lisbon, Portugal.
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Thursday, November 19th, 2026:

Public Session

14:30 Welcome Message from the Host *(Rui Santos Ivo, INFARMED)*

15:00 Plenary Session 1 – Where is EU HTA heading? – The Overall Strategy

(moderated by Valentina Strammiello, EPF & Jörg Ruof, EAA)

- Political Perspective *(Marta Temido, MEP; virtual)*
- EU HTA & JCA Status Update from the Commission *(Valentina Barbuto, DG Santé)*
- Overview from the Coordination Group *(Niklas Hedberg, TLV; virtual)*
- Overview from a Patient's Perspective *(Valentina Strammiello, EPF)*
- Overview from a Clinician's Perspective *(Bernhard Wörmann, DGHO & EHA)*
- EU HTA: Impact on Europe's stance in innovative medicines *(Alexander Natz, EUCOPE)*

followed by Plenary Discussion

16:30 Coffee Break & Group Photo

17:15 Plenary Session 2 – EU HTA State of Play – Initial JCA Experience (Roundtable)

(moderated by Mariane Cossito, INFARMED & Dirk Arnold, ESMO)

- Learnings EMA – EU HTA Collaboration *(Noémie Manent, EMA)*
- Patient Involvement *(Solène Jouan, EPF)*
- Medical Society Involvement *(Robin Doeswijk, EHA)*
- Member State Assessor Perspective *(Belén Torres Garrido, AEMPS)*
- Procedural Experience: HTD Perspective *(James Ryan, efpia & AstraZeneca)*

18:45 Summary & Wrap Up *(Rui Santos Ivo, INFARMED & Jörg Ruof, EAA)*

19:00 End of Public Session

Friday, November 20th, 2026:

EAA Working Session

08:30 Welcome *(Tiago Rodrigues, INFARMED & Bernhard Wörmann, DGHO & EHA)*

08:35 EU HTA: Current State of Play and Insights from the Coordination Group *(Marco Marchetti, AGENAS & HTACG; virtual)*

08:50 JCA: Applied Methods Overview *(Sara Couto, INFARMED & Martin Scott, numerus)*

09:10 Roundtable: JCA Reports Revisited

Clinical Guideline Overview & Top-line Methodological Commentaries on Initial JCAs

(Kleoniki Roka/ Janbernd Kirschner (virtual)/ Anne-Marie Dingemans)

09:45 Break-Out Sessions: JCA Reports Revisited

Introduction *(Elaine Julian, EAA)*

1) Low-Grade Glioma *(Anita Granero/ Kleoniki Roka/ Robin Doeswijk)*
2) SMA *(Susanne Goldbach/ Janbernd Kirschner/ Markus Frick)*
3) ES-SCLC *(Merel Hennink/ Anne-Marie Dingemans/ Maureen Rutten)*
4) Breast Cancer *(Ingrid Krücken/ Dario Trapani/ James Ryan)*

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

11:45 Report from Break-Out Groups *(moderated by Valentina Strammiello, EPF & Robin Doeswijk, EHA)*

12:45 Outlook Spring Convention Budapest & EAA Pulse Check *(Jörg Ruof & Elaine Julian, EAA)*

12:50 Closing Comments *(Tiago Rodrigues, INFARMED & Jörg Ruof, EAA)*

13:00 End of Convention

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Editorial

A Multi-Stakeholder Glance into the Initial EU HTA Assessments



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As of mid-September 2026, 16 EU HTA Joint Clinical Assessments (JCAs) are ongoing, three have been discontinued and four are finalized. The eagerly awaited initial JCAs are all related to Orphan Designation Medicines: three oncological conditions (Tovorafenib in pediatric low-grade glioma, pLGG; Lurbinectedin and Tarlatamab both in extensive stage small cell lung cancer, ES-SCLC) and spinal muscular atrophy (SMA, intrathecal application of Onasemnogene abeparvovec). Among the three discontinued JCA procedures two of the submitted dossiers failed to meet the requirements laid down in the EU HTA regulation and for Sasanlimab in bladder cancer the marketing authorisation application was withdrawn by the health technology developer (HTD). At the EAA Fall Convention 2026 in Lisbon, we aim to 'bring the pieces together' and reflect the early EU HTA insights from a multi-stakeholder perspective.

The initial assessments harbour a variety of methodological challenges e.g.,

- In their national decision-making, EU member states will need to balance the risks of type 1 error (falsely assigning an additional benefit) and type 2 error (not acknowledging an existing additional benefit). However, based on the single-arm evidence package and the JCA assessment for Tovorafenib this will be particularly challenging, considering the indication of paediatric patients with low grade glioma, a condition with an unquestionably high unmet medical need.
- Conversely, for the intrathecal application of Onasemnogene abeparvovec a randomised controlled trial vs sham injection was provided. However, only one out of the twelve suggested Population-Intervention-Comparator-Outcomes (PICO) schemes could be addressed with such RCT evidence, clearly highlighting i) the challenges to meaningfully address multiple PICO schemes and ii) the increasing relevance of indirect evidence in an EU HTA context.
- Interestingly, heterogeneity across regulatory and HTA approaches are surfacing e.g., with the application of different data cuts for the regulatory vs HTA assessment of Lurbinectedin, a different approach to extrapolation of trial outcomes, that the European Medicines Agency (EMA) explicitly supports in their assessment of Onasemnogene abeparvovec, or the consideration of subsequent therapies that is suggested in the JCA of Tarlatamab.

The overall aim of the EU HTA Regulation is to assess medicines regarding their additional benefit vs current standard of care and accelerate access to such medicines across the Union. Furthermore, as stipulated by recital 1 of the regulation, strengthening a viable and globally competitive industry within the developing European Health Union is at the core of the regulation. A couple of aspects should be considered in this context.

- Scoping Schemes: Suggesting twelve (originally 14) PICO schemes for a condition such as SMA appears overengineered. Three data packages including direct and indirect evidence were submitted by the HTD addressing seven of the PICO schemes. The pressure is on both sides: the HTDs to provide meaningful evidence to support additional benefit claims and the HTA bodies to align their data requirements and provide manufacturers with a clear and feasible scoping guidance. The bottom line is that a plethora of PICO schemes will carry the risk of failing on strengthening the EU Health Union in an increasingly competitive global environment
- Indirect Evidence: Evidence from indirect treatment comparisons, network meta-analyses etc. will play an important role in

the future of EU HTA assessments. Similar to regulatory practice, the totality of available evidence should always be taken into account for HTA. Artificial Intelligence may help to synthesise such data. However, drawing conclusions on relative effectiveness from such data may be difficult for the member states that are in charge of national decision making.

- Finally, the wider/ international context always needs to be considered. With the 'Most Favoured Nation' (MFN) policy prevailing in the US and HTDs expected to behave rationale (i.e., in line with their shareholders' expectations), launch sequence across the globe might be revisited, with Europe at a high risk to falling further behind. Having the most sophisticated HTA system globally but at the same time a delay regarding submitted product applications, i.e. access to products in the EU, is no attractive outlook. Therefore, the vision of the regulation as stipulated in recital 3: 'best outcomes for patients and society as a whole' mandates a strong commitment by the member states and the commission to support and accelerate access of patients for medicines with an additional benefit.

Supporting a successful implementation of the regulation by means of a scientific multi-stakeholder dialogue based on the principles of evidence-based medicine is the 'raison d'être' of the European Access Academy (EAA). The European Patients' Forum (EPF) is co-hosting the convention, ensuring a strong patient voice within EAA's multi-stakeholder initiative.

We are very much looking forward to the EAA Fall Convention 2026 in Lisbon which will be held at the premises of INFARMED. Together with AIFA in Italy and NOMA in Norway the Portuguese INFARMED is covering both, national regulatory and national HTA authority. As the two processes, JCA and EMA's regulatory assessment largely occur in parallel, the national authorities that cover both authorities are very well suited to reflect on the successful implementation of the regulation in these early days.

EAA Convention Speaker Abstracts

Where is EU HTA heading? Overview from the Coordination Group

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Over the past year, the HTA Coordination Group (HTACG) has moved from preparing the EU HTA framework to operational implementation. One main focus is now on making joint clinical assessments (JCAs) a routine part of market access for innovative technologies across Europe.

Until October 5, 2026, no less than 23 JCAs have been started, of which 4 have been published on the EU Commission website, another 15 are ongoing and 4 have been discontinued[1].

Until 2028 all the JCAs are undertaken within the therapeutic areas of oncology drugs or advanced therapy medicinal products (ATMPs). The prediction of the number of JCAs is difficult and the 2026 work programme foresaw around 50 medicine JCAs. That number now seems a bit higher than the actual processes initiated so far in 2026.

Over the last year the HTA CG also expanded the work on joint scientific consultations (JSCs) and opened four request periods with a total of 8-12 available consultations for medicinal products and 2-5 for medical devices.

The HTACG increased the involvement of patients, carers and clinical experts in JCAs and JSCs.

The first published JCAs have generally been welcomed as an important milestone in the implementation of the EU HTA Regulation. Stakeholders have highlighted the achievement of delivering the first assessments on time and establishing a transparent, common European evidence review process. At the same time some comments indicate that the focus is now shifting from procedural implementation to the quality, interpretation and consistency of assessments across therapeutic areas.

Looking ahead, the direction of travel is clear: greater scale, broader scope and stronger coordination. HTACG also strengthens methodological guidance, horizon scanning of emerging technologies, stakeholder engagement and cooperation with the European Medicines Agency (EMA).

In short, EU HTA is evolving into a permanent, predictable and increasingly influential evidence platform. National HTA bodies will continue to make reimbursement decisions, but clinical evidence generation and assessment are becoming progressively more coordinated at EU level, reducing duplication and creating a more harmonised market access environment.

1) https://health.ec.europa.eu/health-technology-assessment_en

Where is EU HTA heading? Overview from a Patient‘s Perspective

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With four Joint Clinical Assessment (JCA) reports for pharmaceutical products already delivered and more in the pipeline, EU HTA is now operating in full swing.

As patient representatives, we worked intensively throughout the negotiations that led to the HTA Regulation, striving to inform and shape the legislation. Today, an important question remains: is the Regulation fully meeting our expectations?

The honest answer is no.

Can it be improved? Certainly. And we are confident that it will be, because the commitment and willingness to make this unprecedented collaborative effort succeed are clearly there. However, any future evaluation of the Regulation will represent a critical turning point. It must be grounded in a robust scientific methodology and focus first on assessing the process before attempting to measure its broader impact. Only in this way can we ensure the long-term sustainability and effectiveness of the framework.

Patients and patient organisations need to see tangible improvements in the areas we have consistently highlighted. This is essential if we are to maintain and strengthen our commitment to this major collective endeavour.

When reflecting on the journey that brought us here, involving all stakeholders and policymakers, I often describe the past twenty years as a remarkable exercise in capacity building and team building. It has been a period during which all of us had the opportunity to better understand the perspectives, challenges and priorities of other actors in the system.

As the European Patients' Forum, we have always supported the spirit and ambition of the legislation. We recognised from the outset its potential to reduce duplication of effort among HTA bodies and to contribute to a more harmonised access to innovative health technologies across Member States.

Today, the Regulation has become even more relevant. The European Union faces growing pressure to strengthen its competitiveness while also responding to external challenges, including the threat posed by Most Favoured Nation pricing policies in the United States.

In this context, reducing patients' access to innovation is a risk Europe cannot afford. Our greatest strengths remain our universal healthcare systems and the scale of our single market. Joint Clinical Assessments are a key instrument for translating those strengths into coordinated action across Europe.

The current global climate generates uncertainty and frustration for patients. For many, access to healthcare is not simply a policy issue; it is directly linked to their quality of life and, in some cases, to their chance of surviving longer. This is why making the EU HTA framework work effectively is not only a regulatory objective but a moral imperative.

Where is EU HTA heading? Overview from a Clinician‘s Perspective

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Access to new drugs within the EU is highly variable. Access rates range from 20 to >90%. The new EU HTA procedure aims at acceleration and harmonization of the assessment of additional benefit. The first 4 published JCA reports highlight some of the current challenges:

- high number of subpopulations
- high number of PICOs
- low number of medical experts
- low number of carers.

PICOs don’t fully align with current guideline recommendations in Europe.

The first results reflect the heterogeneity with the EU. It's unclear whether and how the current procedures will result in faster and easier access to new drugs.

EU HTA: impact on Europe's stance in innovative medicines

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The implementation of the Regulation (EU) 2021/2282 on health technology assessment has now reached a critical milestone with the publication of the first handful of JCA reports. Over the coming 18 months, we should now direct full attention towards ensuring the readiness of EU HTA for Orphan Medicinal Products (OMPs), that will become subject to JCA from 13 January 2028.

At EUCOPE we are actively supporting our member companies, the small and mid-sized innovative pharmaceutical companies, with navigating the procedures. We are also, as member of the HTA Stakeholder Network preparing for the upcoming review of the Regulation in 2028, by gathering experiences from our member companies.

The first phase of implementation of EU HTA has been a period of learning and adapting for everyone - and we have seen that the majority of companies with products subject to JCA has been small and mid-sized ones.

Since Orphan Medicinal Products also tend to be developed by smaller companies, we should take careful note of ways that we can make the procedure equally manageable for those companies that might have smaller market access teams, and fewer resources at hand, to prepare for and successfully navigate the JCA.

Additionally, we should draw lessons from the first JCAs to ensure the specificities of OMPs are recognised in the EU HTA guidance documents, as foreseen in Regulation (EU) 2021/2282 on health technology assessment.

The ultimate success of the EU HTA procedure remains to be seen, based on the use of the JCA reports for national decision-making to inform national HTA procedures and access decisions.

However, with the upcoming revision, we should already identify ways to ensure we meet the aims of reduced burdens for companies and HTA bodies, to achieve the benefit of the "one-stop-shop" for market access in Europe.

EU HTA – State of Play
Initial JCA Experience

Learnings EMA– EU HTA Collaboration

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EMA collaborates with the European Commission and the HTA Coordination Group by providing specific information as required by the HTA Regulation (EU) 2021/2282. The aim is to support the work of the coordination group and its subgroups. The exchange of information, as detailed in the Implementing Regulation (EU) 2024/1381, related to marketing authorisation applications and extensions of indications at pre-authorisation stage and at the time of the evaluation will be discussed. Insights on how the collaboration enables the JCA preparatory phase to start in parallel to the MAA pre-submission phase, but also facilitates the adherence to timelines will be reviewed through the experience gained so far.

Patient Involvement

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The publication of the first Joint Clinical Assessments (JCAs) under the EU Health Technology Assessment Regulation (HTAR) marked an important milestone towards a more coordinated, transparent and patient-centred European HTA system. It also provided a first opportunity to assess how the new framework translated into practice, particularly regarding the meaningful involvement of patients and carers.

Drawing on this initial experience, the European Patients' Forum (EPF), together with other patient organisations represented in the EU HTA Stakeholder Network, decided to reflect on the progress achieved, the challenges identified and the lessons learned, with a view to continuously improving the process for future assessments.

With our colleagues from other patient organisations, we particularly welcomed the efforts made to involve patients and carers in the first JCAs, including opportunities to contribute to the assessment scope and comment on draft reports.

However, this initial experience also highlighted several areas where patient involvement could be further strengthened to ensure that patient perspectives genuinely influenced the assessment process and its outcomes. In particular:

- We emphasised the importance of involving patients and carers from the earliest stages of the assessment process, including the preparatory phase and the definition of the assessment scope.
- We also called for broader and more representative patient involvement, recognising the important role that patient organisations could play in bringing collective expertise and perspectives from the wider patient community.
- Greater transparency was identified as another priority, particularly regarding how patient contributions were considered and how they influenced the assessments' conclusions.
- Beyond patient involvement, we highlighted the need to strengthen evidence generation on outcomes that matter most to patients, including quality of life, treatment burden, functional outcomes and patient-reported symptoms. The first JCAs also raised broader methodological questions, particularly for disease areas where robust comparative evidence was difficult to generate, underlining the importance of exploring additional evidence sources while maintaining scientific rigour.
- We also stressed the importance of making JCA reports more accessible and understandable to patients and the wider public, including through plain-language summaries and translations into all official EU languages. This is particularly important to ensure that the results of European assessments could be understood and meaningfully inform national HTA and reimbursement decisions.

Since our initial analysis, some improvements have already been introduced in the process, particularly regarding the involvement of patient organisations in Joint Scientific Consultations. While this development is encouraging, one important challenge remained: the sustainability of patient organisations' contributions, which required considerable time, expertise and resources but remained largely unremunerated. Ensuring meaningful and sustained patient involvement therefore required not only formal opportunities to participate, but also adequate support and resources to enable patient organisations to contribute effectively over time.

Medical Society Involvement

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Few will still doubt that medical societies have an important place in EU HTA. The most eye-catching role of societies is the identification and nomination of clinical experts for the joint clinical assessments and joint scientific consultations. They do much more: They engage with each other, spreading awareness, ensuring alignment and exchanging best practices. They connect and collaborate with other stakeholders, in particular patient organizations but also industry federations to discuss topics of common concern such as conflict of interest (COI). They inform and educate their members about the what, why, and how of HTA. They encourage their expert communities, at the European and national levels, to interact with their national HTA bodies. They are part of, and drive, discussions with regulators, policymakers and payers as well with HTA bodies about the importance of aligning evidence generation criteria and processes across regulatory and access pathways.

Many medical societies have established or are developing ways of responding swiftly and effectively to requests for expert identification from the HTA Secretariat, hosted by the European Commission. They flag procedural or practical obstacles either directly with the Commission or through the HTA Stakeholder Network Working Group for the Identification of Patients and Clinical Experts. Interaction with the HTA Secretariat is, in EHA's experience, constructive, efficient and effective. Concerns and suggestions by clinical and patient associations in the Working Group are discussed and addressed in earnest by Commission as well as JCA/JSC Subgroup representatives.

Whilst there is broad acceptance and recognition by now of medical societies as important stakeholders in EU HTA, including by HTACG leadership, much of the potential of societies' involvement remains untapped.

When EHA proactively facilitated the formulation of a quick, concise, consensus-based PICO advice by its expert community, the moment we became aware that the first JCA in a hematological malignancy was being launched, this collective expert opinion was shared by the HTA Secretariat but not accepted by the JCA Subgroup.

There have been worrying signals that top experts, identified and encouraged to make themselves available by EHA and other societies and subsequently formally nominated by the HTA Secretariat, were not accepted and passed over by the Subgroups, without any feedback or motivation.

Whether this reflects a lack of trust, lack of willingness or simply a clinging to old ways and contacts, the consequence is that Europe's new HTA mechanism does not benefit from medical societies' main added value: the collective expertise and consensus building of societies' highly specialized European expert networks, in which all individual expertise used in JCA or JSC should be embedded.

Procedural Experience from the HTD Perspective
Early Lessons from the First Joint Clinical Assessments

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Implementation of the European Union Health Technology Assessment (EU HTA) Regulation marks a significant shift in how clinical evidence is assessed across Member States. Joint Clinical Assessment (JCA) was intended to streamline evidence generation and reduce duplication, and improve patient access, but the first procedures have exposed important operational lessons for Health Technology Developers (HTDs). Early experience suggests a process that is workable in principle but must evolve in execution and content.

The JCA process has integrated successfully with the European Medicines Agency (EMA) regulatory pathway. The first published assessments operated broadly within anticipated timelines and showed increasing consistency as experience accumulated. However, variability remains around final publication, partly because privacy and trial-integrity redactions require discussion among the HTD, EMA and HTA Secretariat. This uncertainty affects HTD and national HTA body planning, resource allocation and the timing of downstream national HTA activities.

Scoping is one of the strongest drivers of procedural complexity and workload. Decisions on populations, comparators and outcomes also expand dossier volume and analytical demands substantially. HTDs report that scope explanation meetings remain largely procedural, without sufficient discussion of PICO rationale, acceptable methods or major uncertainties. Earlier and ongoing meaningful dialogue would improve dossier quality, efficiency and mutual understanding.

Assessor information requests are another recurring challenge. Although response periods may range from seven to thirty calendar days, HTDs report that most requests require answers within seven days, irrespective of complexity. Their timing is also unpredictable and may coincide with intensive regulatory work or staff absence, placing pressure on HEOR, market access, statistical programming, regulatory and clinical teams. Methodological expectations are not always made clear during review, yet reasonable interpretations of guidance may later be criticised in reports. Advance notice and real-time clarification could improve quality without extending timelines.

Publication of JCA reports, dossiers and appendices creates unprecedented disclosure, with document packages running to thousands of pages. The complexity and volume of requirements strain all HTDs and may disproportionately affect smaller companies, potentially becoming a barrier to entering Europe. Rather than reducing burden, JCA currently adds substantial cost, while only limited offsets are expected at national level. Disclosure obligations under EMA Policy 70, JCA transparency requirements, privacy law and trial-integrity protections are difficult to reconcile. Positively, coordination between the HTA Secretariat and EMA has reduced the risk of contradictory reporting, demonstrating the value of a consistent publication approach.

Published reports focus heavily on uncertainty and bias, often prioritising methodological purity over pragmatic interpretation and balanced discussion of strengths and limitations. They provide limited context on disease burden, evidence-generation constraints or areas where clinical practice itself rests on weak evidence. Consequently, reports may be perceived as selective methodological assessments rather than decision-support tools. In fact, when the almost unanimous consensus across stakeholders, and most importantly patient

groups in response to the first few JCA reports is that they are inaccessible and risks having the opposite effect, we all need to pay attention and be flexible enough to adapt. In its current form, it therefore remains imperative that national HTA bodies and other stakeholders consistently consider the HTD's JCA and national dossiers alongside the report; reliance on the report or summary alone risks poorer-quality decisions about patient access.

National implementation has also raised questions about using endorsed JCA reports before formal publication. Waiting may delay national procedures or cause duplication of EU-level work. HTDs support national bodies using endorsed reports as soon as they are available, which would advance the Regulation's aims of reducing duplication, increasing predictability and improving patient access.

Overall, the first JCAs show that the EU HTA framework can operate alongside existing regulatory processes. Yet producing and publishing a joint report is not, by itself, success; its value will depend on practical use by Member States, which may take several years to evaluate. Importantly, the Regulation also seeks to bring the right evidence to Europe, protect patient health and support the internal market. If implementation continues to favour mythical methodological purity, fails to acknowledge strengths of the evidence, overlooks clinical and evidentiary context – including orphan medicines and rare diseases, vaccines and advanced therapy medicinal products – and demands excessive analyses and reporting, JCA will undermine rather than advance those aims. It shows HTDs that Europe does not value what you do.

HTDs are making investment and evidence decisions right now and the current reports provide limited support to justify investing in European evidence compared to other regions. Europe now has a choice: JCA can be enabler of health technology innovation and investment, or it can be a disabler and barrier to entry, compromising European patient access and wider European initiatives to make Europe a place for life sciences.

Applied Methods Overview for the EU Joint Clinical Assessment

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Successful preparation for the EU Joint Clinical Assessment (JCA) depends on understanding evidence quality, analytical feasibility and the methods available to address the assessment questions.

This presentation provides an applied overview of statistical methods for JCA, focusing on how internal validity, risk of bias and data availability inform analytical choices.

Direct comparisons based on randomised controlled trials will be considered, alongside limitations arising from differences in study design, populations, endpoints and missing data.

Where direct evidence is insufficient, the presentation will explore indirect treatment comparisons, including population-adjusted indirect comparisons (PAICs), such as matching-adjusted indirect comparisons (MAICs) and simulated treatment comparisons (STCs). Key assumptions, the role of prognostic factors and effect modifiers, and the impact of data limitations will be discussed.

The emphasis throughout is on selecting feasible, appropriate methods, identifying critical uncertainties and prioritising analyses that provide credible, decision-relevant evidence within the JCA framework.

JCA Reports Revisited:

Clinical guideline overview &
top-line methodological commentaries
to the initial JCAs

Paediatric low-grade glioma (pLGG)

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Paediatric-type low-grade gliomas in the era of evolving targeted therapies

Pediatric-type low-grade gliomas (pLGGs) are the most common brain tumors in childhood. They are a heterogeneous group of different WHO2021 grade 1 and 2 tumors with a generally excellent overall survival. Despite the excellent prognosis, pLGG survivors may suffer from neurological, functional, endocrinological, and visual complications as a result of their disease or treatment. Furthermore, survivors might need several lines of therapy due to multiple relapses, which has an additional major impact on their quality of life and their families. Thus, pLGGs are considered a chronic disease.

Overview of relevant guidelines/ guideline recommendations

International recommendations from the SIOP Europe Brain Tumor Group and the RAPNO Working Group emphasize the multidisciplinary approach and maintaining quality of life over aggressive treatment. Maximal safe surgical resection is the primary treatment modality. For those tumors that are not amenable to surgery and have indications for treatment, first-line systemic chemotherapy is recommended for tumor control. Recent advances in molecular profiling of pLGGs have provided new insights into the RAS/MAPK pathway implicated in pLGGs, adding novel targeted therapies to our therapeutic arsenal. Currently, the EMA-approved first-line targeted treatments, if systemic therapy is required, are everolimus for pLGG patients with SEGA and the combination trametinib-dabrafenib for pLGG patients with BRAF V600 mutations.

Overview of the EU PICO and comparison/ alignment with the guidelines

Current published recommendations refer only to first-line treatment; thus, the EU PICO framework for the tovorafenib JCA, consisting of 8 distinct questions, led to inconclusive assessments. Providing comparative data in pediatric oncology seems extremely difficult because of the disease rarity and could delay or exclude patients' access to innovation.

An overall clinical perspective/ summary

Children with a relapsing or progressive pLGG have a high probability of requiring multiple lines of therapy alongside preservation of long-term sequelae and quality of life, and might not be able to wait for clinical trials to prove benefit. While clinical and regulatory control remains essential to ensure patient safety and evidence integrity, the EU HTA framework must evolve. Implementing adaptive, flexible pathways and real-world evidence ensures robust oversight without delaying the adoption of life-saving pediatric innovations.

Spinal Muscular Atrophy (SMA)

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Intrathecal onasemnogene abeparvovec has been approved for 5q-SMA in patients with Spinal Muscular Atrophy (5q-SMA) aged 2 years and older. The associated Joint Clinical Assessment (JCA) is the fourth procedure conducted under the EU HTA Regulation (EU HTAR) and the first assessment in an Advanced Therapy Medicinal Product, ATMP. The key clinical evidence supporting the Marketing Authorization by the European Medicines Agency is derived from the randomized, sham-controlled STEER study (HFMSE[1] difference: 1.88 points; 95% CI: 0.51–3.25); the Committee for Medicinal Products for Human Use, CHMP, extrapolated the clinical trial findings to milder phenotypes and adults. In contrast, the JCA reports evidence only for the population included in the clinical trial — namely, non-ambulatory patients aged 2 to under 18 years. Three data packages provide the evidence for addressing the 12 PICO questions in the JCA: i) a direct comparison against sham (as a proxy for best supportive care) and indirect comparisons against ii) nusinersen and iii) risdiplam. At the EAA meeting in Lisbon the findings from the clinical trial data, the European Public Assessment Report, EPAR, and the JCA outcomes, will be revisited leveraging the perspective of the European Guidelines for SMA treatment.

1) HFMSE: Hammersmith Functional Motor Scale Expanded

Extensive Stage – Small Cell Lung Cancer (ES-SCLC)

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Lurbinectedine and Tarlatamab, new drugs for the treatment of ES-SCLC

Extensive-stage small cell lung cancer (ES-SCLC) is an aggressive malignancy with rapid metastatic spread and historically poor survival outcomes. The current standard first-line treatment for patients with adequate performance status is platinum-etoposide chemotherapy combined with a PD-L1 inhibitor (atezolizumab or durvalumab), followed by maintenance immunotherapy.

The addition of immunotherapy has led to meaningful improvements in survival. In the IMpower133 study, median overall survival (OS) increased to **12.3 months** with atezolizumab plus chemotherapy versus 10.3 months with chemotherapy alone. In the CASPIAN trial, durvalumab plus chemotherapy achieved a median OS of **12.9 months** versus 10.5 months with chemotherapy alone. Long-term follow-up demonstrated durable benefit, with approximately **17-18% of patients alive at 3 years** compared with **5-6%** in the chemotherapy-alone arms.

At relapse, treatment selection depends on the chemotherapy-free interval. Platinum rechallenge may be appropriate in patients with sensitive relapse, while lurbinectedin, topotecan, or enrolment in clinical trials are important options for resistant or refractory disease.

Key HTA considerations

- Long-term survivors are emerging, with ~17-18% alive at 3 years after chemo-immunotherapy.
- No validated predictive biomarkers currently guide treatment selection.
- Access to effective second-line therapies remains limited.
- The balance between modest population-level gains and clinically meaningful long-term survival benefits is a key consideration for HTA assessments.

Emerging Strategies Beyond First-Line Immunochemotherapy

Lurbinectedin maintenance (IMforte): The phase III IMforte study evaluated maintenance lurbinectedin plus atezolizumab following induction carboplatin-etoposide-atezolizumab in ES-SCLC patients without progression after first-line therapy. Maintenance treatment significantly improved PFS, with a median PFS of approximately 5.4 months versus 2.1 months for atezolizumab alone (HR 0.54). Interim overall survival analyses have shown a favourable trend, although mature OS data remain awaited. These findings suggest that maintenance lurbinectedin may further delay relapse following chemo-immunotherapy and represent a potential new treatment option in ES-SCLC.

Tarlatamab (DeLLphi-305): Tarlatamab is a DLL3-directed bispecific T-cell engager targeting a protein expressed in >85% of SCLC tumors. In the phase III DeLLphi-305 trial, tarlatamab significantly improved outcomes compared with standard chemotherapy in previously treated SCLC with median OS 13.6 months vs 8.3 months (HR 0.60) and modest improvement of PFS 4.2 months vs 3.7 months (HR 0.71).

The safety profile was characterized primarily by cytokine release syndrome and neurologic events, which were generally manageable with established monitoring and mitigation strategies.

HTA perspective: Despite improvements with chemo-immunotherapy, median survival remains approximately one year. The emergence of new maintenance and second line strategies such as lurbinectedin and DLL3-targeted therapies such as tarlatamab may meaningfully extend disease control and survival, while also introducing new considerations regarding treatment sequencing, management of novel toxicities, and cost-effectiveness.

Outlook EAA Spring Convention 2027 in Budapest, Hungary

EU HTA – Member State Uptake: Central and Eastern European Perspective



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Article 31 of the EU HTA R[1] stipulates that no later than January 13th, 2027, the Member States (MS) shall report to the European Commission on their consideration of the joint work. The EAA Spring Convention 2027 is scheduled for April 15/16th in Budapest, Hungary. By that time the MS report as well as detailed, locally disseminated, information is expected to be publicly available and will inform the discussion at the convention. The focus of the convention will be MS uptake of the initial Joint Clinical Assessments (JCA) taking into account both, the aforementioned MS report as well as first-hand experience of various participating MS representatives. Furthermore, as the EAA convention is for the first time taking place in Central and Eastern Europe (CEE), a particular focus will be on the perspective and situation in the CEE region.

National uptake of the EU HTA assessments raises a variety of questions:

- How will MS approach the science / value dichotomy that is embedded in the regulation i.e., how does each of the MS translate the 'non-judgmental' European scientific assessment into its national appraisal framework[2]. This question is of particular interest for MS that don't have an established categorical framework such as the ASMR / SMR ordinal ranking system that is utilized in France or the German Additional Benefit classification system.
- As reiterated frequently in the EU HTA R, duplication of effort i.e., repetitive submission and subsequent evaluation of identical evidence on EU and MS level should be avoided. Nevertheless, MS may request information from the Health Technology Developer (HTD) that was not provided on the EU level and that is considered relevant for national decision making. Hence, design, content, and not least volume of such 'delta dossiers' warrant a thorough analysis to ensure successful implementation of the regulation and a reflection on efficiency gains on national level attributable to the EU HTA R.
- Downstream national pricing and reimbursement decisions are another area of high relevance. Pricing and reimbursement processes largely differ across the EU and are influenced by the global political environment, e.g., MFN. It remains to be seen how the EU HTA assessments will be used to inform such processes considering local filings by the HTDs. To leverage the established HTA wording: the additional benefit of EU HTA assessments to inform and optimize national pricing and reimbursement decisions needs to be evaluated.
- At the heart of the EU HTA R are two key intentions: i) to provide a scientific basis for MS to identify medicines and medical devices that harbour an additional benefit and ii) to facilitate and accelerate access of patients to medicines with a proven additional benefit. Hence, the ultimate question will be whether access to medicines with an additional benefit is accelerated across the MS. Achieving both of these intentions will be key in an increasingly competitive global environment.

1) https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=CELEX:32021R2282
2) https://pmc.ncbi.nlm.nih.gov/articles/PMC11587063/pdf/jmahp-12-00028.pdf

EU HTA key performance criteria, as developed and published by the EAA network, emphasize the necessity to include MS-specific factors including national health systems and budgets when assessing the overall performance and success of the regulation[3]. Furthermore, a detailed glance into the different perspectives of all involved stakeholders is required to obtain a reliable indication of the reality of the 'state of play'. The two initial measurement timepoints for the EAA Pulse Check revealed a widening gap between HTA body and HTD perception regarding the overall success of the implementation of the regulation[4].

A variety of joint EU health initiatives such as e.g., the EU4Health Program[5] are aiming to strengthen health systems across the CEE region. Within the EU HTA R the emphasis e.g., for scoping, is on the inclusive benefit for all MS' needs. Having a particular focus on the CEE region at the EAA Spring Convention 2027, will therefore be very timely, and we are thankful for Corvinus University to host the event at their premises.

We are warmly inviting you to join the EAA Spring Convention 2027 in Budapest.

3) https://pmc.ncbi.nlm.nih.gov/articles/PMC12821655/pdf/jmahp-14-00005.pdf
4) Julian et al, Pharmacoeconomics, submitted
5) https://health.ec.europa.eu/funding/eu4health-programme-2021-2027-vision-healthier-european-union_en

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