

Roundtable Discussion

Perspectives on the KPIs based on first experiences

- Patient Perspective (*Antonella Cardone, CPE*)
- Haematology Perspective (*Étienne Lengliné, EHA*)
- System Level/ Member State Perspective (*Frank-Ulrich Fricke, TH Nürnberg*)
- Health Technology Developers' Perspective (*Maria João Garcia, Roche*)

Performance Measurement Criteria from Patient Perspective

Insights from Cancer Patients Europe



Accessibility & Reduction of Delay

- No data yet available on KPI progress.



Incorporation of Patient Input

- CPE supported patient identification for JCAs/JSCs (several selected) → feedback: input not clear how and if considered. Patient input often gives the impression of a consultation exercise in evolution
- PICOs remain insufficiently patient-driven.



Informed, Patient-Centered Decision-Making

CPE survey on JCAs:

- **Positive:** accessible Secretariat, responsive support, development of materials (ex: factsheets).
- **Negative:** tight deadlines, almost no follow-up post-involvement.

Education & Training:

- **Ad hoc** education, based on identified gaps in the specific patients involved.
- **Goal:** bring the patients recruited up to speed with the EU HTA process (JSC and JCA).

Moving Forward Strengthening Patient-Centered HTA

- **Testing** a *patient-led* approach in EU HTA JCAs, based on identified gaps in patients' involvement.
- Ensure patient input shapes **PICOs, Plain Language Summaries, and final reports.**
- **Goal:** make patient perspectives **systematic, valued, and impactful** in EU HTA.
- **Capacity Building** among the patient community



KPI From Clinician / Hematology perspective

KPI related to the PICO framework

- **Patients:**
 - Unmet medical need (R/R last line) vs. others
 - Subgroups
 - **Intervention:**
 - Drug already available in a subsequent line of treatment (sequence)
 - Combination therapies
 - Maintenance treatments
 - **Comparator**
 - **Outcomes**
- Vs. JSC
 - HTA perspective

% of submitted dossier

JCA Measures:

- Adequacy with guidelines
- Heterogeneity accross SM

KPI From Clinician / Hematology perspective

KPI related to reducing uncertainty

- **Half to two third of new approval rely on non comparative design / intermediate outcome**
 - EU cohort of RWE for synthetic arms (grading the quality of annotation)
 - Surrogacy validation
 - QOL as outcome in non curative intent situation
- **Definition of supportive data requirement**
 - Earlier line / Combination / Proximity to the requested indication
 - Timeframe

KPI From Clinician / Hematology perspective

KPI related to Transparency

- **What**
 - Availability for Assessor / co-assessor vs. all MS vs. stakeholders
 - Patient level information availability
 - QOL as outcome in non curative intent situation
- **When**
 - Supplementary analysis (subgroups , heterogeneity , RMST etc..) for appraisal
- **Who**
 - Expert selection , validation , COI management
 - EMA LOQ availability

KPI From Clinician / Hematology perspective

Time and access

- **Defining consensual timepoints**
 - Assessment / Appraisal / Administrative decision
 - From application to the patient
- **Drugs with a clinical added value** vs. others Derogative access
- **Access for all** (inside and across member states) definition if there is OOP or hospital charges associated with some access.

Clinician involvement

Should be representative of the community (diversity)

Numbers of experts

Selection / Validation ?

Experts for assessment different from appraisal

Plenary Session Part 2 -Roundtable on KPIs for EU HTA – System/ Member State Perspective



KPI's from a system's/ MS perspective

| Health System Goal | System Level/ MS Perspective |
|---------------------------------|--|
| Health improvement | 1. PICO related KPI (Scope: Exhaustiveness) 2. Learning & training the system |
| Efficiency of the health system | 3. Reduce duplication of effort (Scope: Utilization of JCA report and reduction of workload) 4. Time to patient access [§] |
| Equity of the health system | 5. Equity of patient access (Scope: availability across MS contingent on budgetary capacities) [§] |

KPI's from a system's/ MS perspective

| System Level/ MS Perspective | Goal Attainment |
|--|--------------------------|
| 1. PICO related KPI (Scope: Exhaustiveness) | Current status of PICOs? |
| 2. Learning & training the system | Too early |
| 3. Reduce duplication of effort (Scope: Utilization of JCA report and reduction of workload) | Current status of JCA? |
| 4. Time to patient access | Too early |
| 5. Equity of patient access (Scope: availability across MS contingent on budgetary capacities) | Too early |

KPI's from a system's/ MS perspective

| System Level/ MS Perspective | Goal Attainment |
|--|--|
| 1. PICO related KPI (Scope: Exhaustiveness) | <ul style="list-style-type: none">• Number of PICOs in current JCAs? |
| 3. Reduce duplication of effort (Scope: Utilization of JCA report and reduction of workload) | <ul style="list-style-type: none">• Number of available JCAs?<ul style="list-style-type: none">• None ready yet• 2 JCAs on ATMPs• 8 JCAs on oncology products• 5 JCAs for orphan medicines (1 outside oncology) |

What are the number of PICOs in the first assessments?

| INN / Common Name | Substance type (classification) | Date of EMA validation of the MAA | Days elapsed | No. of PICOs |
|--------------------------|---------------------------------|-----------------------------------|--------------|--------------|
| Lifileucel | ATMP | 27.03.2025 | 225 | |
| Tovorafenib | Chemicals | 27.03.2025 | 225 | |
| Sasanlimab | Biologicals | 22.05.2025 | 169 | |
| Onasemnogene abeparvovec | ATMP | 22.05.2025 | 169 | |
| Lurbinectedin | Chemicals | 19.06.2025 | 141 | |
| Camizestrant | Chemicals | 19.06.2025 | 141 | |
| Tarlatamab | Biologicals | 17.07.2025 | 113 | |
| Catequentinib | Chemicals | 17.07.2025 | 113 | |
| Senaparib | Chemicals | 14.08.2025 | 85 | |

When can we expect the first JCAs?

| INN / Common Name | Substance type (classification) | Date of EMA validation of the MAA | Days elapsed | Expected completion |
|--------------------------|---------------------------------|-----------------------------------|--------------|---------------------|
| Lifileucel | ATMP | 27.03.2025 | 225 | 07.05.2026 |
| Tovorafenib | Chemicals | 27.03.2025 | 225 | 07.05.2026 |
| Sasanlimab | Biologicals | 22.05.2025 | 169 | 02.07.2026 |
| Onasemnogene abeparvovec | ATMP | 22.05.2025 | 169 | 02.07.2026 |
| Lurbinectedin | Chemicals | 19.06.2025 | 141 | 30.07.2026 |
| Camizestrant | Chemicals | 19.06.2025 | 141 | 30.07.2026 |
| Tarlatamab | Biologicals | 17.07.2025 | 113 | 27.08.2026 |
| Catequentinib | Chemicals | 17.07.2025 | 113 | 27.08.2026 |
| Senaparib | Chemicals | 14.08.2025 | 85 | 24.09.2026 |

EAA Fall Convention 2025

Measuring EU HTAR KPIs - HTD Perspective

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Disclaimer

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The views expressed are those of the author and do not necessarily reflect the views of the employer. Any mistakes are the responsibility of the author.

HTD Prioritized KPIs – Scope and Ambition

Insights from the EAA Spring Convention 2025

| Health System Goal | Prioritized KPI | Scope/Ambition |
|---------------------------------|--|--|
| Health improvement | PICO related KPI | Optimization of PICOs: To achieve convergence of clinical standards for HTA over time and enable HTDs to focus comparative evidence generation against the most relevant comparators across the EU. This provides clarity for future clinical development programs. |
| Efficiency of the health system | Functioning JSC process | Address demand & align timelines: JSCs are considered critical for HTDs to generate appropriate evidence for high-quality EU HTA submissions. The process must accommodate the dynamic nature of clinical development programs. |
| | Functioning JCA process | Workability and clarity: Requires predictability throughout the JCA process, from the starting date and final scope delivery through to the submission deadline. This includes realistic response timelines and efficient communication. |
| | Shorter duration of the national decision-making process | Streamlined national HTA and improved patient access: The upfront additional investment and analyses required by HTDs must be offset by more efficient national procedures and a shorter duration of the national decision-making process to ultimately improve patient access. |

HTD Prioritized KPIs – First Experiences

| Health System Goal | Prioritized KPI | First Experiences | |
|---------------------------------|--|--|---|
| Health improvement | PICO related KPI | Early experience shows inefficiency: Scoping process seems to be inefficient and the scoping explanation meeting is not leveraged for the benefit it could bring. |  |
| Efficiency of the health system | Functioning JSC process | Capacity, flexibility and predictability issues make it non-attractive: The call-based system is challenging and the under capacity makes the mechanism non-attractive and unfit for most companies' development plans. |  |
| | Functioning JCA process | System is ramping up: Operational 'growing pains' (related to planning, procedures, and timelines) have occurred but appear to be progressively remedied. |  |
| | Shorter duration of the national decision-making process | Uncertainty remains: No JCA report has been published yet; it remains unclear how consistently Member States will “give due consideration”, i.e. how they will adopt and integrate JCA reports in the national decision-making. |  |

Doing now what patients need next

