

Market Access and Pricing for Pharmaceutical Products and Medical Devices

Thomas Ecker

Navigating JCA – Where are we at month 20?

EFSPI Regulatory Statistics Workshop
20.08.2026



Agenda

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- 1 **What the first published JCA reports tell us**
Emerging patterns after the first completed assessments
 - 2 What the reports don't tell you
Practical lessons from supporting early JCA procedures
-

Overview of current JCA procedures

3

Completed

- Tovorafenib - Paediatric low-grade glioma (Orphan Drug)
- Lurbinectedin – ES-SCLC (Orphan Drug)
- Tarlatamab – ES-SCLC (Orphan Drug)

15

Ongoing

- 12 oncology indications
- 6 Orphan Drugs
- 4 ATMPs
- 1 accelerated assessment

3

Withdrawn/
discontinued

Withdrawn MAA:

- Sansalimab

Discontinued (Dossier failed to meet requirements):

- Catequentinib
- Tacquell



Tovorafenib addresses a rare paediatric cancer with substantial unmet need and limited treatment options



Submitted Label

Tovorafenib is indicated as monotherapy for the treatment of patients 6 months of age and older with **paediatric low-grade glioma (LGG)** harbouring a **BRAF fusion** or **rearrangement**, or **BRAF V600 mutation**, who have **received one or more prior systemic therapies**



Final label

Tovorafenib is indicated as monotherapy for the treatment of patients 6 months of age and older with **paediatric LGG harbouring a BRAF fusion or rearrangement**, or **BRAF V600 mutation**, who have **progressed after one or more prior systemic therapies**

- **Assessor:** National Centre for Pharmacoeconomics (NCPE), Ireland
- **Co-assessor:** Institute for Quality and Efficiency in Healthcare (IQWiG), Germany



- First completed JCA, with the JCA report published on **9 June 2026**
- Assessors considered the differences between the submitted indication and the **final approved therapeutic indication**:
 - The JCA Subgroup concluded that no revision of the assessment scope was required.
 - Changes to the final label were not considered to have a meaningful impact on the available clinical evidence.
 - Interpretation of efficacy and safety outcomes remained unchanged despite the label update.



Overview of two recent JCA procedures: All PICOs were addressed through direct and/or indirect evidence



Lurbinectedin (Zepzelca®)

- Orphan Drug
- **HTD:** Pharma Mar
- **Indication:** maintenance treatment of adult patients with ES-SCLC whose disease has not progressed after first-line induction therapy with atezolizumab, carboplatin and etoposide
- **No. of PICOs:** 1
- **Assessor:** Institute for Quality and Efficiency in Health Care, Germany
- **Co-Assessor:** National Authority of Medicines and Health Products, Portugal



Tarlatamab (Imdylltra®)

- Orphan Drug
- **HTD:** Amgen
- **Indication:** treatment of adult patients with ES-SCLC, who require systemic therapy following disease progression on or after first-line treatment with platinum-based chemotherapy
- **No. of PICOs:** 7
- **Assessor:** Institute for Quality and Efficiency in Health Care, Germany
- **Co-Assessor:** National Centre for Public Health and Pharmacy, Hungary



- Both JCA reports were published 40 days after EC Decision, compared with 50 days for Tovorafenib.
- An RCT served as the basis for both assessments, allowing all PICOs to be addressed, through direct evidence for Lurbinectedin and RCT subpopulations plus indirect evidence for Tarlatamab.

JCA dossiers become highly extensive when multiple PICOs are supported by evidence

	 Tovorafenib	 Lurbinectedin	 Tarlatamab
 PICOs	8 PICOs , 3 subpopulations	1 PICO	7 PICOs , 4 subpopulations
 PICOs addressed	 2 of 8 (25%)	 1 of 1 (100%)	 7 of 7 (100%)
 JCA dossier	 60 documents 684 pages	 23 documents 1206 pages	 78 documents 6118 pages
 Extent of redactions	 Limited RWE data feasibility report is redacted	 No redactions	 No redactions



Limited stakeholder involvement and unclear impact of input

	 Tovorafenib	 Lurbinectedin	 Tarlatamab
 No. carer & experts	1 carer 1 clinical expert	1 carer 1 clinical expert	2 carer 2 clinical experts
 Procedural step	• Assess. scope proposal • Draft JCA report preparation • Revised draft JCA report	• Assess. scope proposal • Revised draft JCA report	• Assess. scope proposal • Revised draft JCA report



- **Limited stakeholder involvement** and heterogeneous level of detail: Only one to two clinical expert(s) and caregiver(s) contributed throughout the JCA process
- **Lack of transparency:** The impact of stakeholder input on the final assessment scope and JCA reports remains unclear. While clinical expert input may have influenced a scope change for tarlatamab, stakeholder feedback in other JCAs appears to have gone unaddressed.
- **Need for guidance and support:** Carers highlighted a lack of information and uncertainty on how to review the JCA report and practical meaning. carers expressed concerns that their involvement may remain “symbolic” rather than enabling meaningful participation.

Methodological scrutiny, not product judgment, drives the tone of JCA reports

Important clarification

- Purpose of JCA report: structured, **objective assessment** of **clinical evidence**
- Focus: **methodology & comparability** of evidence

As a result:

- Strong focus on **methodological scrutiny** of evidence
- Extensive discussion of **limitations** and **uncertainties** (particularly pronounced in unanchored MAICs / ITCs - multiple pages on assumptions, bias, and validity constraints)
- **Evidence gaps** are highlighted in detail

Observations



Implications

- Underrepresentation of the **value story**, the unmet medical need, the clinical “benefit perception”
- Report can be **misread** as weak performance – even when it is a consequence of methodological restraints, not clinical inferiority
- Risk: Readers might conclude „product cannot demonstrate value“ if **context** is missing





So far there are a number of critical developments which should be followed closely

1

- Tovorafenib
- COMP: No product approved in indication; vincristine is SoC in FL
- CG: 8 PICO's with 7 comparators or comparator-elements

2

- Tovorafenib
- HTD: presentation of unanchored MAIC, based on study abstract
- CG: results not included, MAIC not evaluated in JCA-R

3

- Tovorafenib
- Rates for PFS at 6 and 12 months were requested in the assessment scope; HTD did not provide.
- CG calculated these values and included them in the JCA-R

4

- Lurbinectidin
- EMA: Product is approved based on 1st data cut
- CG: JCA-R reports only 2nd data cut

5

- Lurbinectidin
- EMA (CHMP, COMP): Significant benefit based on OS
- CG: No significant advantage in OS



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Our learnings from early JCA submissions (I/II)

1.

Patient and clinical expert input remains underutilised

Current engagement mechanisms do not consistently capture patient perspectives, unmet need, or clinical practice realities.



2.

JCA increases evidence generation requirements

The level of detail expected for evidence submissions, particularly for SLRs and evidence justification, exceeds traditional European HTA requirements and requires substantial additional resources from HTDs.



3.

Assessment begins during completeness check

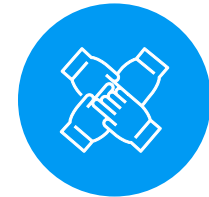
Completeness checks assess not only dossier completeness but also methodological choices, search strategies, and the feasibility of the proposed evidence approach.



4.

Operational readiness is critical

Short response timelines for requests for information require dedicated cross-functional teams, rapid decision-making processes, and readily available expert resources throughout the assessment.



Our learnings from early JCA submissions (II/II)

5.

JCA reports already shape evidence interpretation

Assessment reports do not merely summarise submitted evidence but also influence which analyses are considered relevant and discussed within the assessment.



6.

The fact-check process offers no opportunity for contextualisation

HTDs have only restricted opportunities to provide context and interpretation of assessment findings, highlighting the importance of a transparent and structured response mechanism.



7.

The JCA process continues to evolve

Recent procedural refinements have improved interaction between assessors and HTDs. However, variability in implementation and the need for clearer communication channels suggest further process optimisation is still required



PICO scoping: first insights of ongoing JCA projects (n=3)

PICO Scoping Process and Predictability

- PICO scoping completed within **<80 days**
- **<5, <10, or <15 PICOs** per assessment
- **High predictability** for population and comparator, but declining with increasing number of PICOs

Comparator

- Number of PICOs largely driven by number of relevant comparators
- The following pattern can be observed: all potential comparators are listed as individual PICOs, likely due to an AND linkage, and then the same comparators are repeated as a separate PICO connected via an OR linkage
- Unexpected off-label comparators included (not guideline-recommended) [n=1]

Subgroups

- Number and relevance vary widely across assessments; methodology appears **inconsistent**
- Common national HTA subgroup standards (e.g., age, sex for G-BA) are not systematically included
- Cut-off values sometimes differ from clinical trial definitions
- Cut-off values are not defined for all subgroups (inconsistent)
- Requested subgroup analyses can differ between the defined populations within the same assessment
- Certain country-specific requirements can still be identified (e.g., Germany-specific subgroup or comparator structures)

Population

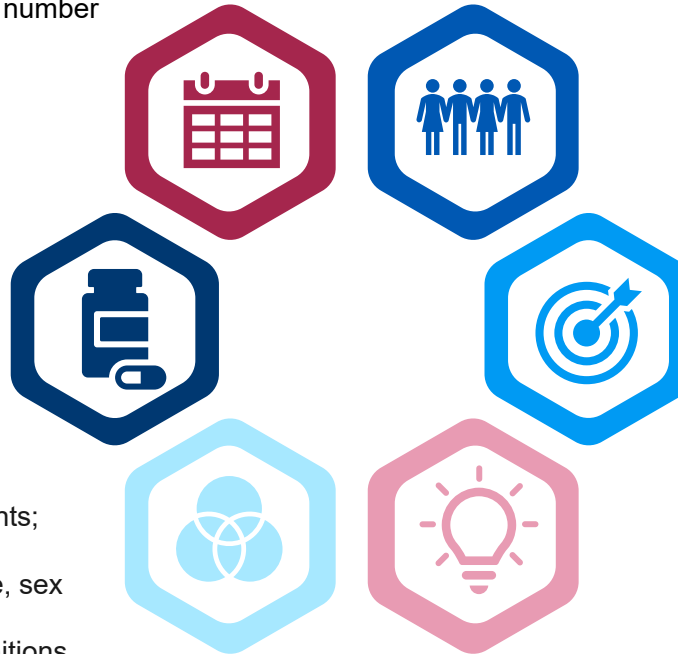
- 1-4 populations per assessment
- Subpopulation mainly defined by **prior therapies**

Outcomes

- Some outcomes are specifically defined (e.g., generic HRQoL “preferably via SF-36”), others remain very broadly defined (e.g., “symptoms of disease”)
- Outcomes sometimes requested beyond those assessed in clinical trials
- AEs not requested in detail
- No specific analytical requirements (e.g., TTE analysis, MID definitions)
- Outcome list is focused and limited (QoL, symptoms, key indication-specific outcomes)
- Outcome list is specified across all PICOs

Overall

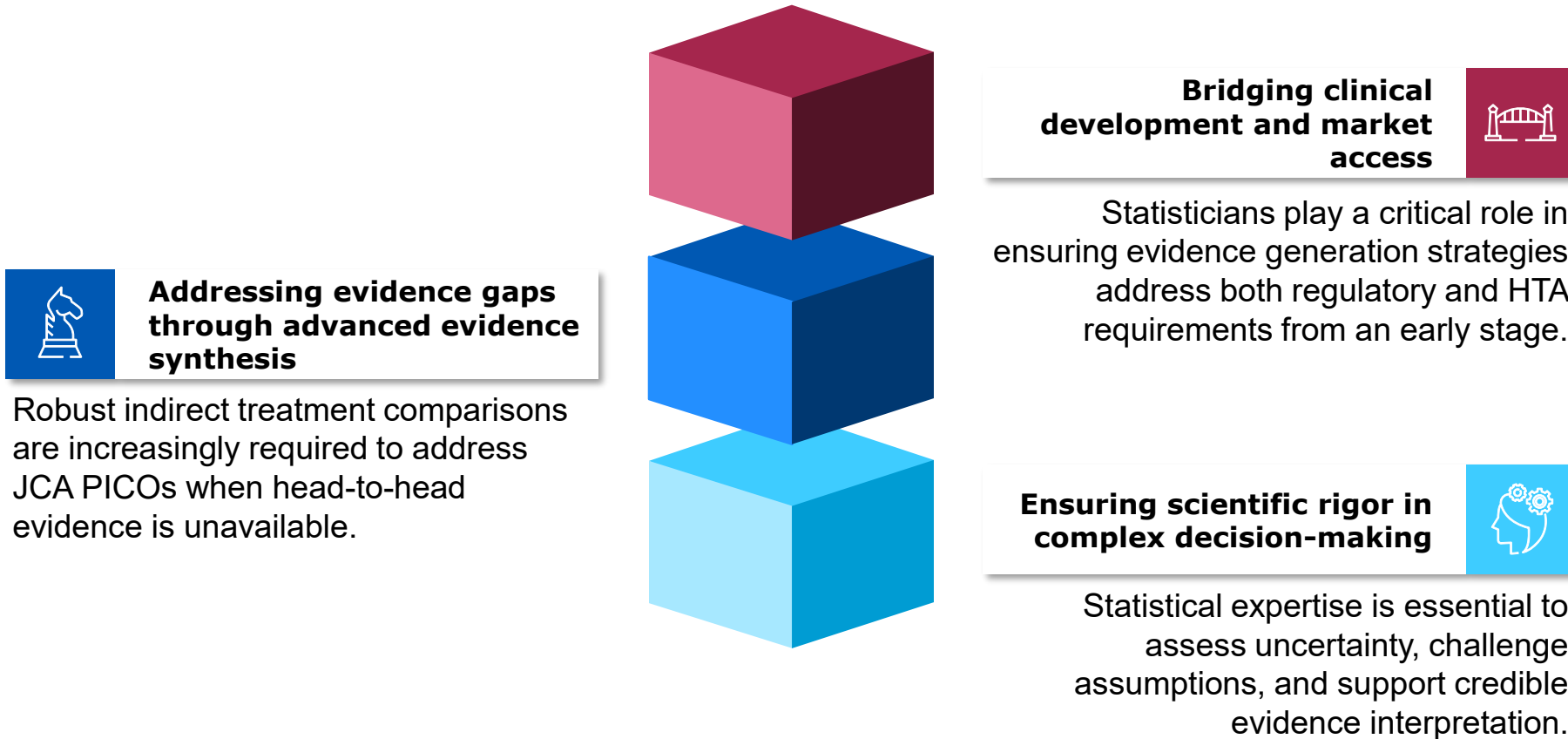
- Predictability becomes particularly challenging when multiple comparators are involved, especially given the limited transparency on how the CG approaches comparator selection.
- Country-specific requirements allow for the identification of the German PICOs





Statistical leadership is becoming a critical success factor for EU HTA

Statistical expertise is becoming essential to align clinical development, HTA requirements, and patient access.





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Feel free to contact us!



Thomas Ecker

✉ t.ecker.ext@ecker-ecker.de
☎ +49 177 6452463



Janine Leismann

✉ j.leismann@ecker-ecker.de
☎ +49 (40) 41 330 81-26