Health Technology Assessment in cancer drug development

SESSION 2: IMPLEMENTATION OF HTA REGULATION: PRACTICAL LEARNINGS

Collaborative Industry Perspective

Overview

Timelines	JCA Process Experiences	MAA Process Experience	Transparency	National Implementation
• JCA and MAA timelines • Key milestone alignment	• Scoping phase • Dossier submission • Review process	• Regulatory Strategy • Internal HTA integration • Cross-functional collaboration	• Disclosure alignment • Report usability	• Early use of endorsed reports • National decision context

JCA timelines: variable with a mixture of rigidity and pragmatism

EMA-LED CLOCK

1 **EMA validation**

Observed procedure start can follow validation, with early-case variation.

2 **Final PICO scope**

Claimed indication defines scope; EMA label changes trigger updates.

3 **Dossier submission**

Industry preparation must begin many months before EMA filing.

4 **CHMP opinion**

Draft report after CHMP

Assessor requests can come at any time on numerous occasions

5 **MAA**

6 **JCA publication**

JCA publication timing variation may depend on redaction and document release discussions with EMA.

Scope receipt	Scope to submission	Confirmation check	Draft report to HTD	Endorsed report	Published report
Avg. 84 71–109 days	Avg. 100 99–101 days	Avg. 63 56–69 days	Avg. 82 72–99 days	Avg. 54 33–72 days	Avg. 31 16-53 days

EMA VALIDATION TO JCA PUBLICATION **413 days (356 – 473 days)**

FIXED

Official response days

Timelines written into the Regulation and Implementing Acts cannot move—even when the request is complex.

POTENTIALLY FLEXIBLE

Guidance touchpoints

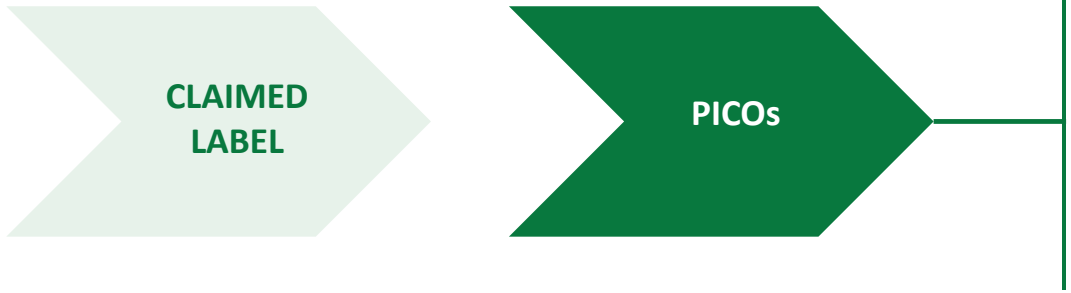
Pragmatism is possible where the HTD presents a credible rationale and a clear win–win outcome.

CONCLUSION **The JCA has to fit into the EMA pathway; we are seeing increasing consistency in starts, but variability in publication**

*All figures refer to the first 4 JCA reports

Scoping is iterative—and determines the size and workload

SCOPE LOGIC



SUBPOPULATION

Can expand dossier volume

SUBGROUP

Can expand analysis burden

EXPLANATION MEETING

Current experience: more procedural than explanatory.

- Why these PICOs?
- Which methods are acceptable?
- Can key questions be answered early?

WORKSHOP RECORD

Observed

The scope evolves when the EMA label changes during the procedure.

Unresolved

More clarity/guidance in defining subpopulations and what would be treated as a subgroup, as well as defining individualised treatment comparisons.

Improvement suggested

Enable meaningful dialogue, rationale for why the PICO has been requested, and early methodological guidance before completeness checks.

CONCLUSION

Increased engagement and dialogue would deliver a better understanding of the scope and improve the quality of the JCA dossier and increase efficiency in the procedure – potential to build on EMA experience

IT platform has improved; repeat uploads remain the pain point

OPERATING EXPERIENCE

INITIAL

Upload mechanics were difficult

Teams faced technical issues simply uploading the submission.

CURRENT

The process is materially smoother

Issues appear to have been heard; pragmatic solutions have been possible.

REMAINING GAP

Every update requires the full dossier

Even one changed document triggers re-upload of the complete folder structure.

PREFERRED WORKFLOW

Changed file
One document updated

→

Incremental upload
Only new or changed files

→

Version register

Business continuity

Ensure you have a primary point of contact plus at least one designated backup.

Pragmatism

Continue using practical solutions where timing and submission choices create a win–win.

Efficiency + environment

Incremental uploads reduce handling, rework and unnecessary data transfer.

Uncertainty on request timing creates challenges

The challenge

Questions sometimes arrived earlier—or much later—than teams expected/communicated. Staff were on leave, and internal capacity had already been committed elsewhere.

Teams working on the JCA are wanting to do their best

When the requested method is implicit, the HTD may take a reasonable approach only to find the assessor preference differs but only recorded later in the report as a criticism.

RESPONSE WINDOW IS TYPICALLY 7 CALENDAR DAYS (irrespective of complexity)

WHAT GOOD COULD LOOK LIKE

1. **Pre-notify**
   Warn that requests are coming—even if detail is still being finalised.
2. **Clarify**
   Use a short call where wording, method or expected output is unclear.
3. **Mobilise**
   Reserve evidence, statistics, writing and regulatory capacity.
4. **Respond**
   Deliver within the window using the agreed interpretation.
5. **Confirm**
   Close methodological questions before the draft report.

CONCLUSION

LET'S CREATE A WIN-WIN: Earlier signals and real-time clarification improve quality without changing statutory reply periods.

JCA delivery depends on coordinated expertise, capacity and teamwork

CORE CONTRIBUTORS

Health Economics Outcomes Research / Market Access

Own the evidence strategy, PICO prediction, interpretation, submission, key contact and assessor response.

Regulatory

Connect EMA decisions to JCA implications and market-access consequences.

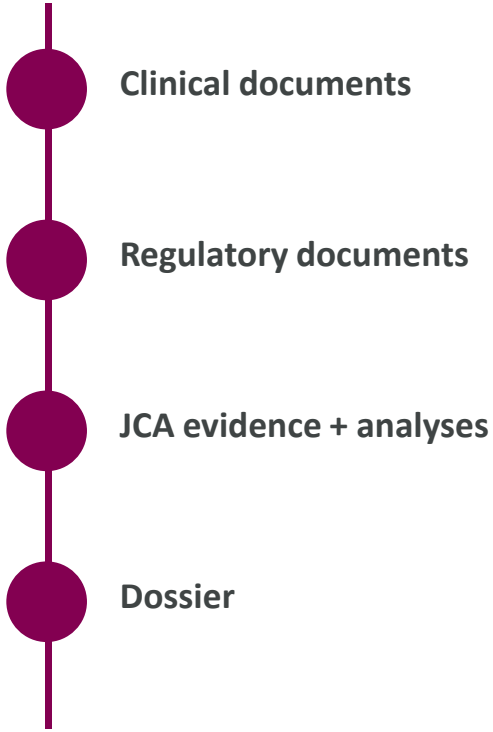
HTA / Statistics + programming

Analyses under short and sometimes unexpected turnaround.

Writing + vendors

Drafting, reviews and dossier production.

ONE CURRENT DOCUMENT SET



CAPACITY TENSIONS

Competing procedures

Internal experts may simultaneously support EMA, FDA and other global regulators.

Changing source material

Teams need to know where every document is—and which version is current. E.g. EMA documents and trial readouts

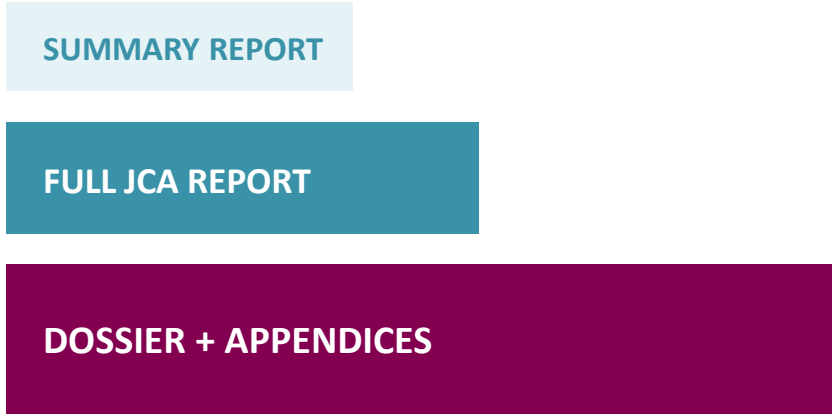
Many hand-offs

Disparate contributors must understand how each process affects the other.

CRITICAL **Teamwork and document control are critical-path activities, not supporting tasks.**

Publication volume is high; decision usefulness to be observed

WHAT IS PUBLISHED



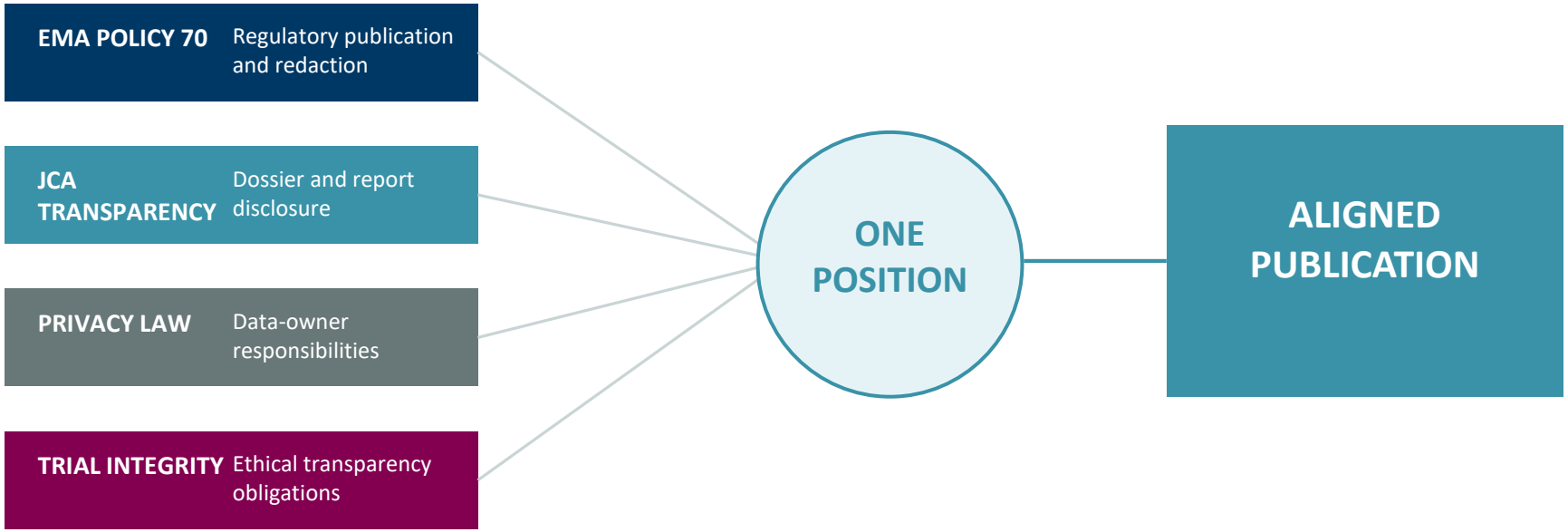
The published documents can reach hundreds—or thousands—of pages

REPORT PROFILE

Highly technical	Large and statistically dense
Limited context	Little explanation of the evidence base and disease challenges
Uncertainty-heavy	Strong emphasis on bias and uncertainty versus evidence strengths
Summary limitations	Not written as a complete source for patients, clinicians or decision makers

Disclosure and redaction needs to be coordinated and consistent

DISCLOSURE OBLIGATIONS



Observed

Disclosure discussions are iterative, not one-and-done.

Positive process

Secretariat alignment with EMA avoids duplicate or contradictory decisions.

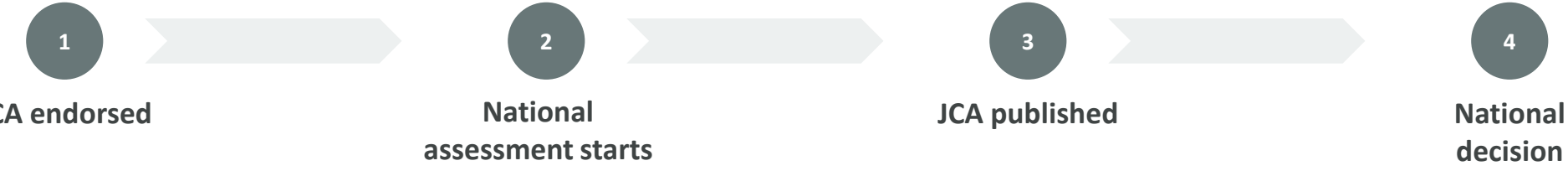
Principle

Balance transparency with privacy and trial integrity.

Non-duplication matters: Risk if two European bodies reach different disclosure positions on the same evidence.

CONCLUSION **Maintain a single EMA-aligned disclosure approach while recognising that redaction requires dialogue.**

Endorsed JCA reports can inform assessment before publication



GERMANY

NOT USED AT OPENING STAGE

Assessment started before publication; the endorsed report was not used in the initial procedure.

IRELAND

ENDORSED REPORT INFORMED INITIAL NCPE VIEW

NCPE used the endorsed report before formal publication, although it could not yet attach it publicly.

Industry view: availability—not publication timing—should determine when national bodies can use the report

AVAILABLE TO MEMBER STATES ≠ PUBLISHED

CONCLUSION **Delaying national use of a JCA report that has already been endorsed and shared until published can compromise an objective of the Regulation to improve patient access.**

CONCLUSIONS – Industry Perspective

- As expected, the JCA has fitted into the EMA pathway
- Increased engagement and dialogue would deliver a better understanding of the scope and improve the quality of the JCA dossier and efficiency in the procedure → potential to build on EMA experience
- Continue using practical solutions where timing and submission choices create a win–win.
- Earlier signals and real-time clarification improve quality without changing statutory reply periods.
- JCA delivery success depends on coordinated expertise across all relevant functions, capacity and teamwork.
- Maintain a single EMA-aligned disclosure approach while recognising that redaction requires dialogue.
- Delaying national use of a JCA report that has already been endorsed and shared until published can compromise an objective of the Regulation to improve patient access.