



Market Access and Pricing for Pharmaceutical Products and Medical Devices

Dr. Vivien Barz
Janik Beuermann

24/03/2026



Strategic role of statisticians for JCA



About the HTA ESIG



EUROPEAN FEDERATION OF STATISTICIANS IN THE PHARMACEUTICAL INDUSTRY
Representing Statistical Associations in Europe

We bring together statisticians working in the pharmaceutical industry with a shared interest in advancing methods and practice in Health Technology Assessment (HTA)

More specifically, the HTA ESIG focuses on the following:

1. Empower HTA statisticians, through a platform for sharing and co-developing best practices
2. Foster interdisciplinary and intradisciplinary scientific dialogue on HTA statistical matters
3. Utilize our collective HTA statistical expertise to provide influential input to policy-making
4. **Promote education and awareness about the importance and applications of HTA statistics**



Want to get more involved? Become a member of the HTA ESIG today - scan the QR code or email htasig@psiweb.org



About us



Dr. Vivien Barz



Janik Beuermann



Ecker + Ecker GmbH



HTA perspective

- Academic background: PhD in the field of Clinical Pharmacy
- Employed with E+E since January 2022, currently as Manager HTA.
- Hands on experience in EU HTA projects, including end-to-end JCA strategy design and implementation, currently supporting one ongoing JCA procedure
- Extensive expertise in German benefit assessment, pricing, and market access
 - Comprehensive understanding of early advice and clinical trial strategy in various indications



Statistician's perspective

- Academic background: Master in business mathematics
- Employed with E+E since 2020, currently as Operation & Expertise Lead Biostatistics
- Hands on experience from supporting three ongoing JCA procedures
- Comprehensive knowledge of methodological requirements in EU HTA and German benefit assessment.
- Extensive experience in statistical programming, with a focus on post-hoc analyses for health technology assessment (HTA) and evidence synthesis.

Consultancy for Market Access and Pricing & Reimbursement

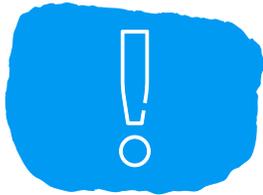
- Founded 2011 in Hamburg
- Consulting services in the fields of German and international Market Access and Pricing & Reimbursement
- Interdisciplinary consulting team in the field of life science, pharmaceuticals, health economy, and biostatistics
- European Partner Network covering 30 European countries

Housekeeping: Acknowledgements and Disclaimer



Acknowledgements

- EFSPI and the HTA ESIG Training Subteam
- PSI for facilitating the webinar
- Ash Bullement for the moderation



Disclaimer

- The view presented are the views of the presenters, not necessary those of the affiliated organizations.
- The content of this slide deck is based on our experience with current JCA projects, experiences might differ depending on the size, structure and organization of the different stakeholders.



Agenda

- 1 JCA introduction
- 2 Strategic role of statisticians for JCA
- 3 Q & A



Agenda

1 JCA introduction

2 Strategic role of statisticians for JCA

3 Q & A



Overview of the EU HTA procedure: **Joint Scientific Consultation (JSC)** and **Joint Clinical Assessments (JCA)** as the two main components



Assessment Scope

- Population(s)
- Intervention
- Comparator(s)
- Outcome(s)

CAVEAT:

- Multiple PICO(s) are possible as scope must be inclusive of Member States' needs
- PICO(s) remain anonymous



Joint Clinical Assessment (JCA)

- Based on the submitted dossier
- Requests for additional data are possible throughout the entire assessment period
- Assessors from Member States (HTA bodies)

Important:

- JCA report will not include a value judgement!
- Neutral description of the presented evidence
- JCA report serves as a basis for national appraisals



JSC

Start of Procedure

PICO(s) to HTD

Dossier Submission

Endorsement of JCA report



National Appraisals

Adoption and implementation of EU HTA in national procedures not yet complete



JCA Dossier

- The JCA dossier must include direct or indirect evidence addressing each PICO.
- If a PICO cannot be addressed with evidence, a justification is required.
- Timelines are not adjustable, irrespective of requested scope.



Joint Scientific Consultation (JSC)

- Early scientific advice!
- No pre-submission meeting!



Decisions on the extent of the added benefit remain within the competence of the Member States

Joint Clinical Assessment on European level

ASSESSMENT

- Health problem and current use of technology
- Description of technical characteristics
- Safety
- Clinical effectiveness

- **No value statements**
- **Neutral basis for national appraisal**

National appraisal at country level (EU27)

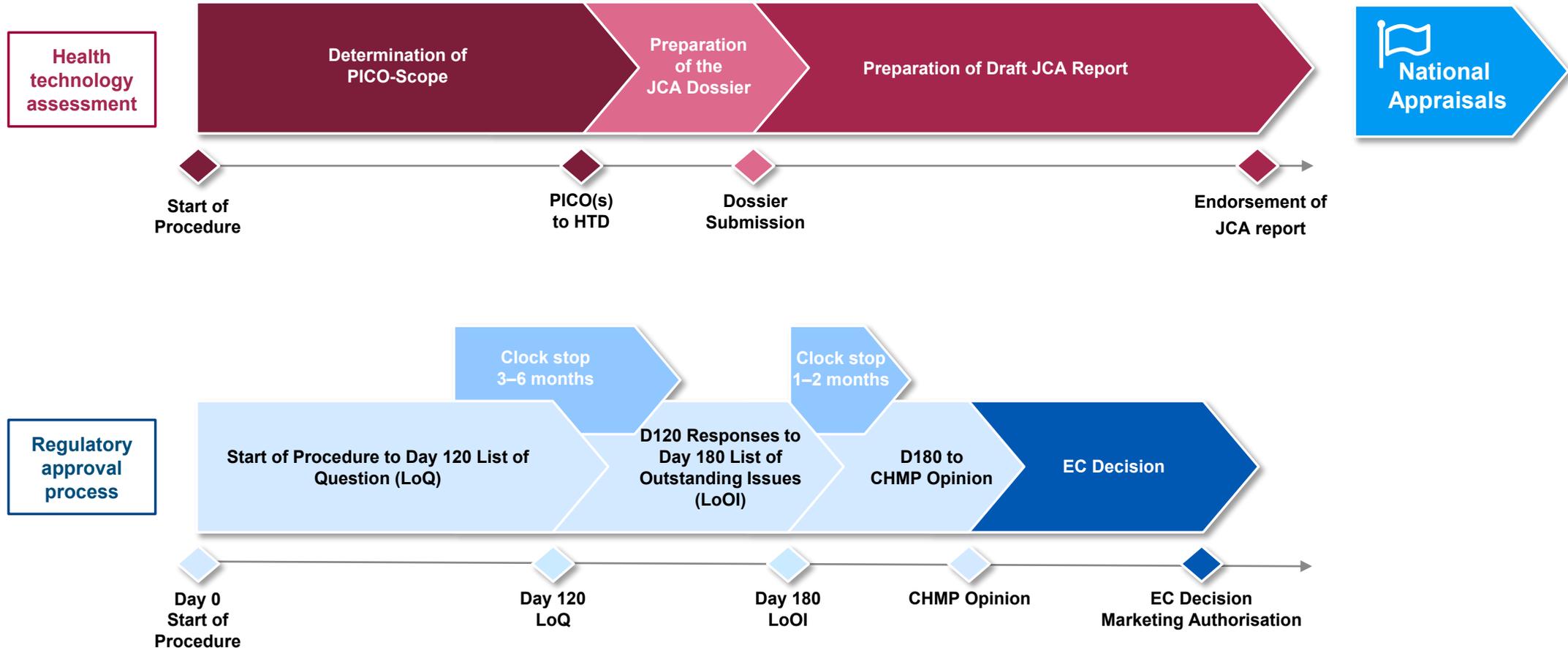
APPRAISAL

- Cost and economic effectiveness
- Ethical considerations
- Organisational aspects
- Patient and social aspects
- Legal aspects

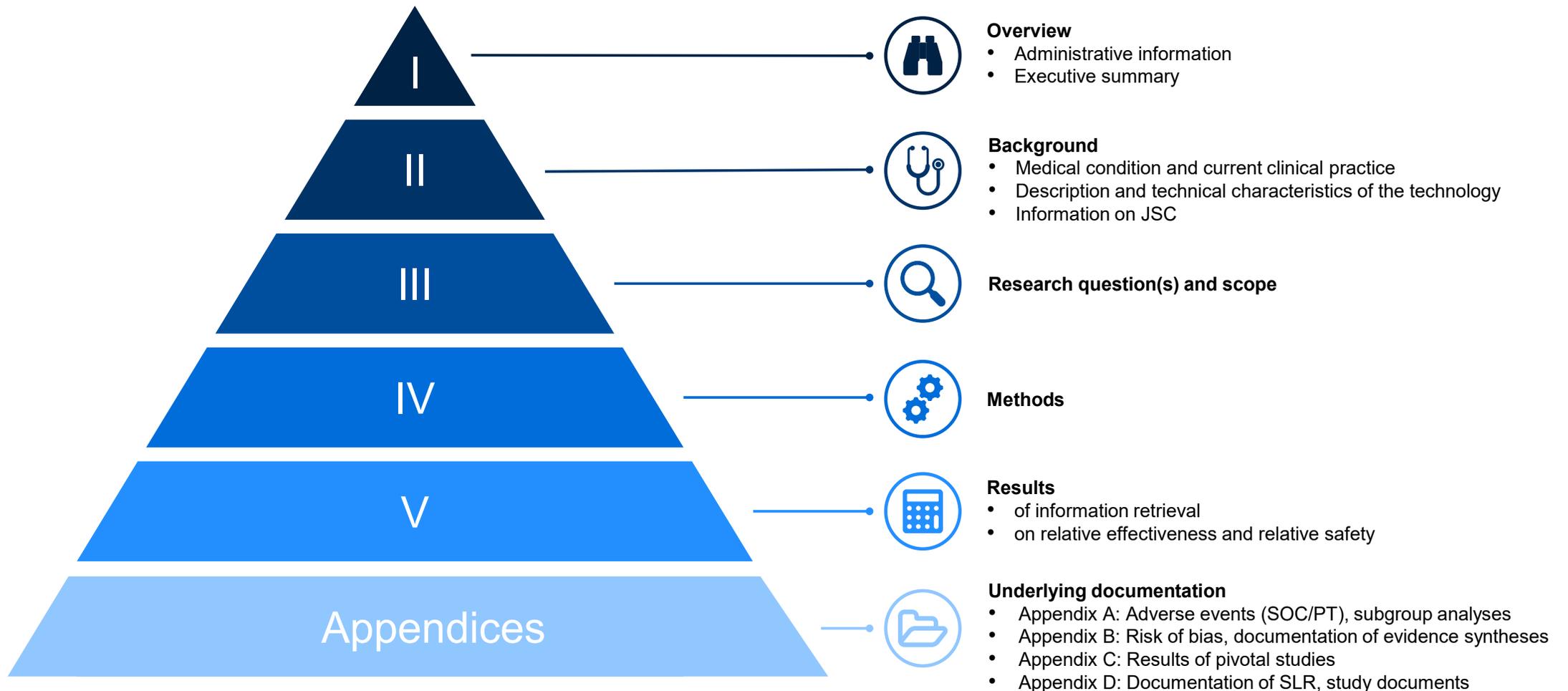
- **Determination of additional benefit**
- **Basis for P&R decisions (remains sole responsibility of MS)**



The EU HTA process is dependent on, and runs in parallel with the EMA marketing application process



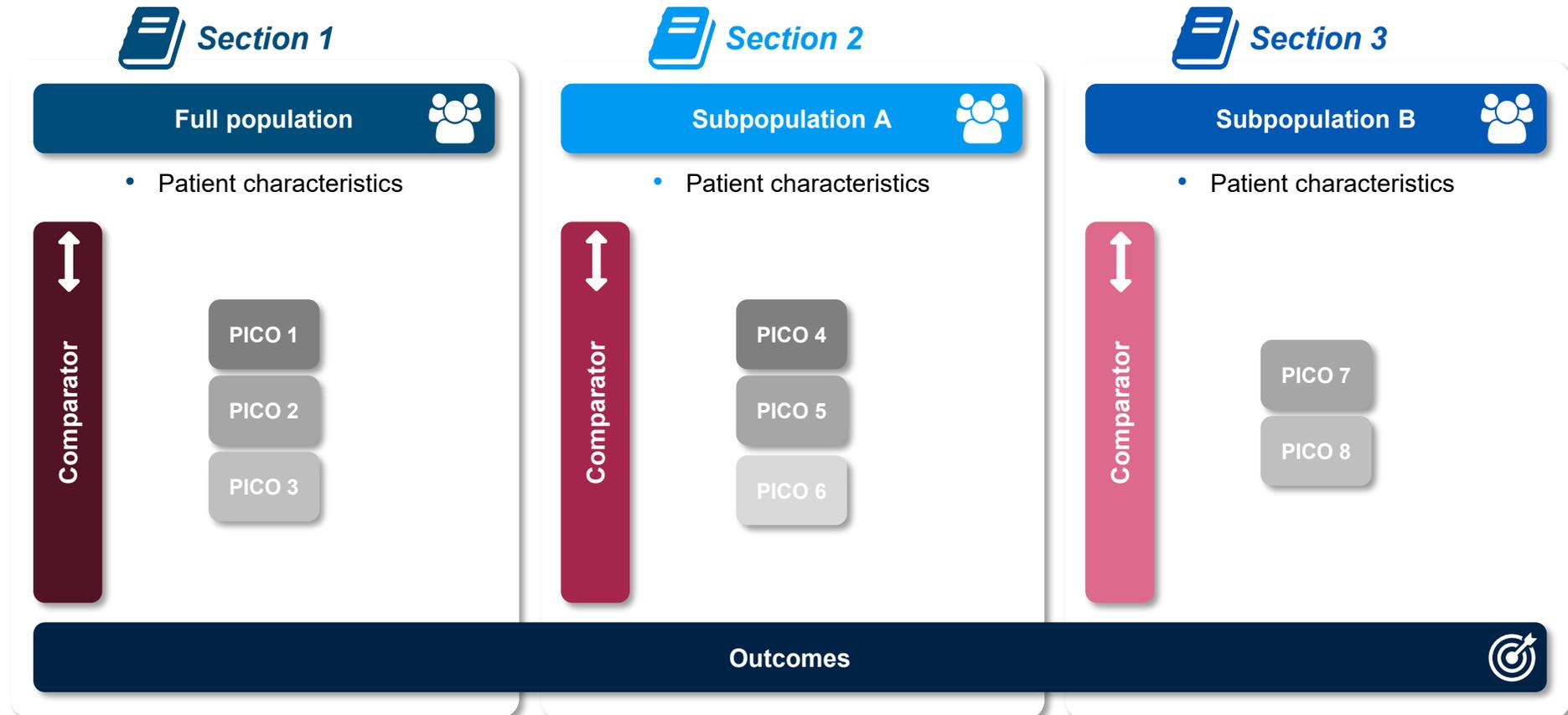
High-level structure of the JCA dossier



≈ The SLR for EU HTA can be extensive – if direct comparative evidence is not available, evidence for ITC must be searched for

| | Available studies | Methodological option | |
|-----------|---|---|---------------------|
| 1. | RCTs comparing the new medicinal product with the comparator from a given PICO | Direct comparison (single study or meta-analysis) | Certainty of effect |
| 2. | Different RCTs with either the new medicinal product <u>or</u> the comparator from a PICO | Anchored ITC | |
| 3. | Single arm trial with new medicinal product (or non-randomised comparison of the new MP with the comparator from a given PICO) | Unanchored ITC vs external/non-randomised control | |

Chapter V is divided into individual sections according to the patient populations



If no evidence is provided for a PICO question, this must be justified

Anne Willemsen: [the JCA subgroup will] “not accept that there is simply no data on something.”

“If the drug producer cannot provide scientific data necessary for the HTA, it needs to explain, why it is not possible”.



5



5 Results

5.1 Results from the information retrieval process

5.1.7 List of studies included overall and by PICO question

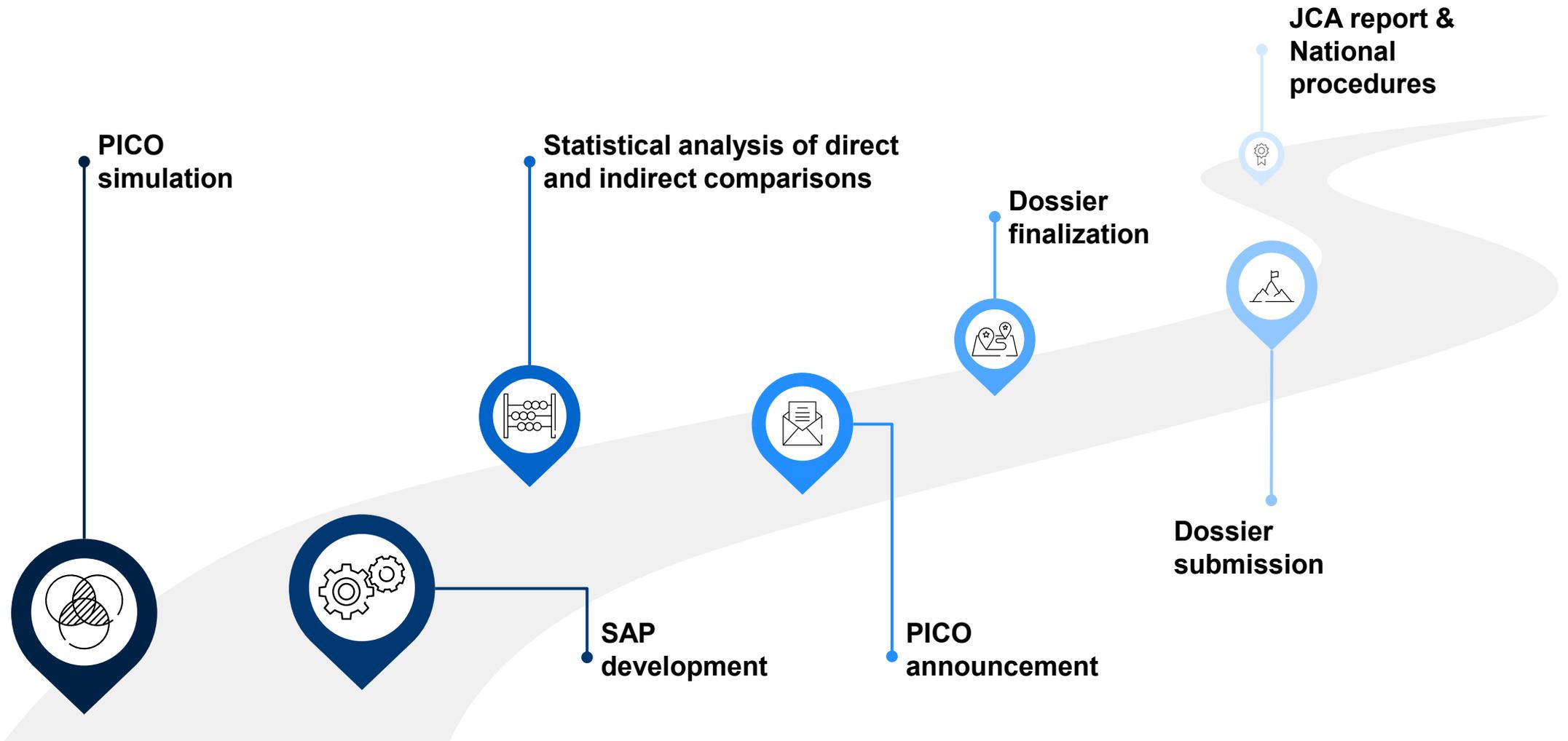
- [...] The tables should include all PICO questions from the assessment scope. If no evidence is provided for a specific PICO question in the assessment scope, this should be recorded under the relevant PICO heading (“No evidence provided by the HTD”) and **justified**.

Conclusion

For PICOs that do not have supporting evidence, an appropriate **justification** needs to be provided! The rationale should be **consistent** with the respective **national strategy**.



Navigating the JCA: Key project milestones





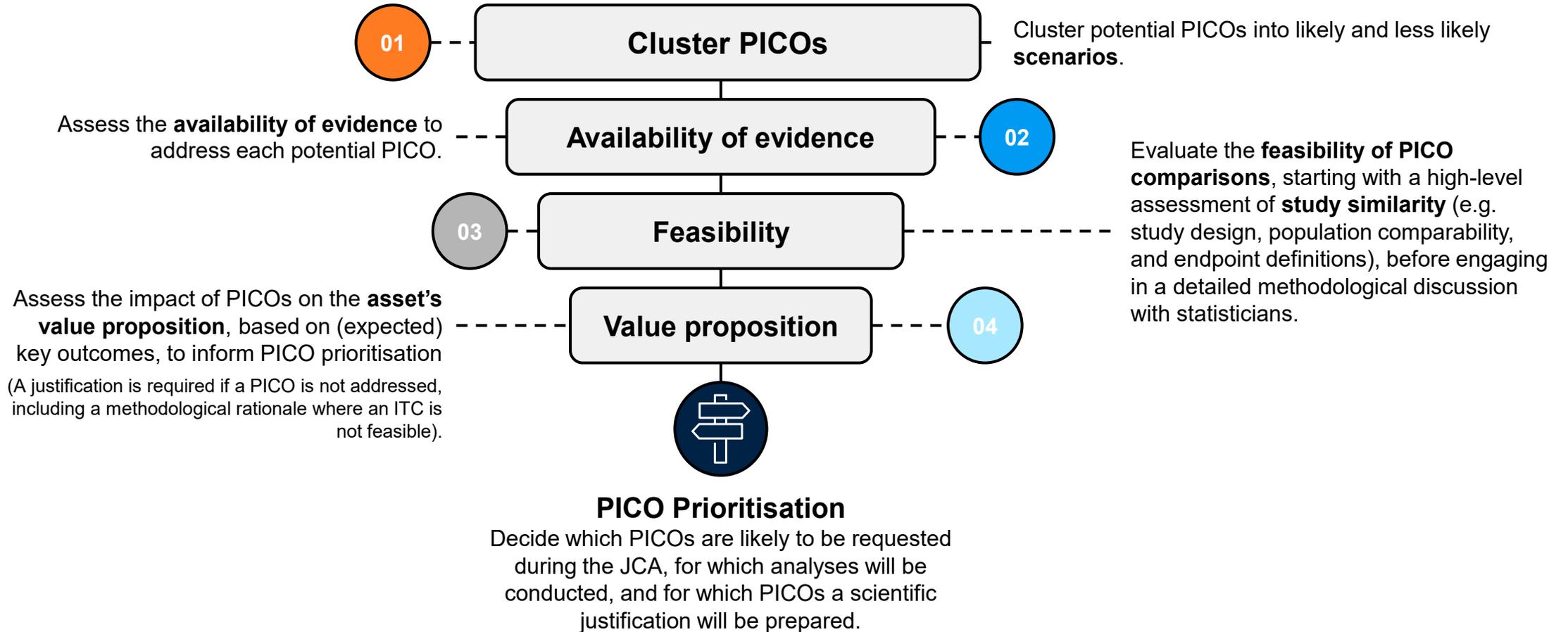
Agenda

1 JCA introduction

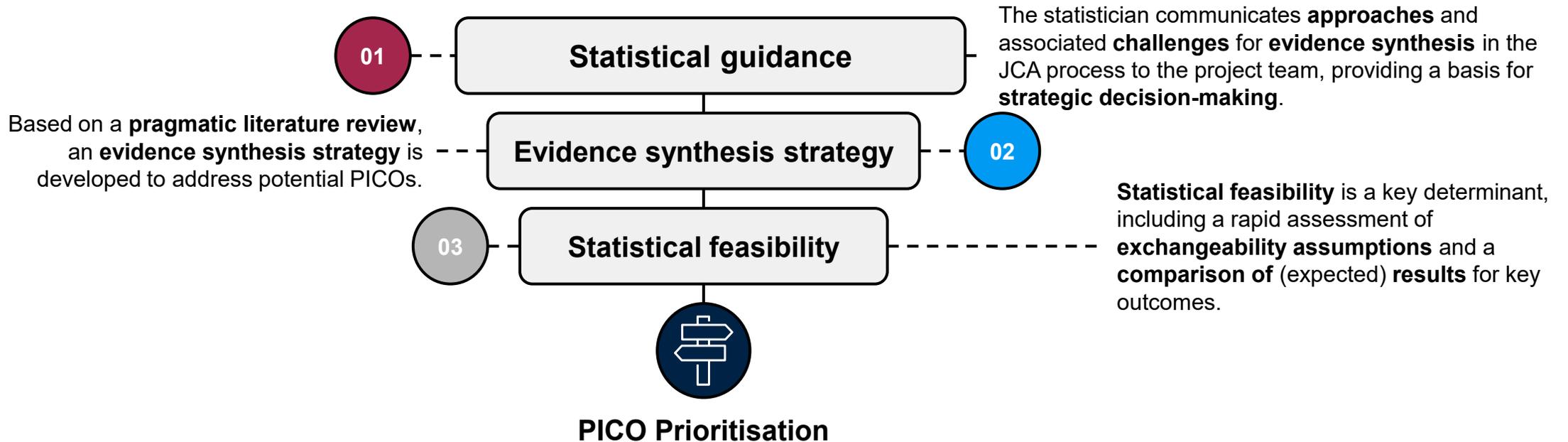
2 Strategic role of statisticians for JCA

3 Q & A

How to anticipate and prioritise PICOs for the JCA



How to anticipate and prioritise PICOs for the JCA



EU HTA SAP: Integrating HTA requirements and statistical strategy

HTA perspective

- Since the **EU HTA SAP** must be submitted in the JCA appendix, the dossier lead discusses the SAP early with the statistician, based on **anticipated PICO**s and decisions on which PICOs will be analyzed in the dossier.
- Confirm that all **JCA-relevant endpoints** and **comparisons** are included in the EU HTA SAP.



Statistician's perspective

- The statistician leads the project team discussion on the **strategic direction** of the **EU HTA SAP**, focusing on three **key questions**:
 - Where?
 - When?
 - What?
- Leveraging an in-depth understanding of the study SAP and EU HTA statistical guidelines, the statistician identifies **methodological gaps** that need to be addressed within the **EU HTA SAP**.
- Targeted **sensitivity analyses** are applied to strengthen the **robustness** and **credibility** of the results.
- The statistician **authors the EU HTA SAP** and coordinates the **review process** in close collaboration with key project stakeholders.

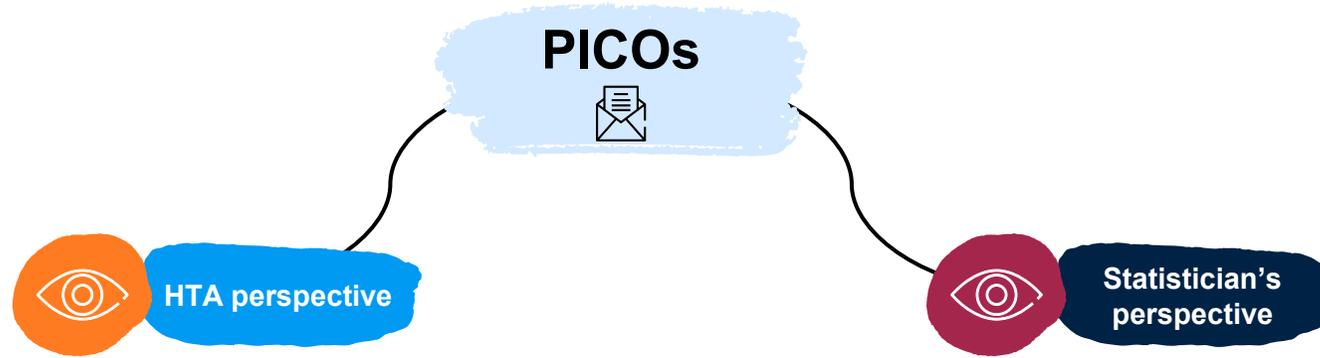
Ensuring methodological robustness of direct & indirect comparisons for EU HTA

- 
1
 - Evaluate findings from **effect modification** and **exchangeability assessments** to justify the inclusion or exclusion of indirect comparisons, and clearly explain **assumptions and limitations**.
- 
2
 - Incorporate direct and indirect comparison results into the relevant sections, **interpreting outcomes** in the context of the **asset's value story** and highlighting potential uncertainties or biases.
- 
3
 - Based on comparison results, discuss with the statistician whether **sensitivity or subgroup analyses** are required, helpful, and feasible.
- 
4
 - Present **detailed methodology** for all analyses, maintaining close communication with the statistician to **ensure alignment**.
- 
5
 - Identify analyses, particularly indirect comparisons or post-hoc evaluations, that may be questioned, and prepare **justifications** and **methodological rationale** in advance.

Ensuring methodological robustness of direct & indirect comparisons for EU HTA

- 
1
 - Research for **effect modifications/prognostic factors** for assessing the **similarity assumption** in evidence synthesis before starting the analyses.
- 
2
 - Coordination of programming or own programming for necessary **post-hoc analyses of pivotal studies**.
- 
3
 - **Extraction of the relevant data of comparators** that should be addressed via indirect comparisons.
- 
4
 - Coordination of programming or own programming for necessary **evidence synthesis**.
- 
5
 - Detailed evaluation of **exchangeability assumption** for evidence synthesis.
- 
6
 - Ensuring the **accuracy** of calculations.

PICO announcement: Strategic and operational considerations



- Identify any **implications** from the PICO announcement:
 - Check for any **unanticipated PICOs** and evaluate available evidence to address them.
 - For **ITCs**, assess **study suitability** and whether results are favorable or not
 - decide if analysis should be performed or justification for not performing an ITC should be provided.
 - Discuss **feasibility** of **additional outcomes** or analyses with the statistician and decide how to address them in the dossier.
- **Adapt the analyses** from the previous step so that they meet the requirements of the final PICOs.
- For very unexpected requests, it may be necessary to **dive deeply into the data** to find necessary information.
- Have some **time blocked** around the date of PICO announcement – if something unexpected arises, your colleagues will have to update the dossier within **100 days** and will appreciate receiving the **updated results as soon as possible**.

Dossier finalization: Cross-functional review and validation

01

- Incorporate **any new or updated data** into the dossier.

02

- Update all **methodological sections** including justification in case PICOs cannot be addressed in close **collaboration with statistician**.



HTA perspective



Statistician's perspective

01

- The statistician helps establish a workflow for generating **dossier-ready result tables**. It should be ensured that:
 - Result tables include all required information
 - The footnotes accurately describe the applied methods
 - The numbers are correct

02

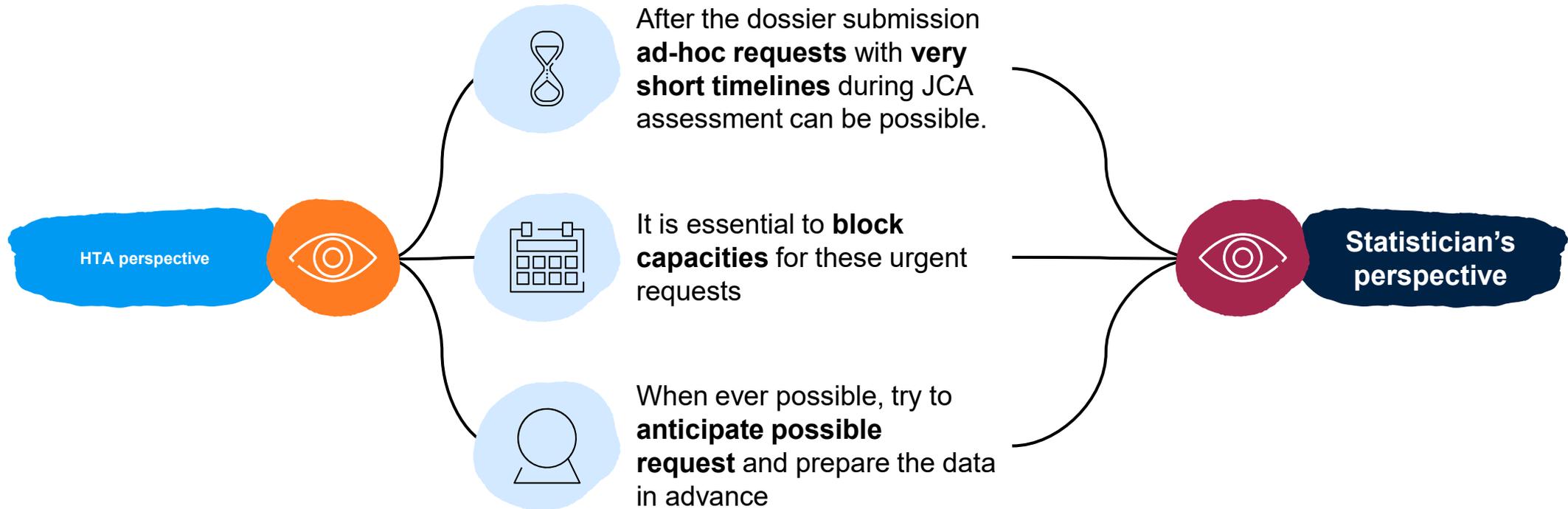
- Check the **completeness of graphs** (e. g. Kaplan-Meier-plots, forest plots,...) and tables

03

- **Reviewing** parts of the dossier (e.g. statistical methods, results interpretation,...) with a methodological perspective



Dossier Submission: Managing ad-hoc requests

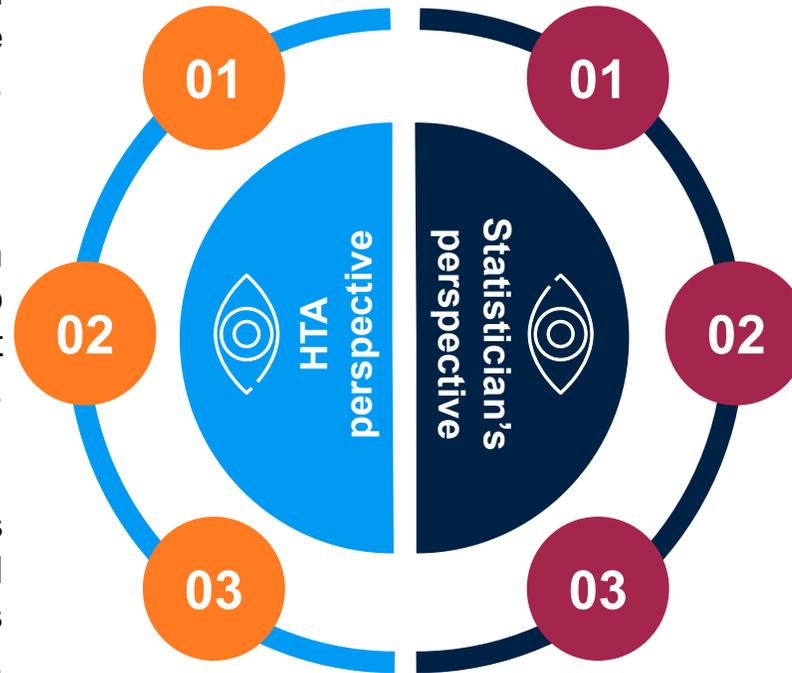


From JCA to national HTA

Maintain **close collaboration** with **national affiliates** to ensure alignment on JCA dossier contents.

For any PICO not addressed in the JCA dossier, be prepared to provide **supporting evidence** at the **national level**.

Recognize that some countries may require **additional analyses** even if the PICO is addressed in the JCA dossier.



Communicate **key statistical aspects** of the JCA report to local affiliates to support anticipation of the impact on the national decision making.

Prepare additional **post-hoc analyses** (e. g. responder analysis for PRO with different MCIDs) for national dossiers.

Be the **point of contact** for detailed statistical questions from national agency when they refer to results from JCA.

Useful links and resources – JCA

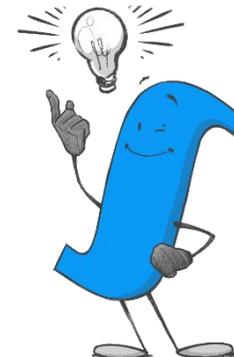
| Legal Framework | |
|----------------------------------|--|
| Regulation (EU) 2021/2282 | <ul style="list-style-type: none">• Regulation (EU) 2021/2282 |
| Implementing act | <ul style="list-style-type: none">• Implementing act: JCA for medicinal products (incl. annex) |

| Guidance Documents by the Coordination Group | |
|--|--|
| Procedural guidance documents | |
| <ul style="list-style-type: none">• Scientific specifications of medicinal products subject to joint clinical assessments• Guidance on the appointment of assessors and co-assessors for JCA and JSC• Procedural guidance for JCA medicinal products• Guidance on the scoping process | |
| Methodological guidance documents | |
| <ul style="list-style-type: none">• Methodological guideline for quantitative evidence synthesis: direct and indirect comparisons• Practical guideline for quantitative evidence synthesis: direct and indirect comparisons• Guidance on outcomes for joint clinical assessments• Guidance on reporting requirements for multiplicity issues and subgroup, sensitivity and post hoc analyses in joint clinical assessments• Guidance on validity of clinical studies | |

| Guidance by the CG: format and templates | |
|---|--|
| JCA | |
| <ul style="list-style-type: none">• Guidance on filling in the JCA dossier template, dossier template, table template collection, technical specifications for dossier submission | |

| Further information | |
|---------------------|--|
| JCA | <ul style="list-style-type: none">• Overview and further information |

Product-specific JCAs and for submission of early information for JCAs (to request access link to the HTA IT platform)  SANTE-HTA-JCA@ec.europa.eu





Agenda

- 1 JCA introduction
- 2 Strategic role of statisticians for JCA

- 3 Q & A

 **Discussion and Q&A**





Market Access and Pricing for Pharmaceutical Products and Medical Devices

Feel free to contact us!



Janik Beuermann

✉ j.beuermann@ecker-ecker.de

☎ +49 (40) 41 330 81-659



Dr. Vivien Barz

✉ v.barz@ecker-ecker.de

☎ +49 (40) 41 330 81-675