What is EU HTA?

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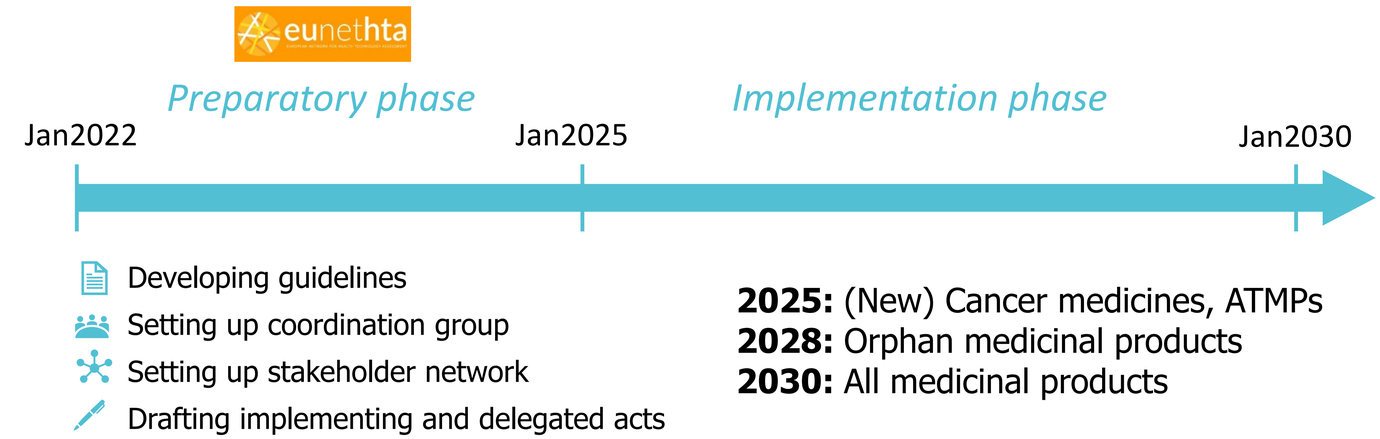
- on behalf of EFSPI HTA European Special Interest Group, sponsored by PSI and EFSPI

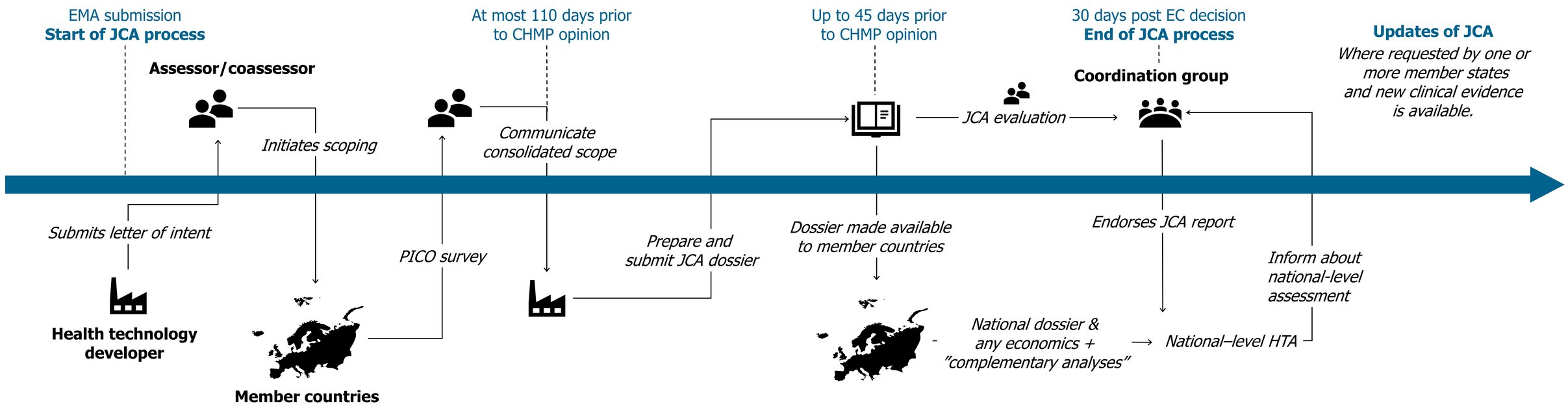
OVERVIEW

- In December 2021, the **EU-Regulation on HTA** (HTA-R) was passed, requiring a **JCA** (Joint Clinical Assessment) at the EU level.
- Among other things, HTA-R mandates **joint EU-level assessment of all new medicines in parallel with the EMA regulatory review**, based on a dossier submitted by the manufacturer
- Having a **joint assessment covering the needs of all EU member states** and tied to EMA timelines, is a completely new paradigm that will have **major implications** for how companies set up and align HTA and regulatory work, and undertake statistical analyses to meet both HTA and regulatory needs.

EU-HTA REGULATION PREPARATION AND IMPLEMENTATION



JOINT CLINICAL ASSESSMENT



Member states individually decide scope based on expected regulatory label
PICO(s): populations, comparators and outcomes

HTA dossier prepared based on expected regulatory label
May only have ~9 weeks to prepare

Evaluation starts before label final (if final label different, short grace period to resubmit)

Pricing/reimbursement decisions at national level using national HTA evaluation (which must give "due consideration" to JCA, and can include additional complementary data/ analyses)

Why should statisticians care about EU HTA?

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OPPORTUNITIES

Faster patient access across EU

Less effort due to less duplication

Member state equity with respect to HTA

Increased **transparency**

RISKS

Slower patient access, if processes insufficiently operational

Increased effort if 'one-size-fits-none'

Bias towards major HTA countries' evidence preferences

Data overinterrogation and "cherry picking"

MATTERS CALLLING FOR STATISTICAL LEADERSHIP

Design-driven vs policy-driven

EUnetHTA21 draft guidelines have a strong preference for RCTs, yet at the same time must…

- …cover all medicinal products ***and***
- …provide member states with actionable answers to HTA policy questions that were generally not known at time of trial design.

How to strike the balance between relying on RCTs when we can, but going beyond when we must?

Value judgments and statistics

The JCA targets no less than 27 stakeholders with different standards of care and reimbursement systems: what one member state finds unacceptably uncertain to grant reimbursement may be seen as a chance for improving health care by another.

How to ensure analysis, reporting, and assessment that accommodates different value perspectives?

Regulatory and HTA synergy

Estimands have become key in an EMA setting – not so in draft EUnetHTA21 draft guidelines.

Maneuvering HTA perspectives and preferences of 27 member states, is it a missed opportunity to not rely more on estimands to explicate the HTA research question(s) of interest?

How to ensure synergy between the EMA and EU HTA estimand view?

Consistency and transparency

Reflecting the perspectives and preferences of 27 stakeholders, data may tell many different stories, and those stories may not always align.

Public disclosure of a cacophony of technical statistics is not the same as transparency.

How to communicate consistency issues in a way that will increase public trustworthiness of EU HTA?

PICO(s): Population(s), Intervention, Comparator(s), Outcome(s)

The foundation of the EU HTA assessment is based on the Populations, Comparators, and Outcome measures that are HTA-relevant across and among the EU member states – based on (varying) standards of care at the time the regulatory process *starts;* this is different from the regulatory context, in which these are established at the time of the trial design.
Both data sources and statistical methods appropriate to answer these PICO(s) are likely to be highly variable.

HOW CAN WE MAKE OURSELVES HEARD?

Review and comment on draft guidelines (limited success so far)?

Propose viable alternatives?

Whitepapers and broader advocacy?

Join us

WHAT CAN YOU DO?

Want to get more involved in this discussion and help impact the future of EU HTA? Become a member of the HTA ESIG today – scan the QR code or email htasig@psiweb.org

Also, keep an eye out for a November 2022 EFSPI webinar:
Statistics in EU HTA: PICO(s), Estimands & More!