EU HTA – A Model for Evidence Generation in Oncology

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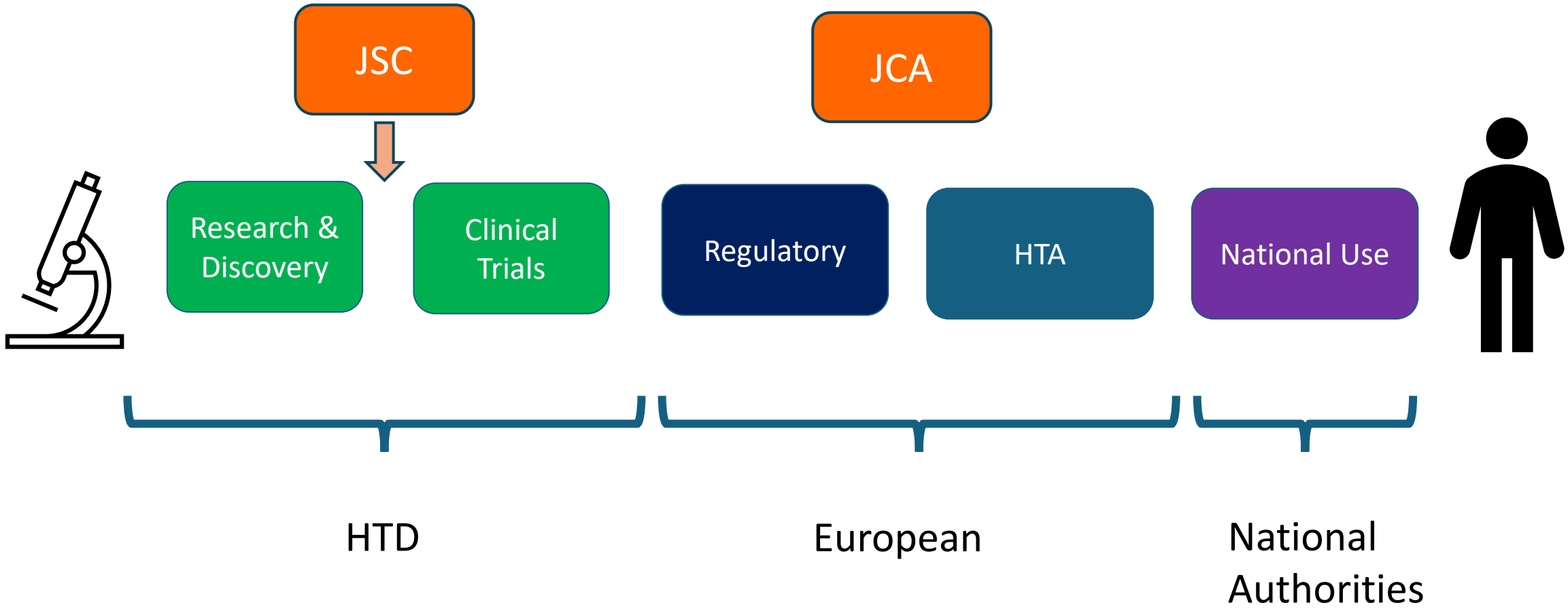
5th October 2026

HTA as part of the horizon



What technologies will be assessed?

Medicinal Products

- 2025-2028
  - New Medicines indicated for the treatment of cancer
  - Advanced therapy medicinal products (ATMP)
- 2028
  - Orphan Medicines

Devices

- 2026
  - Class III implantable **MDs**
  - Class IIb active ARMP* **MDs**
  - **C**lass D **IVD**

that received an **expert panel opinion/views &**

Selection of these as per criteria specified in the HTAR

Since 2025: Joint Clinical Assessments

15 Joint Clinical Assessments

- 11 Oncology
- 3 ATMP

4 Published

- 3 Oncology
- 1 ATMP

4 Discontinued

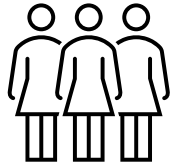
- 4 Oncology
  - 1 Withdrawn
  - 3 Insufficient data

Since 2025: Joint Scientific Consultations

4
HTA &
EMA

3
HTA

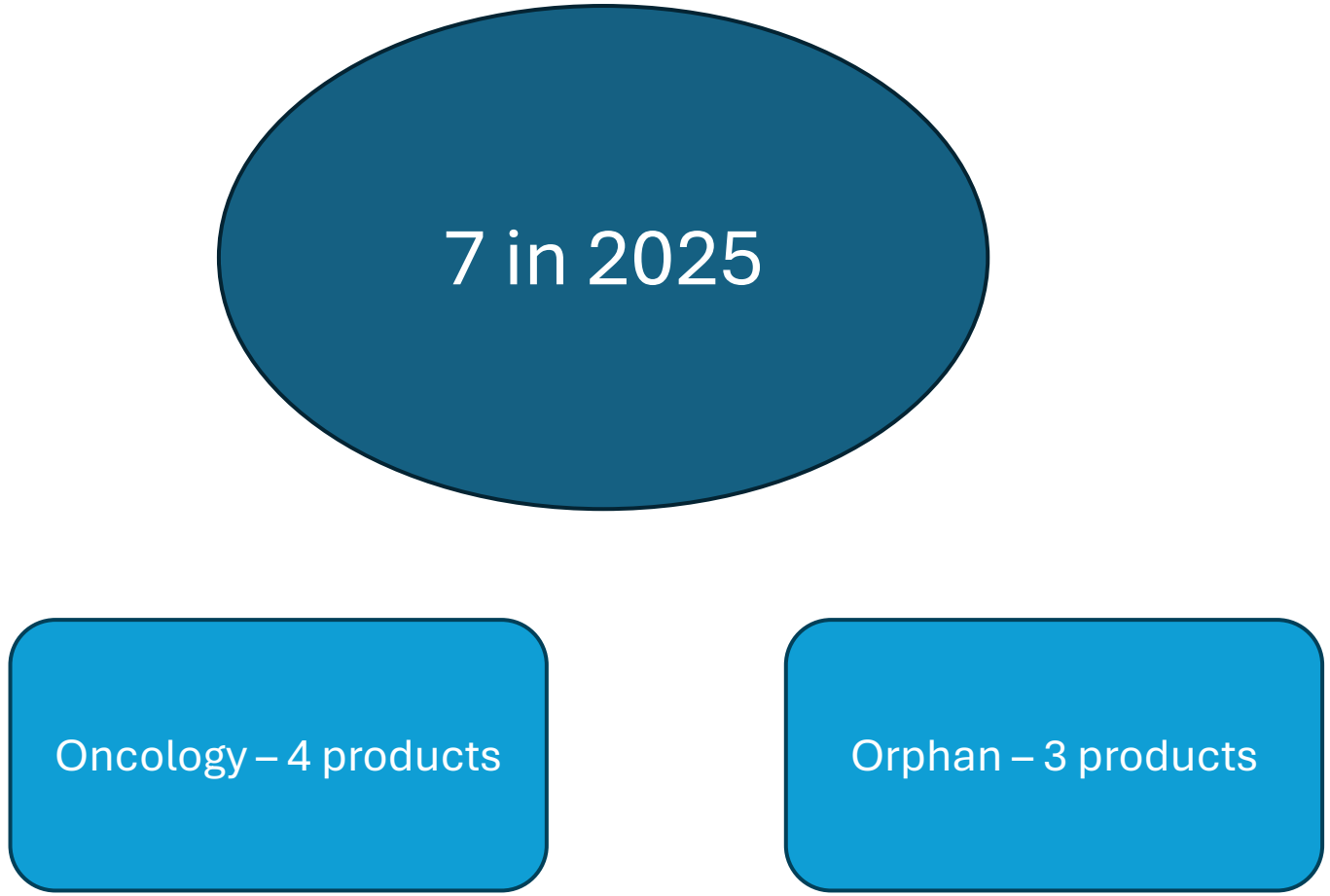
7 in 2025



10 patients
and clinical
experts

Workplan is to complete 8-12 JSCs for MPs in 2026

Since 2025: Joint Scientific Consultations



What does EU HTA offer?

Robust evidence generation

Transparency

Connectivity

Evidence Generation

- EU HTA provides the evidence framework (PICOs & Guidance)
  - Policy questions that payers need to be addressed
  - Maps the disease area (!)
  - Identifies populations of particular interest

- Joint Scientific Consultation can provide the earlier advice on how this is addressed

Transparency

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HTA CG | MEMBER STATE COORDINATION GROUP ON HEALTH TECHNOLOGY ASSESSMENT

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Joint Clinical Assessment Report

Tovorafenib

Endorsed
2021/228

Version 1.0

Endorsed by the Coordination Group pursuant to Article 12(2) of Regulation (EU) 2021/2282 on 30 April 2026

Transparency

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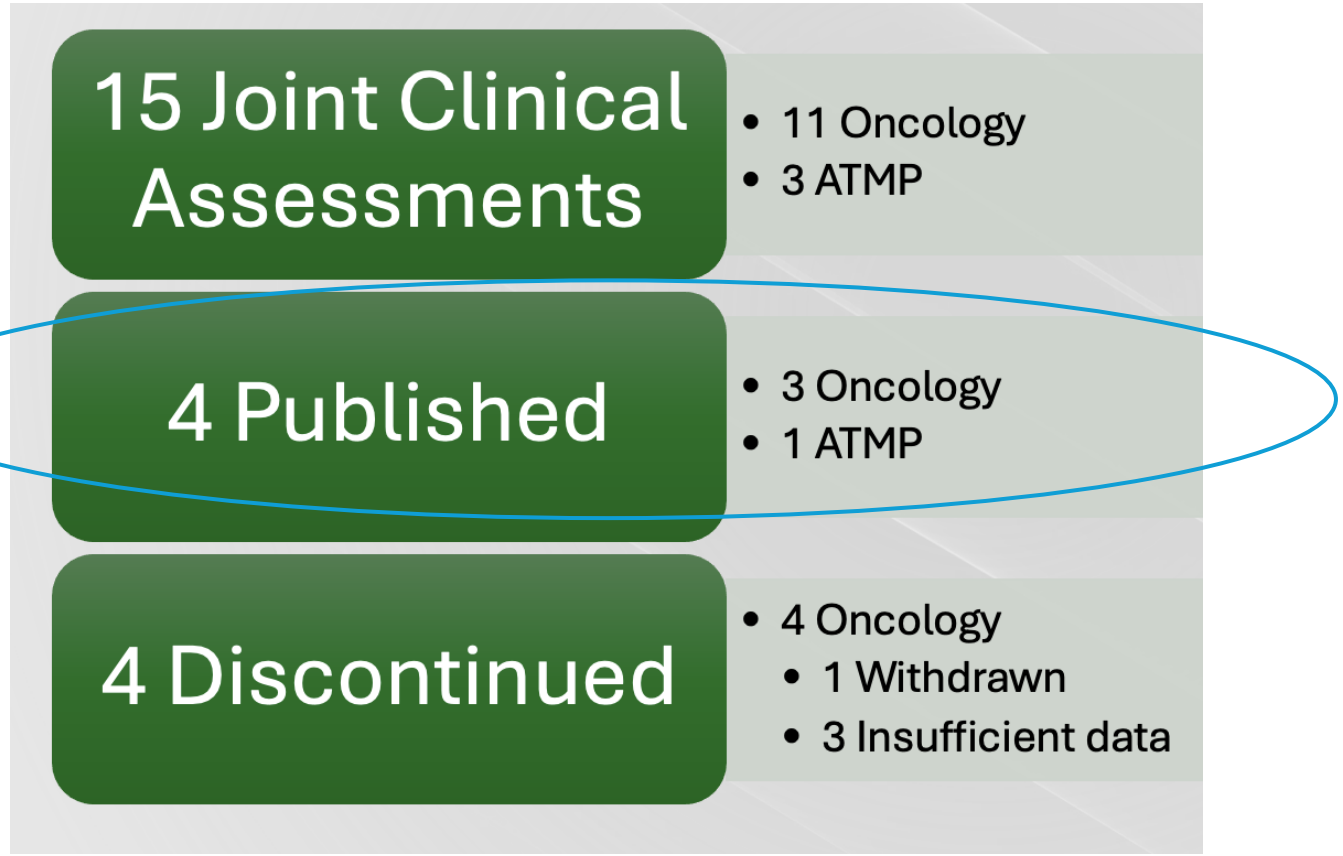
Transparency – what else is published?



IPSEN

Regulation (EU) 2021/2282 on Health Technology Assessment

Dossier of the Joint Clinical Assessment (JCA) of a Medicinal Product (MP)

Tovorafenib (Ojemda®)

Ipsen Pharma

Code: JCA-MP-2024-06

Version 1.0

Date submitted: 4 June 2026

Pur

ANNEX TO THE JOINT CLINICAL ASSESSMENT REPORT

ON

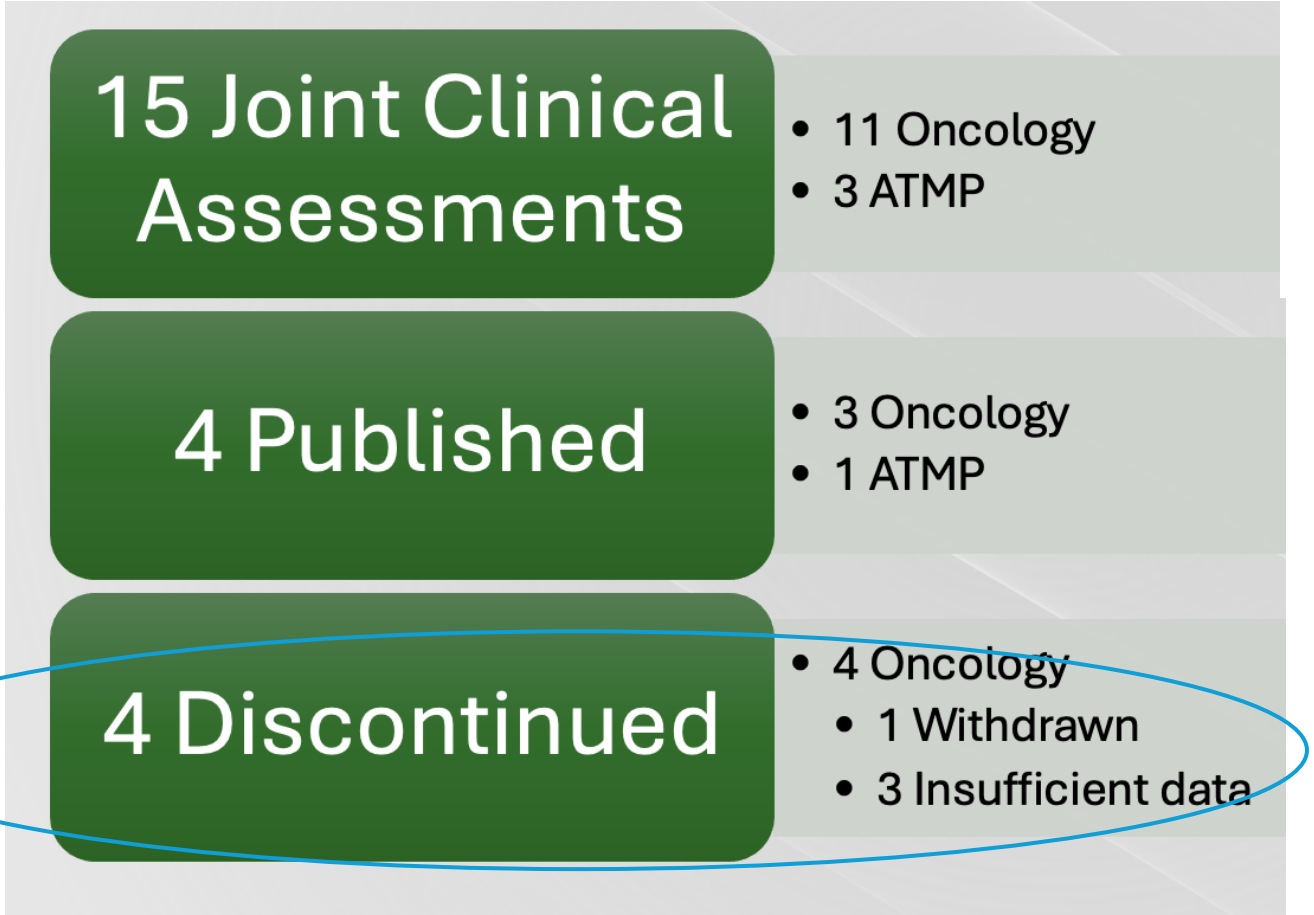
TOVORAFENIB

Factual accuracy check by the Health Technology Developer and response by the assessors

Endorsed
2021/228

Endorsed by the Coordination Group pursuant to Article 12(2) of Regulation (EU) 2021/2282 on 30 April 2026

Transparency



Regulation (EU) 2021/2282 [1]

JCA-MP-2024-03

8 May 2026

The table below lists the information, data, analyses and other evidence that were requested by the Commission in its second request referred to in Article 10(5) of Regulation (EU) 2021/2282 and that were not submitted by the health technology developer Advenchen Laboratories, LLC in the updated dossier (V0.2) of the joint clinical assessment of the medicinal product Catequentinib. Updated dossier submission date: 9 April 2026.

The dossier includes information on two therapeutic indications for the treatment of synovial sarcoma and for the treatment of leiomyosarcoma, referred to as indication a and indication b in the below table.

Transparency

15 Joint Clin
Assessmer

4 Publishe

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JCA-MP-2024-03

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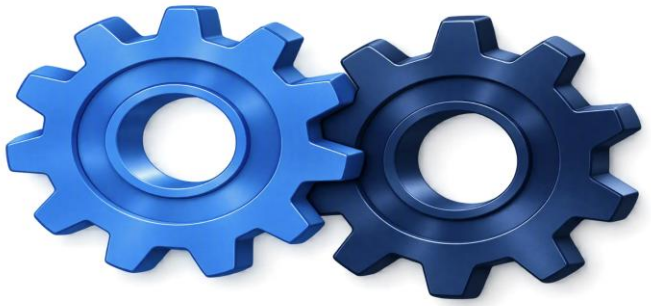
Requirements of Regulation (EU) 2021/2282	Section of the updated dossier	Missing information, data, analyses and other evidence
Article 9(2) Joint clinical assessment dossier "*contains complete and up-to-date information, data, analyses and other evidence […] to assess the parameters included in the assessment scope*"	Section 4. Methods Section 5. Results	**Rationale:** According to Article 9(2) of Regulation (EU) 2021/2282 [1], the dossier shall contain complete and up-to-date information, data, analyses and other evidence submitted by the health technology developer to assess the parameters included in the assessment scope. **Missing for indications a and b:** 1. Transparent and comprehensive identification of all available evidence, followed by a transparent and thorough synthesis to assess the parameters defined in the assessment scope based on the totality of the evidence. 2. Justifications for the inclusion or exclusion of evidence, at a level which enables the assessment of whether the methodological approach is acceptable and, consequently, the validity of the results.
Article 9(2) Joint clinical assessment dossier "*contains complete and up-to-date information, data, analyses and other evidence […] to assess the parameters included in the assessment scope*"	Section 3. Assessment scope	**Rationale:** According to the dossier template [2], Section 3 shall: - reproduce the assessment scope in the format shared with the health technology developer in the Commission's first request; - clearly identify any PICO(s), for which results were not submitted and explain the reasons for their omission. **Missing for indications a and b:** 1. Presentation of the PICOs in accordance with the Commission's first request; 2. Explanation for the omission of results on relative effectiveness and relative safety of PICOs, which would allow the

Transparency

- Publication and national implementation
- Factual accuracy or something else

Faster publication means streamlined national implementation

Connectivity



- Evidence Scope
  - Landscaping the treatment oncology pathways around Europe
  - Identifying the outcomes that matter at face of healthcare provision
  - Connecting evidence via indirect comparisons (and beyond?)
- Publication of relative effect metrics
  - A cross border, transparent record of relative effectiveness early
  - Follow up data can be published and we start to see longitudinal evidence
  - Connectivity to registry, potentially adaptive studies.
- Description of uncertainty and evidence gaps
  - Absence of evidence or absence of submission
  - Clear identification of the uncertainties can lead to better solutions

How should we be planning Evidence Generation in oncology?

Randomise as early as possible

Both sides prefer randomised evidence. Where classic RCTs are hard, use adaptive designs to preserve randomisation.

Use pragmatic & registry-based RCTs

Randomised trials run in routine care or built on disease registries can extend evidence beyond the tightly controlled pre-approval population.

Adopt a shared estimand framework

Regulators and HTAbs may need different estimands. Design trials that can estimate several in parallel, agreed with both audiences up front.



1 April 2025
EMA/115125/2025

Joint HTAb-regulatory perspectives on understanding evidence challenges, managing uncertainties and exploring potential solutions

Outcome of a workshop series between HTA bodies and regulators

What bodies are jointly recommending



Capture non-primary outcomes properly

Keep collecting disease status, symptoms, quality of life and safety after progression or switch to next therapy - don't stop at the primary endpoint.



Ensure timely access to patient-level data

IPD, including baseline covariates for both arms, is essential for robust indirect comparisons and confounding adjustment.



Use real-world data where it actually helps

- RWD is valuable for: natural history and comparator mapping.
- Post-hoc external comparators built after the fact remain unreliable for confirmatory decisions.

Investment in healthcare

How can HTA help direct that investment

Is evidence generation just about a single technology or can it serve many needs