Whitepaper

DLRC

From Regulatory Approval to Patient Access

A practical guide to integrating regulatory, HTA and market access planning in Europe from development to patient access

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Why this matters now

The European access landscape is becoming more

structured, coordinated, and demanding

for healthcare developers.

This evolving environment means that regulatory approval alone is no longer enough.

Strategic alignment between regulatory, HTA and market access is therefore becoming a key driver of successful product development and launch planning, rather than a downstream consideration.

Understanding and managing these interfaces is essential to moving from regulatory approval to real-world patient access in Europe.

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Abstract

Regulatory authorisation is a critical milestone, but it does not automatically translate into patient access. Developers must also anticipate EU and national Health Technology Assessment (HTA) requirements and prepare for country-specific pricing, reimbursement and market access pathways. These considerations increasingly influence clinical development and launch planning well before approval.

The key interface between regulatory, HTA and market access lies in practical strategic choices: how the target population is defined, which comparators are relevant, which endpoints matter, how mature the evidence basis is, and how submission and launch sequences are planned.

For regulatory professionals, whose day-to-day focus is primarily on regulatory strategy and procedures, a clearer understanding of these interfaces can help connect regulatory planning with downstream HTA, pricing and market access requirements. Shared terminology and practical trigger points can support timely cross-functional input, better-aligned clinical development and a smoother transition from market authorisation to national market access and commercialisation.

Introduction

The European access environment is becoming more structured, more evidence-driven and more closely linked to clinical development planning. The EU HTA Regulation has introduced a formal framework for Joint Clinical Assessments (JCA) and Joint Scientific Consultations (JSC), while national pricing and reimbursement processes continue to determine whether and how a medicinal product can be used in individual EU markets.

For regulatory professionals, this creates a practical challenge. Many decisions that appear primarily regulatory can also influence later HTA and payer discussions. Examples include the proposed indication, anticipated target population and label assumptions, the choice of comparator, endpoints, evidence timing and the sequence of submissions across markets. If access implications are considered only after pivotal choices have been made, the ability to adapt can be limited by evidence already generated or by decisions that can no longer be changed.

A working understanding of the interface between regulatory, HTA and market access can help teams recognise where early alignment and integration is useful and where additional input may be required. This paper moves from a broader access context after authorisation to the specific points where regulatory planning, HTA expectations and Market Access considerations intersect in practice. It provides three things: first, a clear explanation of key HTA and Market Access concepts; second, realistic examples of where early regulatory-HTA decision-making adds value; and third, a structured overview of service modules that can be used flexibly depending on the clinical development stage, product type, target indications and strategic considerations.

Regulatory approval and patient access are connected, but not the same pathway

In Europe, regulatory approval and patient access follow different but connected pathways. A medicinal product may be authorised at European level, but national systems still decide how it is assessed, reimbursed, priced and ultimately made available to patients.

This means that the same clinical development programme may be viewed through more than one lens. Regulators focus on quality, safety and efficacy when assessing whether the product's benefit-risk balance supports approval. HTA bodies and payers focus on relative clinical value compared with standard of care, relevance to local practice, budget impact and the conditions under which a product should be funded. These perspectives overlap, but they are not identical.

A changing European environment requires earlier coordination

- The EU HTA framework has made clinical HTA assessment more visible at European level, especially for products in scope of Joint Clinical Assessment
- Joint Scientific Consultations create an opportunity to test evidence-generation plans before pivotal decisions are locked
- National pricing and reimbursement decisions remain essential. EU JCA (HTA) does not replace national value assessment, funding decisions or launch planning
- The practical consequence is that evidence planning, regulatory strategy and access strategy need to be coordinated together and earlier than in many traditional project models

Examples of possible disconnects:

- A comparator selected for regulatory feasibility may not reflect the relevant standard of care in key European settings
- Endpoints may support authorisation but require additional justification for HTA relevance or payers.
- A broad regulatory label may translate into narrower HTA assessment populations

Clear terminology helps regulatory teams recognise access-relevant questions

HTA and market access are often used as broad terms. For a regulatory team, it is helpful to distinguish the main concepts.

Terminology:

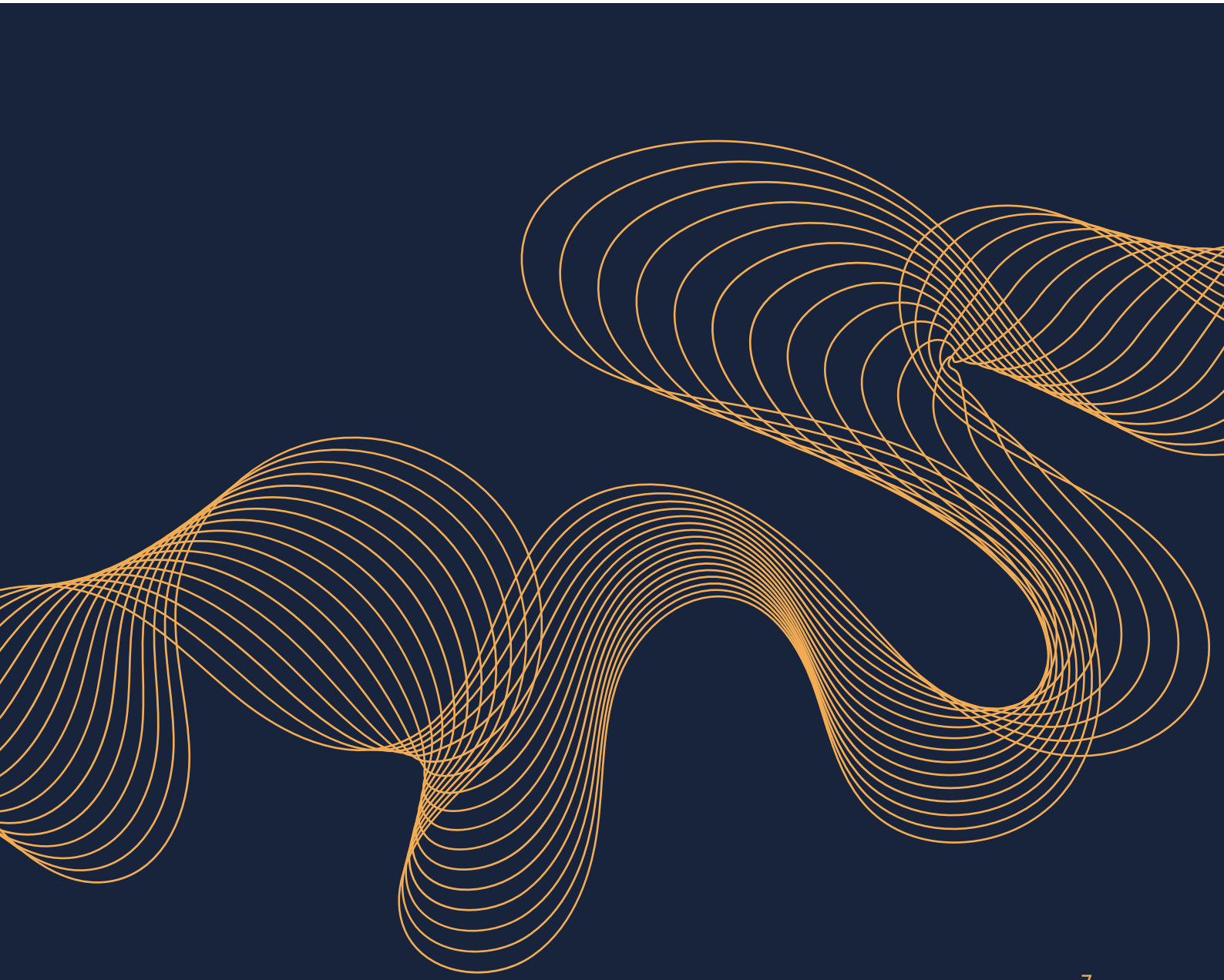
Term	Meaning	Relevance for development planning
HTA	A structured assessment of the clinical value of a health technology, often compared with standard-of-care.	HTA considerations can influence which comparators, endpoints and populations are most relevant.
JCA	A novel EU-level joint clinical assessment of the relative clinical evidence for certain medicines.	JCA is linked to, and runs in parallel with, the centralised marketing authorisation procedure and requires early planning/cross functional co-ordination.
JSC	A scientific consultation with HTA bodies, optionally joint with the CHMP (EMA), on pivotal evidence needs.	A JSC can inform clinical development plans before key study decisions are fixed.
PICOs	The assessment scheme(s) comprising Population, Intervention, Comparator and Outcomes relevant to the clinical assessment.	Regulatory labels and HTA PICOs may differ; early anticipation can help align evidence strategy with approval and access needs.
Market Access	The practical pathway from approval to reimbursement, pricing and use in healthcare systems.	Regulatory success needs to be translated into value and positioning that supports patient access.
Pricing & Reimbursement	National processes that determine whether and how a product is paid for.	These processes are country-specific and shape reimbursement conditions, price expectations and launch planning in each market.

Early alignment helps avoid late-stage disconnects

Regulatory planning does not need to resolve every HTA, pricing or reimbursement question in detail. However, access-relevant considerations can affect key development decisions, particularly where the choice of population, comparator, endpoints and approval conditions may later influence HTA and reimbursement outcomes.

HTA or market access input may be particularly relevant when:

- Pivotal trial assumptions are being defined
- The target population or intended label is still evolving
- Comparator choices may differ between regulatory and HTA perspectives
- Endpoint selection needs to support both approval and value demonstration
- EU HTA, national HTA advice or pricing and reimbursement planning may influence the development or launch strategy



Ordinary development decisions can carry downstream HTA and access implications

The value of an integrated Regulatory-HTA clinical development becomes most visible at specific decision points in the development and submission pathway. These are not separate HTA topics; they are ordinary development decisions that can carry access implications.

Decision points for Regulatory-HTA integration:

Decision point	Regulatory lens	HTA/Market Access lens	Integrated question
Target/Label population	Which population can be supported for approval?	Which subpopulation will be assessed and how do different benefit outcomes in subpopulations affect pricing?	Is the intended label aligned with likely HTA PICOs?
Comparator	Is the control arm acceptable for regulatory evidence generation?	Does the comparator qualify for benefit assessment?	Will the comparator support both approval and access arguments and, if uncertainty persists, should this be clarified through JSC or addressed through additional evidence?
Endpoints	Are endpoints valid and sufficient for efficacy-risk assessment?	Are endpoints accepted by HTA?	Can endpoint selection support both, regulatory and HTA assessments?
Authorisation pathway and evidence maturity	Is the programme aiming for CMA or MAA, and how mature are the data at submission?	CMA may enable earlier market entry but less mature data can make positive HTA outcomes more difficult and affect pricing and reimbursement potential	Does the regulatory pathway balance earlier access with downstream HTA and pricing expectations?

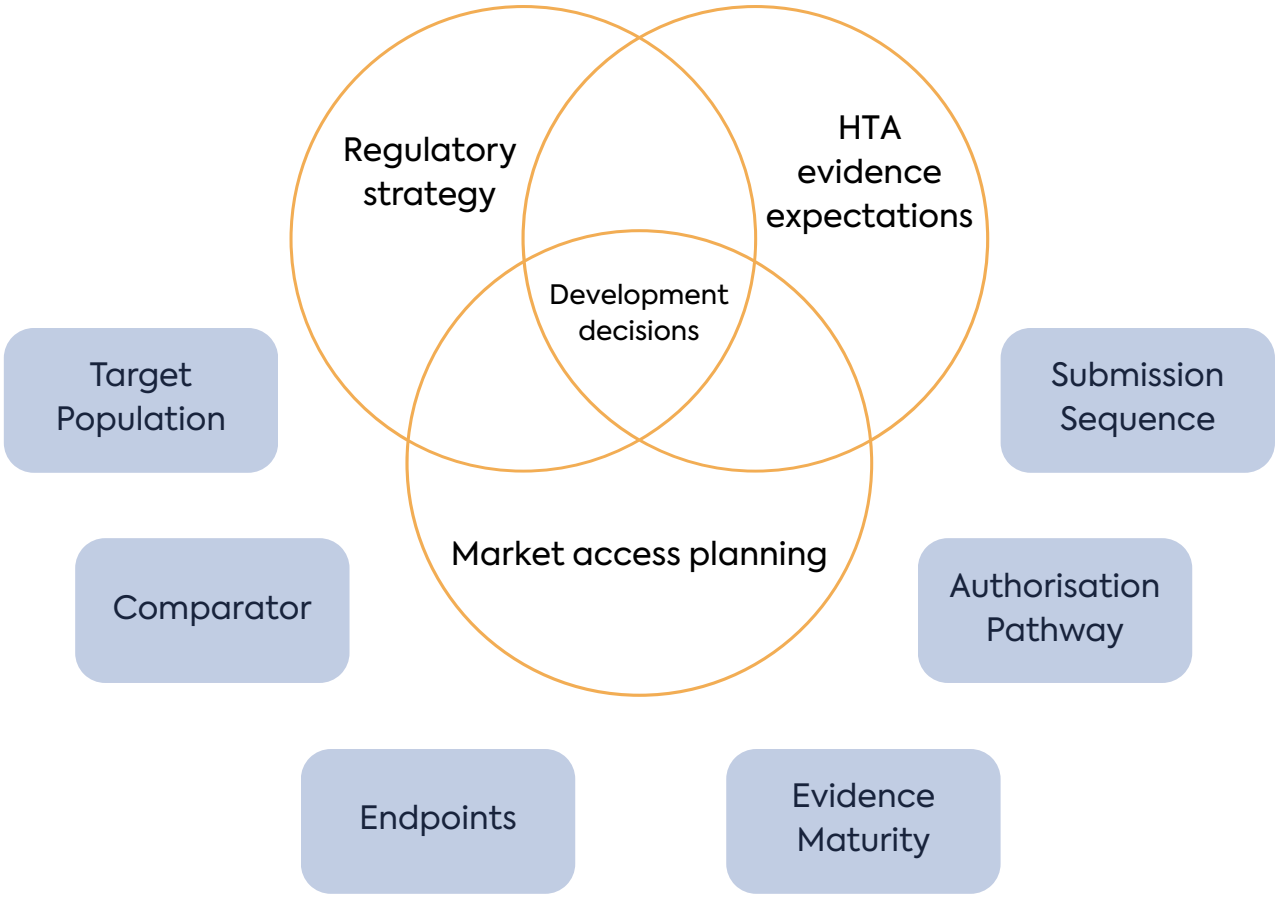
DLRC's Regulatory-HTA integration point

DLRC's integrated Regulatory-HTA model is designed to make these interfaces visible early, so that access-relevant implications can be considered before key development and submission decisions are fixed.

The following approach can turn early signals into coordinated action:

1) Identify access relevant regulatory decisions early
2) Interpret how those decisions may be viewed by HTA bodies and payers
3) Align regulatory, clinical, HTA and market access assumptions
4) Translate alignment into concrete trial decisions, timelines and outputs.

Development decisions sit at the interface of regulatory, HTA and market access

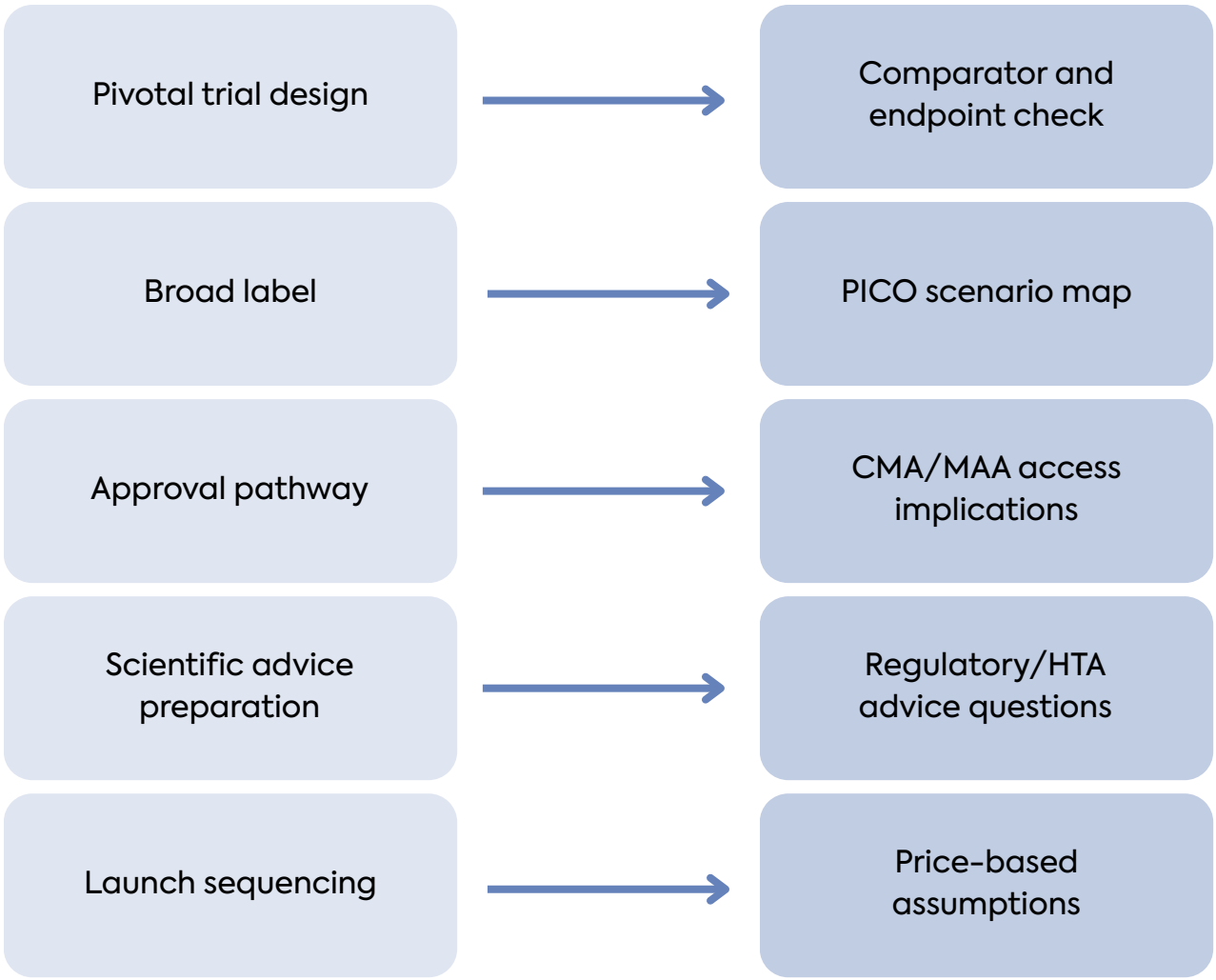


HTA & market access input is most useful when linked to clinical development and submission planning

In practice, access-related input may include an early review of likely HTA assessment questions, comparator and endpoint choices, evidence maturity at submission, and the way an intended label may translate into national HTA settings.

Such input is most useful when linked to concrete clinical development and submission decisions. It can help assess whether planned evidence is likely to support both authorisation and subsequent value assessment, whether a conditional approval pathway may create pricing or reimbursement challenges, or whether additional scientific advice with HTA bodies should be sought before pivotal assumptions are fixed.

Examples of project decisions that benefit from early HTA/market access input



Evidence uncertainty can affect HTA, pricing and reimbursement conditions after authorisation

Single-arm data, immature survival outcomes, surrogate endpoints or limited data for subpopulations can all influence HTA, pricing and reimbursement discussions after authorisation. The key question is how remaining uncertainty will be interpreted and whether it may affect the relative benefit claim, the value proposition, pricing expectations, reimbursement conditions or future evidence requirements.

Evidence uncertainty and access implications:

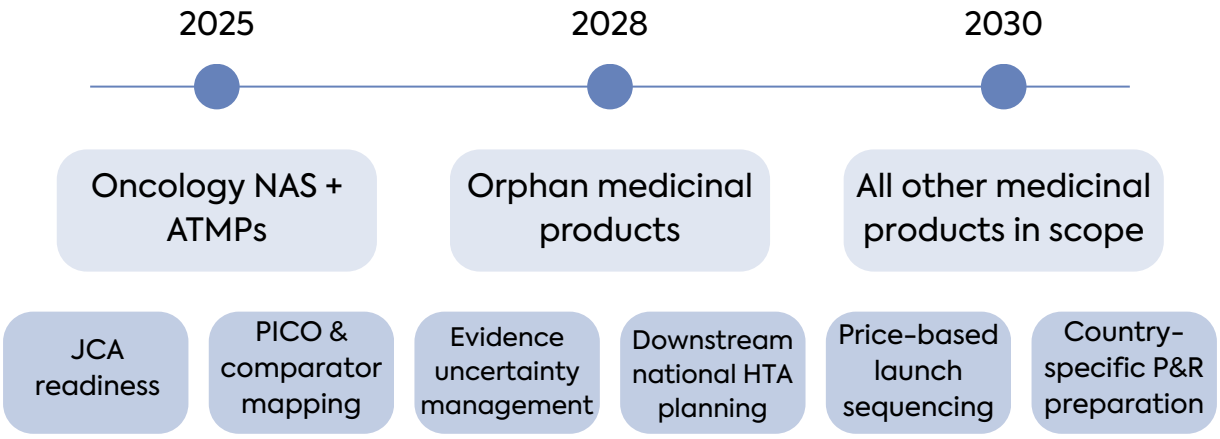
Source of uncertainty	Possible access implication
Single-arm or non-comparative data	Relative benefit may be impossible to demonstrate without robust external or available indirect comparison
Immature survival or durability data	HTA bodies and payers may consider long-term benefit is insufficiently proven at launch
Surrogate endpoints	Validated justification may be needed to show why the surrogate endpoint is clinically meaningful and predictive of relevant outcomes
Small subpopulations or limited patient numbers	Assessment conclusions may be less certain, especially where HTA bodies split the population into narrower groups.
Planned post-authorisation evidence	The timing and relevance of future data may influence reassessment, price negotiations or reimbursement conditions.

Practical implication:

Evidence planning should consider how remaining uncertainty will be explained, reduced or managed in HTA, pricing and reimbursement discussions after marketing authorisation.

EU HTA timelines and national access requirements create planning dependencies across clinical development, approval, and market launch

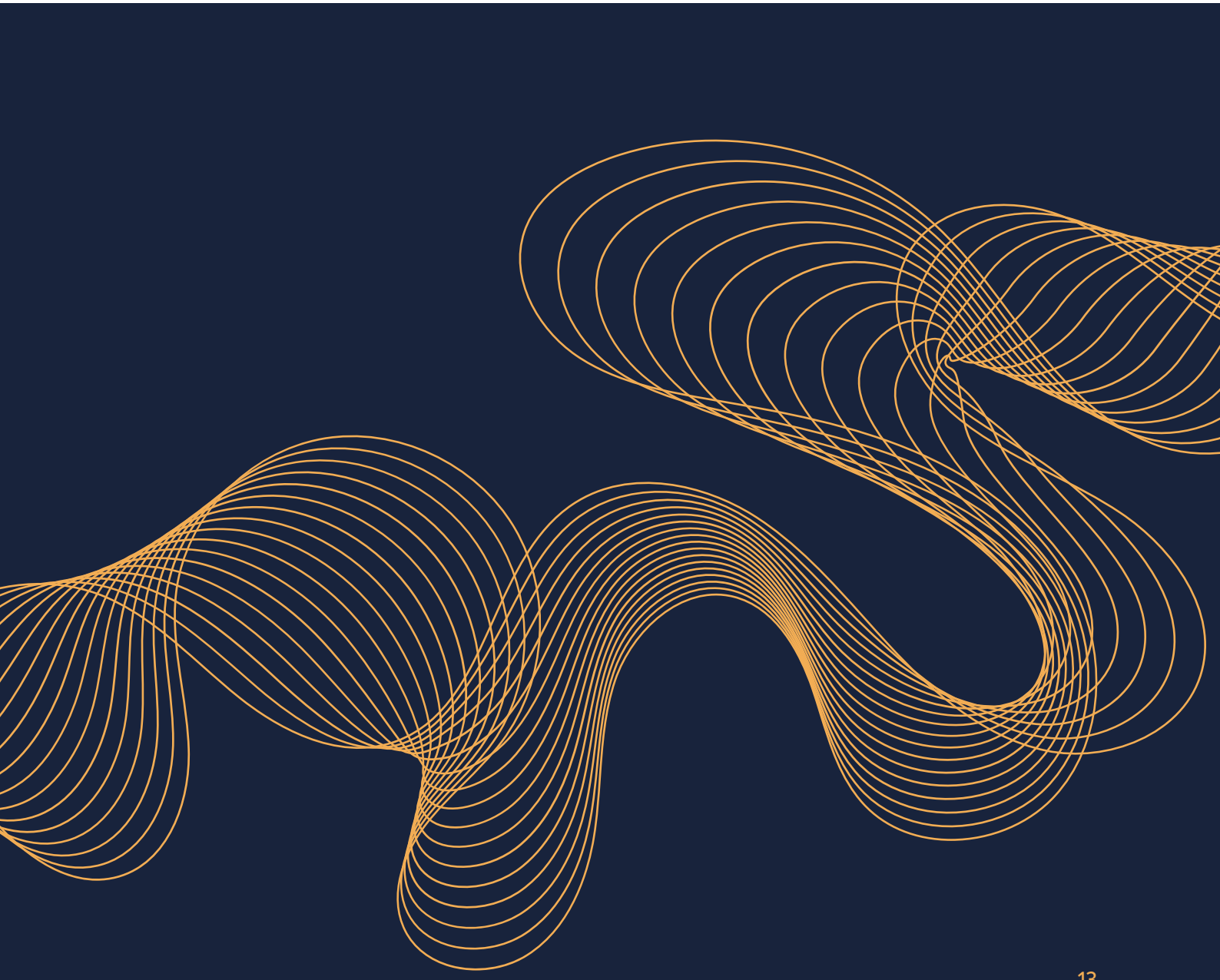
EU HTA requirements are being phased according to a defined timeline. Since January 2025, new active substances in oncology indications and Advanced Therapy Medicinal Products (ATMP) have been subject to Joint Clinical Assessment. Orphan medicinal products will follow from January 2028, and all other medicinal products within the scope of Article 7 of the HTA Regulation from January 2030. At the same time, national HTA, pricing and reimbursement processes remain central to patient access in individual European markets.



The phased introduction of EU HTA creates practical planning dependencies across regulatory submission, EU-level assessment and national market access. Where a product is likely to fall within the JCA scope, the regulatory timelines determine when JCA readiness activities need to start. Before pivotal evidence assumptions are fixed, PICO and comparator mapping can help anticipate likely assessment populations, relevant comparators and outcomes, including potential differences from the intended regulatory label. Where evidence is limited, immature or based on surrogate endpoints or small subpopulations, evidence uncertainty should be assessed for its possible impact on HTA conclusions, pricing expectations and reimbursement conditions. After EU-level assessment and authorisation, planning moves into downstream national HTA procedures, country-specific pricing and reimbursement requirements, and price-based launch sequencing. Typical outputs may include a JCA readiness assessment, PICO scenario map and comparator rationale, evidence uncertainty map with mitigation options, national HTA planning overview, launch sequencing assumptions and pricing and reimbursement pathway map for priority markets.

Planning areas:

JCA readiness	PICO and comparator mapping	Evidence uncertainty management
Downstream national HTA planning	Price-based launch sequencing	Country specific pricing and reimbursement preparation



Regulatory-HTA integrated planning areas can be grouped around recurring planning needs

The following planning areas illustrate how regulatory, HTA, pricing and market access considerations can be combined in practice. A product-specific engagement reflects the product situation, indication, development stage and access objectives, focusing on the decisions that matter most for the specific development, submission and launch pathway.

Planning needs across the European access pathway:

Evidence & advice planning

- PICO and comparator planning
- Evidence gap assessment
- JSC advice strategy

Regulatory submission & EU HTA preparation

- JCA applicability and readiness
- CMA/MAA access implications review

Launch & national access

- EU market access roadmap
- Pricing and reimbursement strategy
- Value story and evidence narrative

Illustrative case examples for development and access planning

The following examples show how access considerations may affect development and submission planning.

Scenario 1: Oncology asset approaching pivotal trial design

A company is preparing pivotal study assumptions for an oncology asset. From a regulatory perspective, the main questions are whether the proposed study design can support authorisation and whether the planned endpoints are acceptable. From an HTA perspective, additional questions arise, e.g., Is the comparator standard-of-care in relevant European markets? Would surrogate endpoints be meaningful for relative clinical benefit? Could the intended label lead to multiple PICOs?

Useful input at this stage may include a focused review of comparator choices, endpoint relevance and likely HTA assessment scenarios. The practical value is that potential evidence gaps can be identified before protocol assumptions are fixed.

Scenario 2: Product pursuing conditional marketing authorisation

A product may qualify for conditional approval based on promising but still immature data. This can support earlier market entry, particularly in areas of high unmet need. However, the same limited evidence package may create uncertainty in HTA and reimbursement discussions.

Useful input may include an assessment of likely HTA limitations, post-authorisation commitments and potential pricing implications. The practical value is a clearer understanding of the trade-off between earlier approval and downstream consequences for market access.

Scenario 3: Orphan product preparing for EU filing

An orphan product may have a strong clinical rationale and a clearly defined unmet need, but the access pathway can differ substantially between countries. The key planning question is not only whether the EU JCA applies, but how the orphan status will be treated in national HTA and reimbursement systems after authorisation.

For example, some countries may apply specific rules or modified assessment pathways for orphan medicines, while others may require a more conventional relative-effectiveness assessment. In Germany, for example, orphan drugs still benefit from a special AMNOG framework as long as annual sales remain below the relevant statutory threshold; once the threshold is exceeded, a regular assessment against an appropriate comparator may be triggered. These differences can affect evidence expectations, pricing discussions, reassessment risks and the timing of national access planning.

Useful input may include mapping orphan-specific HTA rules across priority markets, identifying where and when orphan-specific privilege or modified assessment pathways may no longer apply, assessing whether the evidence package can support both the initial assessment and potential reassessment, and reviewing the role of patient-reported outcomes (PROs). Validated PRO instruments may be particularly relevant where treatment benefit may not be fully reflected by conventional clinical data and where national HTA bodies recognise PRO as patient-relevant.

Targeted readiness checkpoints can reveal whether access considerations are sufficiently reflected in the clinical study protocol

A short readiness check can help identify whether JCA, pricing or reimbursement considerations should be explored further. If several answers are unclear, more focused access input may help clarify downstream access requirements.

EU HTA readiness

- JCA scope
- PICO assumptions
- Comparator relevance

Evidence readiness

- Endpoint relevance
- Evidence maturity
- Post-authorisation evidence

Launch readiness

- National HTA planning
- Price-based sequencing
- Country-specific P&R needs

Conclusion & Future Considerations

Regulatory approval, HTA, pricing and reimbursement are separate steps, but they are part of the same overall journey from clinical development to patient access. Decisions made during clinical and regulatory planning can have a direct influence on how convincingly a product's value can be assessed by HTA bodies and reimbursed by payers.

Integration does not need to make market entry preparation more complicated. When applied, Regulatory-HTA integration helps regulatory, clinical and medical teams identify important questions earlier, involve specialised expertise at the right time and translate strategic considerations into practical actions.

For regulatory professionals in particular, the key takeaway is to recognise when regulatory choices may have downstream access implications and to create a bridge to HTA and market access expertise before the opportunity to influence pivotal trial choices has passed.

Expert Support for Your HTA and Market Access Journey

DLRC's service approach is built around that bridge. In a vendor landscape where regulatory, HTA and market access expertise are often separated, DLRC brings these capabilities together within one integrated consulting set-up. Regulatory and HTA/Market Access teams work together in a regular and harmonised way around the same development, evidence and submission decisions, rather than providing siloed input at separate points in time. This helps developers receive more coherent advice, align evidence planning more effectively and build a clearer route from regulatory approval to patient access.

What Expert Support Means for You:

Maximised evidence ROI

Every research investment should support future reimbursement success. We help you prioritise the evidence generation activities most relevant to HTA bodies and payers, so your development programme delivers stronger pricing and reimbursement outcomes. This reduces evidence gaps and helps direct investment where it creates the greatest value.

Accelerated time-to-reimbursement

Speed to market launch and reimbursement creates a competitive advantage. Our HTA planning starts early, helping to strengthen evidence generation strategies for pivotal studies. By identifying payer-relevant evidence requirements before pivotal studies begin, we help reduce downstream evidence gaps and support a smoother path for reimbursement.

Data transformed into value stories

Clinical data alone is rarely enough to secure favourable access decisions. We help identify evidence gaps early, shape studies that address HTA and payer requirements, and develop submissions that clearly communicate clinical value. This strengthens your value story and supports more effective decision making.

Key takeaway

Integrated regulatory, HTA and market access planning is most valuable when it is early, proportionate and decision focused. It helps developers understand not only how to reach market authorisation, but how to prepare for the access questions that follow.

Disclaimer

This whitepaper is intended as a practical knowledge document and does not constitute legal, regulatory of pricing advice. Product specific conclusions require a case-by-case assessment of the product, indication, evidence package, regulatory strategy and relevant national market access context.

Meet the Author

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Carolin Knorr is Principal Consultant HTA & Market Access at DLRC. She has twenty years of experience at the interface of HTA, pricing and reimbursement and regulatory, with particular focus on European market access. In her role at DLRC, she supports healthcare developers in connecting regulatory strategy with HTA and Market Access planning, including EU HTA readiness, scientific consultation strategy, evidence gap assessment and practical reimbursement considerations.

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