Pharma Design Limited

‘From CTA applications to MAA and HTA dossier submission, Pharma Design supports the pharmaceutical development of medicinal products. We help our customers achieve full access to European Markets by preparing a good strategy from the early stages of clinical development.’

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Navigating the European Clinical Trial Landscape

Comprehensive CTA Submission Strategy for EU, UK, EEA, and Switzerland

Integrated Strategic Planning:

- Selection of Member States based on disease prevalence, site expertise and competition, import and supply chain and regulatory speed.
- Managing CTIS waves and parallel non-EU submissions.

Core Documentation & Quality Excellence (Do-it-right first time):

- Development of HA and EC Dossiers: ICFs, Insurance, local templates
- Expert preparation of high-quality dossiers to avoid questions and reduce timelines.

End-to-End Operational Support:

- Handling of Import/Export licenses
- Professional medical translation and cultural adaptation of Patient Information Sheets (PIS) and Informed Consent Forms (ICF).

Clinical Trial Applications

Accelerating Development via Early HA Interaction

Strategic Engagement with EMA (PRIME) and MHRA (ILAP)

EMA PRIME (Priority Medicines):

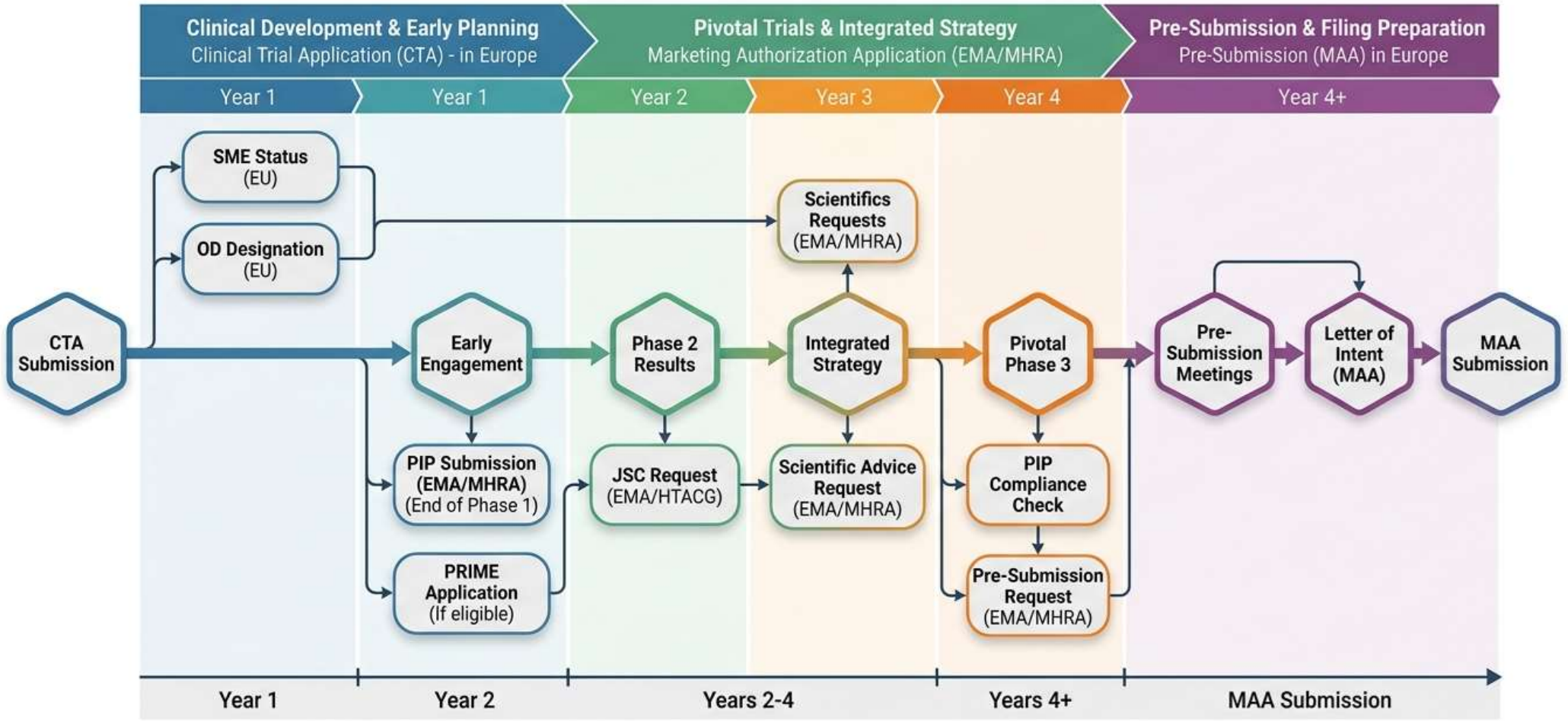
- Identification of eligibility based on "unmet medical need" and "potential for major therapeutic advantage."
- Enhanced support and optimised development plans to reach patients faster.

UK ILAP (Innovative Licensing and Access Pathway):

- Securing the Innovation Passport (The entry point to ILAP).
- Development of a Target Development Profile (TDP): A living roadmap to coordinate regulatory and access milestones.

Early dialogue reduces "blind spots" in the development program and improves alignment on endpoints before pivotal trials begin.

Development flowchart



Bridging the Gap: Early HTA Engagement

Proactive HTA Alignment via JSC and HTACG

EU Joint Scientific Consultation (JSC):

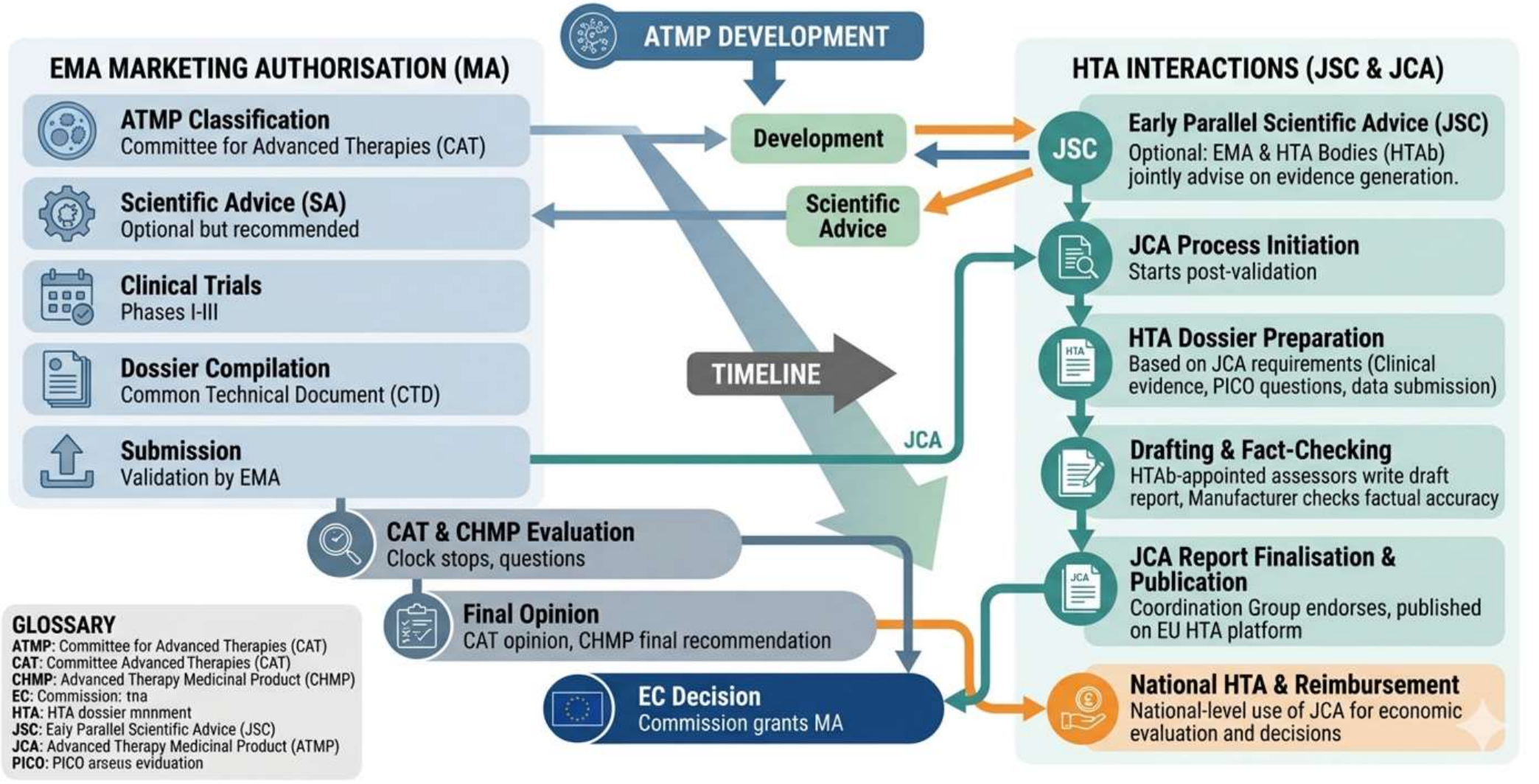
- Engagement with the HTACG (HTA Cooperation Group) to address the 2025/2026 mandatory EU JCA requirements.
- Alignment on PICO (Population, Intervention, Comparator, Outcomes) before Phase 3 study start.

Integrated Evidence Generation:

- Ensuring Phase 3 designs meet both Regulatory (Safety/Efficacy) and HTA (Relative Effectiveness/Value) requirements.

Strategic Outcome: Minimising the risk of "no added benefit" ratings by incorporating payer-relevant endpoints into the pivotal protocol.

Joint Clinical Assessment (JCA) now mandatory for ATMPs



Paediatric & Orphan Strategy

Securing Compliance and Exclusivity via PIP and Orphan Designation

- Paediatric Investigation Plans (PIP):
  - Early Submission (End of Phase 1): Strategic drafting and submission to EMA (PDCO) and MHRA to prevent MAA validation delays.
  - Management of Deferrals and Waivers to align with adult launch timelines.
  - Avoid scrambling for agreement with EMA at phase 3 stage.

- Orphan Drug Designation (ODD):
  - Preparation of the "Prevalence and Significant Benefit" narrative for EMA (COMP).
  - Incentive Maximization: Securing 10 years of market exclusivity and fee reductions.
  - Parallel UK Orphan strategy for MHRA recognition.

The Integrated Scientific Advice Roadmap

Aligning Regulatory and Market Access Strategy

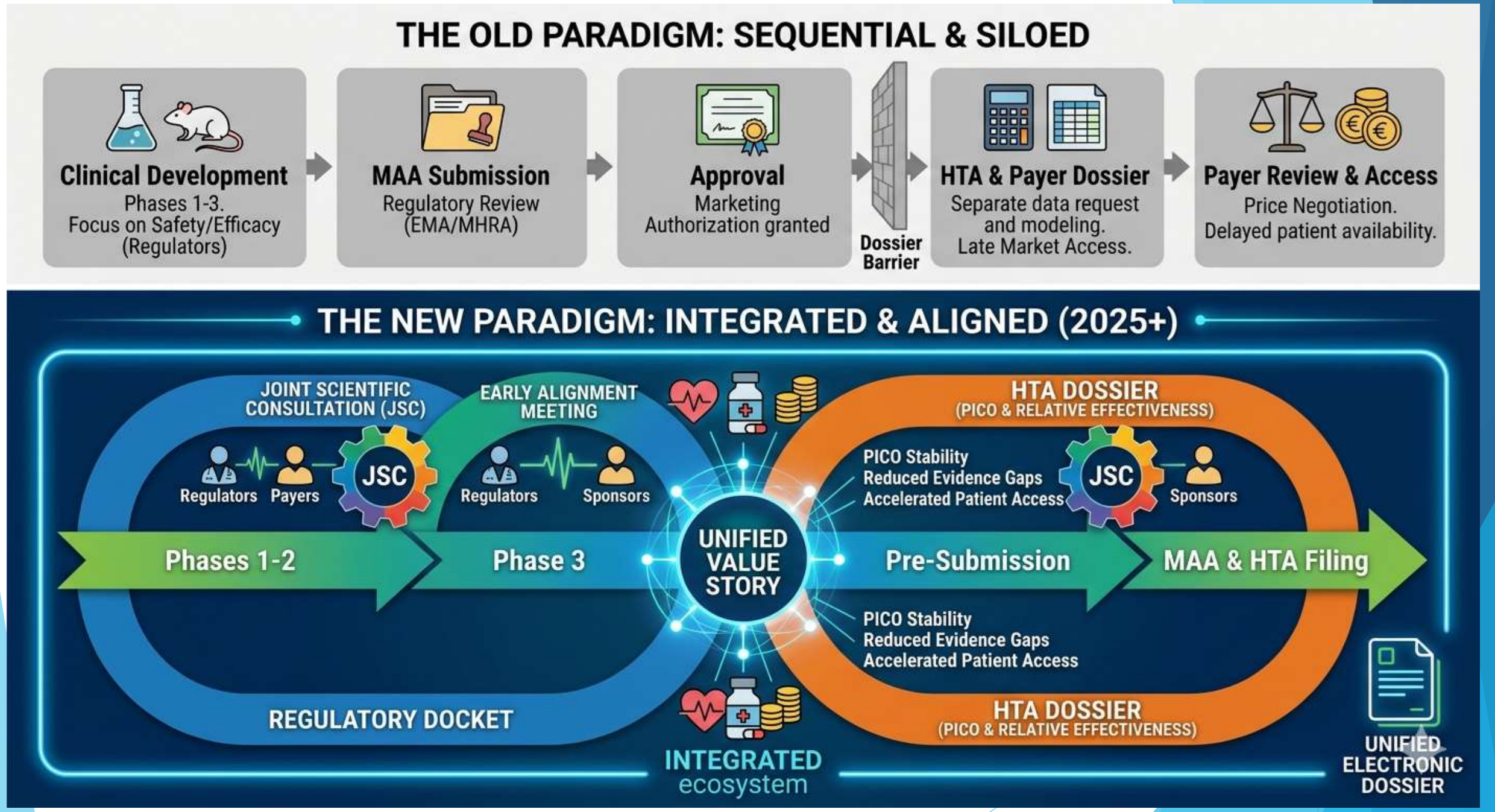
Formal Scientific Advice or Protocol Assistance:

- Strategic preparation for meetings with EMA (SAWP) and/or MHRA.
- Specific focus on Protocol Assistance for Orphan medicines.
- Discuss pain-points early, clinical development gaps or any CMC issue

Integrated Regulatory/HTA Advice (JSC or MHRA/NICE):

- Multi-stakeholder meetings to test the clinical and economic narrative simultaneously.
- Are you really collecting patient-reported outcome measures in a way that enables your Market Access strategy?

The "One-Voice" Strategy: Ensuring that answers provided to regulators do not contradict the value story intended for payers.


THE OLD PARADIGM: SEQUENTIAL & SILOED
Clinical Development
Phases 1-3.
Focus on Safety/Efficacy
(Regulators)
MAA Submission
Regulatory Review
(EMA/MHRA)
Approval
Marketing
Authorization granted
Dossier
Barrier
HTA & Payer Dossier
Separate data request
and modeling.
Late Market Access.
Payer Review & Access
Price Negotiation.
Delayed patient availability.
THE NEW PARADIGM: INTEGRATED & ALIGNED (2025+)
JOINT SCIENTIFIC
CONSULTATION (JSC)
Regulators
Payers
JSC
EARLY ALIGNMENT
MEETING
Regulators
Sponsors
HTA DOSSIER
(PICO & RELATIVE EFFECTIVENESS)
PICO Stability
Reduced Evidence Gaps
Accelerated Patient Access
JSC
Sponsors
Phases 1-2
Phase 3
UNIFIED
VALUE
STORY
Pre-Submission
MAA & HTA Filing
PICO Stability
Reduced Evidence Gaps
Accelerated Patient Access
REGULATORY DOCKET
HTA DOSSIER
(PICO & RELATIVE EFFECTIVENESS)
INTEGRATED
ecosystem
UNIFIED
ELECTRONIC
DOSSIER

The Final Gateway: Pre-Submission Readiness

Ensuring Dossier Acceptance and MAA Filing Excellence

EMA & MHRA Pre-Submission Meetings:

Verification of dossier completeness (e.g., Stability data, Paediatrics, GMO requirements, RMP, labelling, ERA).

- Agreement on Rolling Reviews (where applicable) or Accelerated Assessment.

Deviation Management:

- Negotiating and agreeing on any technical deviations or "missing" data points that can be addressed as post-authorisation commitments.

Submission Quality Control:

- Technical validation of the eCTD structure to ensure a "Right-First-Time" validation by the Health Authority.

About us

- PhD was founded in 2021 by a group of clinical, regulatory, market access and safety professionals willing to combine experience and knowledge from different backgrounds to create an integrated service provider.
- Since then, we have assisted large and small clients to prepare for Regulatory filings across multiple therapeutic areas. Our work has supported over 200 applications, including CTAs, MAAs, PIPs, ODDs, Post Marketing variations.
- Oncology and rare disease are our main areas of expertise with multiple development projects starting from early phase I studies and including different types of products (Mabs, T-cells, bispecifics, small molecules, mRNA immunotherapies).
- We also offer hands-on support to all our non-Europe based colleagues in establishing a presence/legal entity in Europe (EU, UK or Switzerland) and to register with relevant Health Authority portals, including also product registration for Quality and PV compliance purposes).
- PhD operates as a development partner in Europe. Professional expertise that Sponsors can rely on 24/7.

Thank you!

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