

Navigating Market Access with Magnolia

Real World Evidence in Health Technology Assessments

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a medical knowledge group company

Navigating Market Access with Magnolia

Today's Moderators



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Panel Topics

Introduction to HTAs

Role of RWE in HTA decision-making

Case Studies: RWE in Action

Key Takeaways



Our Shared Goal: Access Is at the Center of Everything We Do



One Goal: Many different stakeholders sharing one goal

Different Roles: Different, and necessary roles to reach shared goal

Complicated Journey: Journey can be complicated; many decisions along the way

Data-backed Decisions: Reliance on data to ensure the right decision is made for each patient



Why do the same drugs have different access in different countries?

Introduction to HTAs

What Are Health Technology Assessments (HTAs)?

HTAs are systematic evaluations of health technologies to inform coverage, pricing, and access decisions



What is evaluated

- Medicines
- Medical Devices
- Diagnostic
- Procedures & care pathways



How are they assessed

- Clinical effectiveness
- Safety
- Cost-effectiveness
- Real-world Evidence

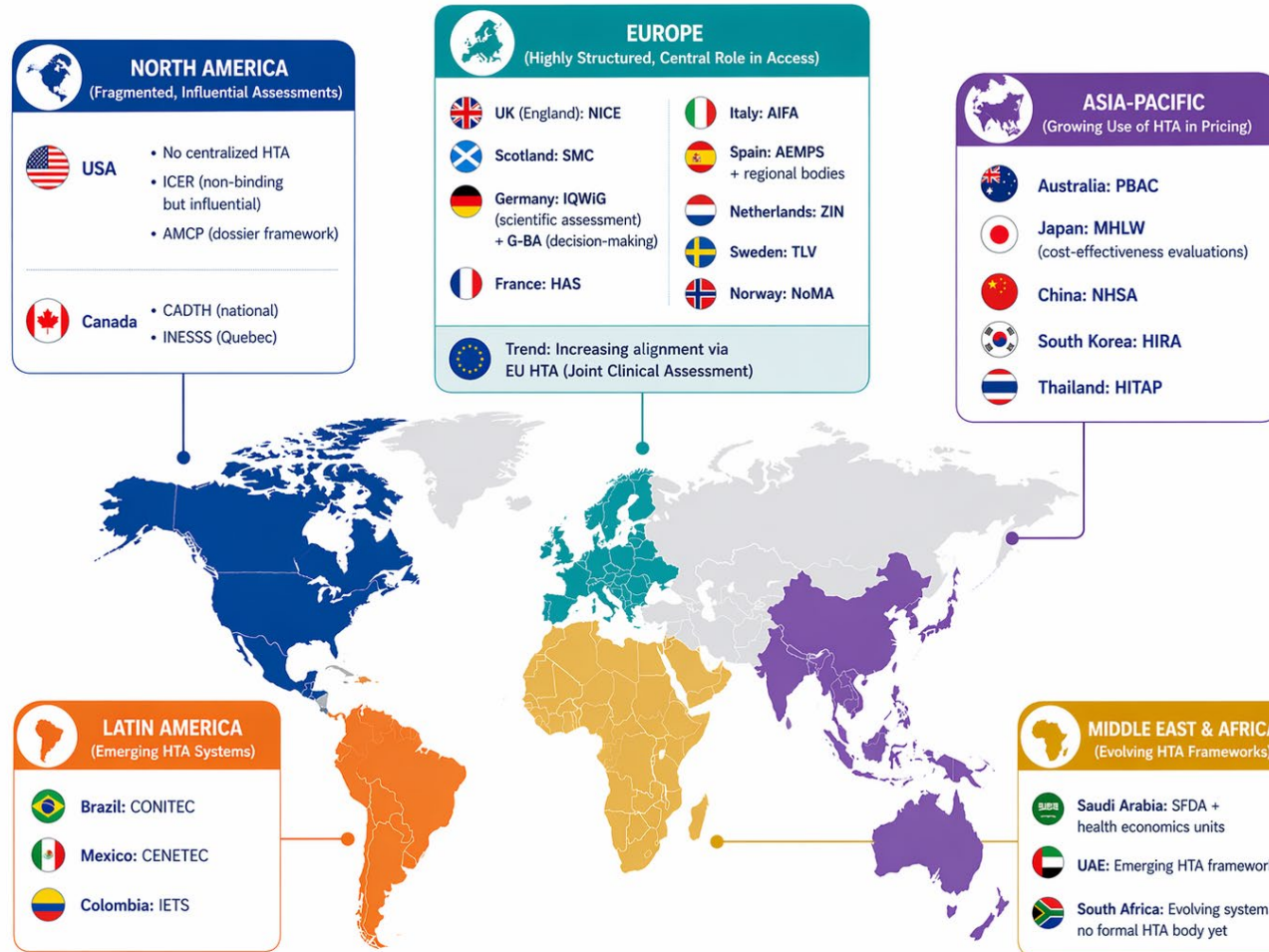


Why do HTAs matter

- Ensure value for money
- Decide reimbursement & access
- Support sustainable healthcare systems

HTAs connect **evidence, value, & access**

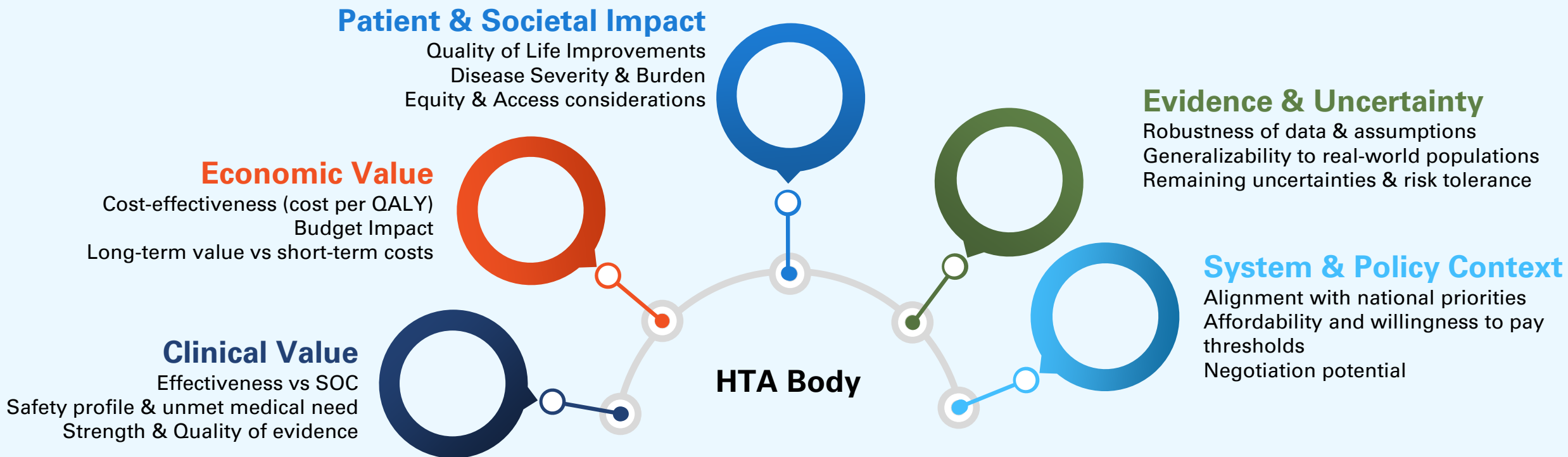
Global HTA Landscape: Key Bodies



Differences across HTA Bodies

- **Centralized vs fragmented** (e.g., NICE vs US institutions)
- **Clinical vs economic focus** (e.g., IQWiG vs PBAC)
- **Binding vs advisory decisions**
- **RWE acceptance maturity** varies by region and agency

How HTAs Evaluate Evidence



HTA decisions balance clinical benefit, economic value, and societal impact under uncertainty.

Role of RWE in HTAs

Where RWE fits into HTA decisions

RWE is most impactful when it addresses specific uncertainties that matter to decision-makers

Generalizability

- Clinical trial populations are narrow
- RWE addresses uncertainty that treatment will benefit broader populations

Long Term Outcomes

- Clinical trials have limited follow-up
- RWE addresses questions about durability of effect and long-term value

Real World Utilization/ System Level Impact

- Clinical trials = controlled environment
- RWE provides evidence on adherence, dosing, treatment duration in real world settings




Evolving Evidence

- HTA decisions are not static
- Indications expand
- Access decisions may change as evidence accumulates

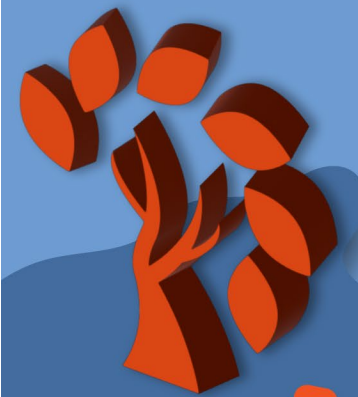


RWE Across the HTA Lifecycle

Iterative Process to Reduce Uncertainty

	Pre-Launch	Launch	Post-Launch
 Goals	Understand disease burden and unmet need Characterize treatment pathways and SOC Inform trial design and comparator selection	Complement RCT data and address evidence gaps Support comparative effectiveness and safety Inform early economic models	Reduce uncertainty and support reassessment Demonstrate long-term value and outcomes Enable lifecycle management and label expansion
 Study Types	Retrospective database studies (claims/EHR) Patient registries Natural history studies	Prospective observational studies External control arms / synthetic comparators Early access program analyses	Longitudinal cohort studies Registry follow-up studies Comparative effectiveness research (CER)
 Relevant Endpoints	Epidemiology (incidence, prevalence) Treatment patterns Healthcare resource utilization (HCRU) Baseline clinical characteristics	Real-world effectiveness (e.g., response rates, progression) Safety and adverse events Adherence and persistence Patient-reported outcomes (PROs)	Long-term clinical outcomes (e.g., survival, progression) Real-world effectiveness vs. comparators Cost and resource utilization Outcomes in subpopulations

Case Studies: RWE in Action



Case Study: Zolgensma (Onasemnogene abeparvovec)

Reimbursement Built Around RWE

Problem HTA bodies faced

- Single-arm trials with small sample size
- Major improvements: survival & function
- ~2M one-time cost

RWE Operationalized

- Mandatory registries at launch
- Data collection a condition of reimbursement
- Outcomes-based payment models
- Coverage with evidence development

RWE Addresses

- Durability of effect
- Generalizability
- Long-Term Safety

RWE Impact

- Embedded in reimbursement
- Enables access despite uncertainty



Case Study: Hepatitis C Direct Acting Antivirals

Addressing System-Level Impacts with RWE



Initial HTA Considerations

- HTA bodies weigh system-level resource allocations
- DAAs: High cure rates; high cost
- Cost-effective per-patient, but can we afford to treat everyone?
- Restricted access due to budget impact uncertainty not clinical uncertainty



RWE Demonstrates

- Real-world cure rates matched trials
- Effectiveness in previously excluded populations
- Downstream health impact (hospitalizations, cirrhosis progression)
- System-level cost-effectiveness



HTA Reassessment

- RWE removed uncertainty that was blocking access expansion
- Real World Effectiveness
- Lifetime cost offsets change the question
- Move from “Can we afford this” to “Should we treat more broadly?”

RWE unlocked access for broader populations

Case Study: Orkambi (Lumacaftor/ivacaftor)

Enabling Access As Evidence Evolves



Initial Rejection

- Trials showed modest effect size
- Uncertainty about long-term impact on exacerbations and survival.
- Very high cost



Managed Access Agreement

- Patients receive treatment
- Data collected in parallel
- Agree to reassess
- RWE enables conditional access and becomes embedded in decision-making



Use of RWE

- UK Cystic Fibrosis Registry
- Treated patients vs historical controls (quasi-experimental design)
- Modest but consistent effects on lung function and disease exacerbation



Ongoing Evaluation

- Uncertainty remains
- RWE allows ongoing access for drug that likely would have been unfunded
- RWE central to ongoing evaluation

Case Study: Multiple Sclerosis Disease Modifying Therapies

RWE Supports Optimal Treatment Decisions

Evidence Gap

- Many similar treatments with similar mechanisms
- Modest effect differences
- Lack of comparative trials

Role of RWE

- Head-to-head comparison across therapies on relapse rates and disability progression
- Treatment sequencing decisions (escalation, switching)
- Subgroup effectiveness

Impact

- RWE informs clinical guidelines and treatment positioning
- Continuous learning system shapes health technology assessment over time



RWE is Most Effective When It Is Used to Reduce Uncertainty

High-uncertainty therapies

Clinical heterogeneity

Large populations

Multi-therapy settings



Support conditional access

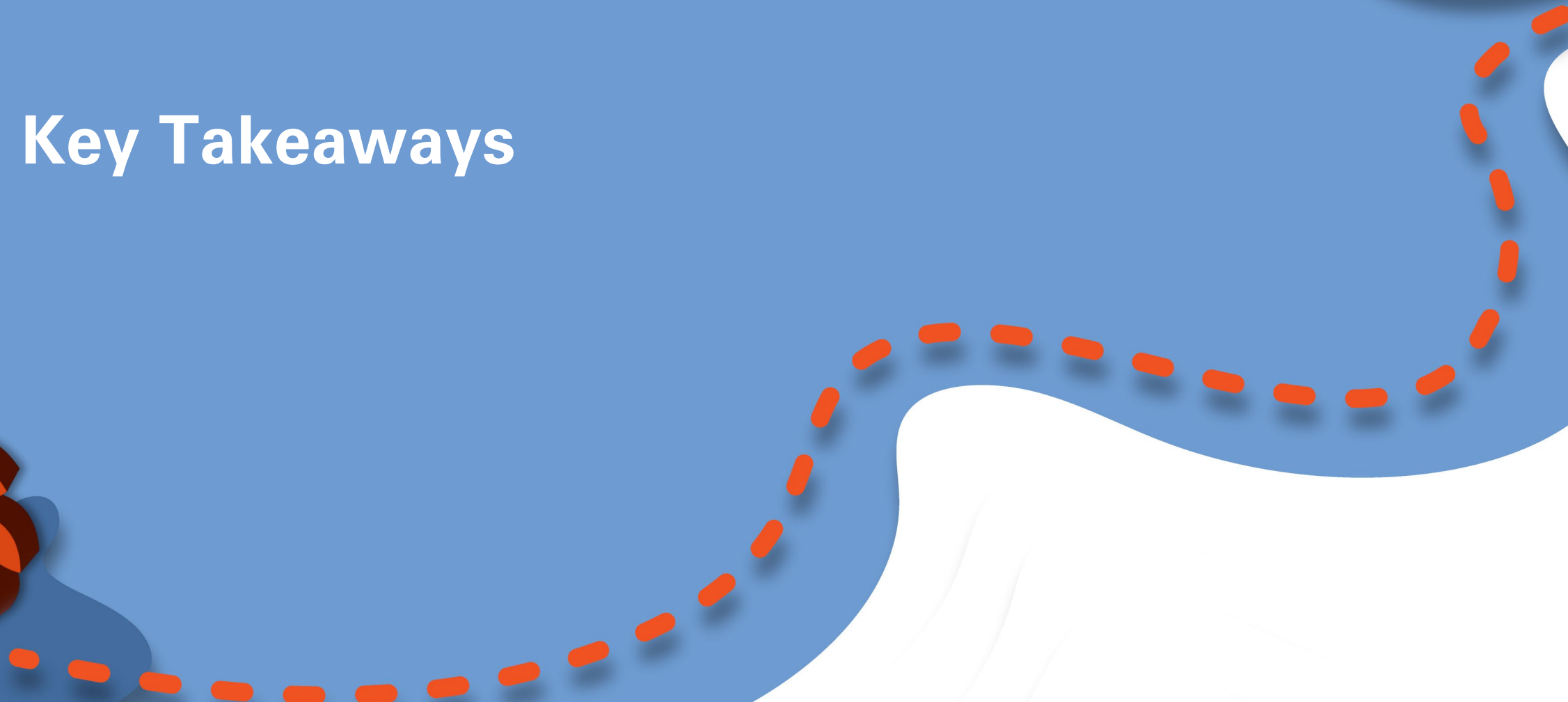
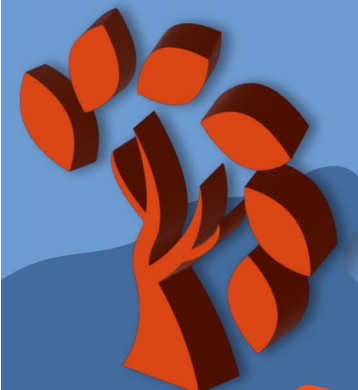
Define who benefits

Informs system-level impact

Enables differentiation



Key Takeaways



Why is This Important in the US?



While US does not have a formal HTA body, HTA thinking is **here**

US payers

- Operating under budget constraints
 - Want to reduce total cost
 - Scrutiny of high cost therapies
 - Formulary tiering and restrictions
 - Utilization management
-

Under international reference pricing or MFN scenarios, US drug prices would be tied to other countries which rely on HTAs

Case Study: HTA Thinking in the US Aduhelm (Aducanumab)



Regulatory Approval

- Aducanumab (Aduhelm) FDA approved for Alzheimer's Disease in 2021
- Key Trial Characteristics:
 - Key Endpoint: Reduction in beta amyloid plaques
 - Limited effect on cognitive improvement
 - Mixed Efficacy Results
 - Narrow Trial Population: Early Alzheimer's



Payer Response

CMS National Coverage Determination (NCD)

- CMS limited coverage to patients enrolled in clinical trials (Coverage with Evidence Development)
- Effectively restricted broad Medicare access

Commercial Payer Response

- Many plans delayed or restricted coverage
- Extensive prior authorization requirements
- Coverage aligned closely with CMS decision

Strategic Implications for Manufacturers

Plan Evidence Generation with Decision-Makers in Mind and Engage Early and Iteratively



Evidence strategy needs to be decision focused, not just data focused

Global and US evidence strategies are increasingly connected

Timing matters

Real-World Evidence Best Practices in HTA Submissions

Ensuring rigor, relevance, and decision impact across data, methods, and evidence integration

Align RWE with Decision Needs

1

Ensure Data Fit-for Purpose

2

Apply Rigorous Study Design

3

4

Integrate RWE into the Evidence Package

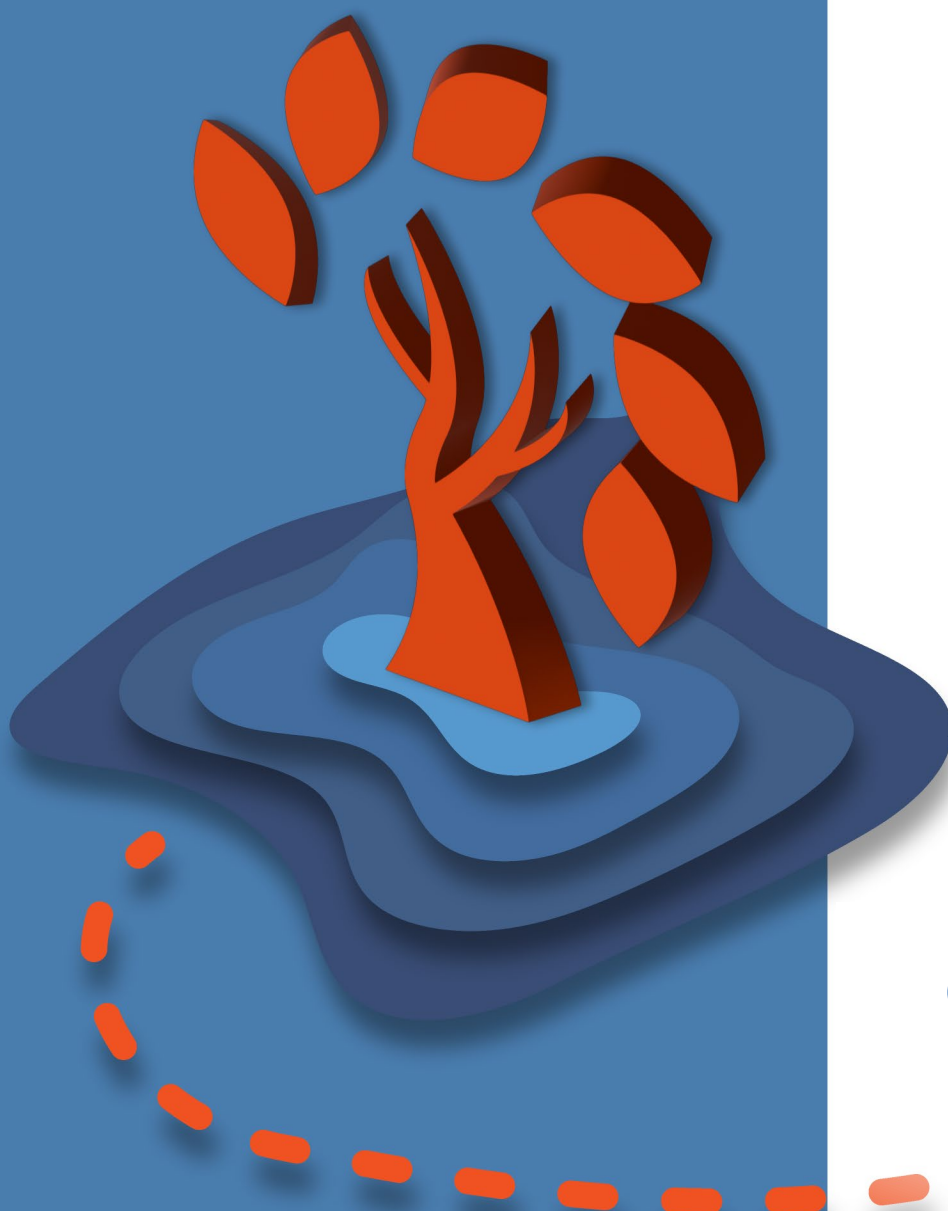
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Engage Early with HTA Stakeholders

6

Communicate Impact Clearly





Join us for our next
Navigating Market Access with Magnolia

**Topic: AI in Healthcare Research,
Emerging Trends, and Real-World
Applications**

**Thursday, May 28, 2026
12:00 - 1:00 PM Eastern**

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