



Real-World Evidence That Wins Access

A lifecycle guide to using RWE to reduce uncertainty, defend value, and accelerate patient access.

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Real-World Evidence That Wins Access reflects an effort by Alkemi to highlight emerging trends in real-world evidence. The information presented is a synthesis of the views of Alkemi and is intended for research purposes only.

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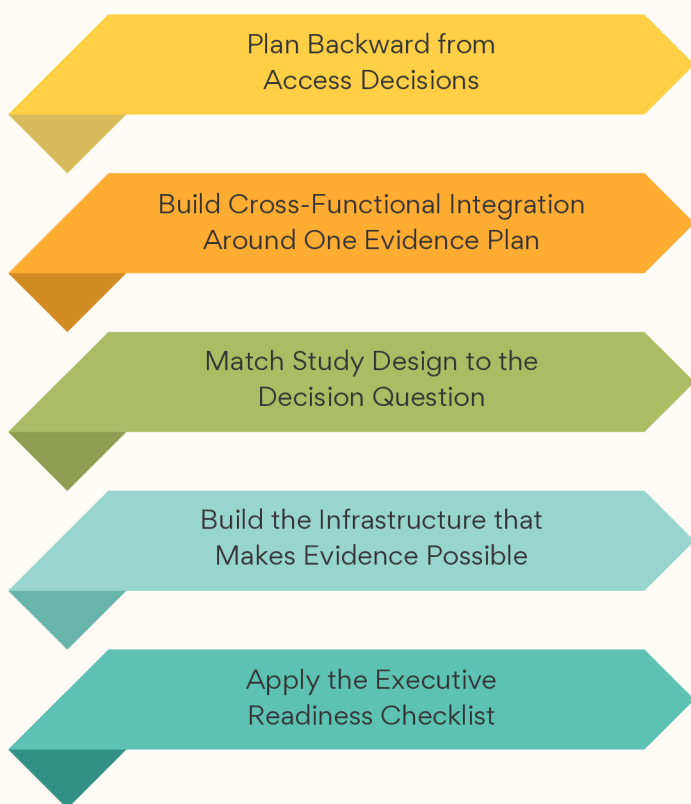
Executive Summary



Planning for real-world evidence starts at innovation.

Early RWE planning gives teams the evidence needed to reduce uncertainty, defend value, and support coverage at launch. When planning begins after approval, the landscape is set and there is limited ability to change the access trajectory.

Exhibit 1: Five Disciplines of Access-Ready RWE



Regulators, payers, and HTA bodies increasingly expect real-world evidence. Infrastructure to generate credible evidence must be built long they ask for it. Many life sciences companies still treat RWE as a post-launch activity. That reactive model leaves teams managing access decisions after payer expectations have already formed.

Planning discipline is what separates RWE programs that change access outcomes from those that only generate publications.

This paper outlines a practical framework for building RWE programs around access decisions, decision-maker standards, and lifecycle value. The goal is simple: help HEOR, Market Access, R&D, Medical Affairs, Marketing, and Commercial leaders treat RWE as a strategic capability, not a late-stage publication activity.

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The Prevailing RWE Model





LATE RWE PLANNING CREATES ACCESS RISK

Life sciences companies often build their RWE programs around a logical but limiting sequence: complete clinical development, earn regulatory approval, and then commission observational studies to satisfy post-marketing commitments, respond to payer evidence requests, and fill publication pipelines. Under this model, RWE begins after pivotal decisions have been made.





By the time most observational programs produce results, the commercial environment that shapes access is formed. Payers have set formulary tiers, written utilization management criteria, and established reimbursement restrictions. Payer expectations about comparative value have crystallized around the evidence available when those decisions were made.

Exhibit 2: From reactive to proactive strategy

REACTIVE RWE

-  Evidence generated after access barriers emerge
-  Driven by payer pushback
-  Seen as cost center
-  Not available when coverage decision made

PROACTIVE RWE

-  Evidence planned before access decisions
-  Driven by anticipated requirements
-  Treated as a commercial asset
-  Enables faster and broader access

Evidence planned early becomes a commercial asset. Reactive evidence generation creates cost, delay, and uncertain return.

Changing those impressions after the fact is substantially harder than shaping key factors in advance. Formulary committees may be reluctant to remove step therapy requirements on the basis of new evidence alone, particularly when utilization controls have already been established.

Budget impact assumptions embedded in payer models persist through subsequent review cycles. Across high-scrutiny launches, companies that bring decision-grade evidence to key access reviews are better positioned for favorable initial coverage than companies that arrive with gaps and promises of future corrective programs.

Medicare’s Coverage with Evidence Development program provides a limited pathway to coverage when evidence questions remain unresolved at launch. Coverage may be available through an approved post-launch trial or registry, but this pathway is uncommon. More often, insufficient evidence leads to delayed, restricted, or denied access.

WHAT CAN AND MUST BE BUILT BEFORE LAUNCH

Except in special cases, such as early access programs, product RWE cannot be generated before launch. However, the foundation for strong comparative product RWE can—and should—be built during clinical development. Teams can generate landscape RWE to clarify the disease burden, treatment patterns, unmet needs, and real-world context for value. These efforts make RWE planning an essential part of development, not a post-launch activity. The next section examines these two types of RWE.

Landscape vs. Product RWE

TWO TYPES OF RWE, TWO DIFFERENT JOBS

One of the most persistent planning errors in evidence strategy is treating RWE as a single category. In practice, the type of evidence a program can generate, the questions it answers, and the audiences it serves all depend on where the product sits in its development and commercial lifecycle. Treating pre- and post-launch evidence activities as the same discipline leads to predictable failures: pre-launch teams skip foundational work that would have strengthened launch positioning, and post-launch teams scramble to build infrastructure that should have been operational at launch. The most useful distinction for planning purposes is to consider landscape RWE versus product RWE, often requiring different data sources, study designs, and governance structures.

Exhibit 3: Landscape RWE vs. Product RWE

	Landscape RWE	Product RWE
Timing	Phase II through launch preparation	Day of first commercial sale and through the commercial period
Primary purpose	Characterize the disease environment, care delivery context, and target patient population for the therapy	Measure how the approved therapy performs in routine clinical practice across the populations in which prescribers are using it
Core questions	<ul style="list-style-type: none"> • What is the real-world disease burden? (Outcomes, costs) • Who is diagnosed, who gets treated, and who does not? • What are the unmet needs and care gaps? 	<ul style="list-style-type: none"> • What are the prescribing patterns, adherence rates, persistence, and real-world clinical outcomes? • How does this product compare with current standards of care in routine use? • What are long-term outcomes, HCRU, and total costs of care
Primary uses	Internal teams, endpoint strategy, epidemiology, JCA, PIE, early scientific advice, market shaping	Payers, HTA bodies, formulary committees, medical affairs, patient advocacy groups, guidelines, care setting value assessment, contracting teams
Common mistake	Using published literature that lags real-world practice instead of current observational data on disease burden and care patterns.	Not building product RWE infrastructure early enough to capture negotiation, uptake, safety, persistence, and outcomes data in time for access reviews.

EARLY EVIDENCE STRATEGY OPPORTUNITIES

Although the product is not yet approved for commercial sale, teams can build the infrastructure that makes RWE possible at launch. This includes data partnerships with health systems and payers, registry design and governance, outcome measurement specs, and burden of illness evidence that anchors the value argument.

Published disease literature can lag current practice. Relying on it exclusively creates commercial risk in therapeutic areas where the standard of care is changing. When a product enters a market with evidence that is several years old, the launch value story rests on an outdated foundation. Real-world database analyses, claims studies, natural history data, and registry evidence can provide a more current picture of the clinical and competitive environment at launch. Landscape RWE establishes the empirical baseline against which the product’s value will be judged.

Manufacturers that define the disease baseline with their own real-world evidence control the comparison.

Landscape RWE also serves a strategic function beyond evidence generation itself. Burden of illness data, treatment pattern analyses, and care gap quantification inform endpoint selection, positioning, market segmentation, and value argument structure before companies engage external audiences. Companies that build this foundation early arrive at payer pre-engagement conversations with evidence rather than projections. That distinction can materially affect the outcome of future negotiations.

RWE STRATEGY STARTS WITH DECISION-MAKER RELEVANCE

A review of patient journey publications (Cribbs et al. 2025) found that 90% of studies captured healthcare utilization metrics. Yet, fewer than half captured essential decision-maker factors, such as treatment experience and disease management outcomes, and more than 75% omitted economic cost or productivity outcomes.

Companies often fund post-launch RWE, yet underinvest in pre-launch RWE.

This imbalance limits the value of RWE for access planning. Utilization data can show how patients interact with the healthcare system, but it may not capture the outcomes decision-makers need to understand unmet need or assess comparative value.

Effective RWE planning must prioritize data sources and outcomes based on decision-maker relevance. Landscape RWE should establish the burden of disease, gaps in current care, and unmet needs. Product RWE should build comparative evidence base needed to demonstrate value after launch.

Creating Commercial Value

RWE CREATES VALUE AT TEN DISTINCT POINTS ACROSS THE PRODUCT LIFECYCLE

The framework below maps ten evidence uses across the product lifecycle. Pre-launch evidence builds the access foundation by defining unmet need, current care, and decision-maker priorities. Post-launch evidence sustains, defends, and extends product value through ongoing demonstration, access renegotiation, and lifecycle expansion.

Exhibit 4: Pre-launch RWE

	Evidence Use	Commercial and Access Value
Pre-launch - Build the access foundation		
1	Disease burden assessment	Quantifies the clinical and economic consequences of the disease in real-world terms. Establishes the baseline against which the therapy's value will be measured and anchors every budget impact and value dossier argument.
2	Epidemiology and patient funnel mapping	Characterizes the diagnosed, treated, and undertreated patient populations. Identifies where patients leave the care pathway without appropriate treatment. Provides the denominator for market sizing and access planning.
3	Unmet patient need characterization	Locates the specific points where the care pathway breaks down — delays between symptom onset and diagnosis, diagnosis and treatment, and treatment and adequate response. Provides the evidence base for the problem-need argument that must precede any value claim.
4	Provider patterns	Documents the clinical referral patterns, specialist use of diagnostics, and sites of care where providers manage patients today. Provides the intelligence needed for medical education strategy, field force deployment, and guideline engagement.
5	Care linkage gap detection	Maps the structural barriers (coverage gaps, care coordination failures, site-of-care restrictions, and regional variation in specialty access) that will determine real-world uptake regardless of clinical efficacy, and that require active access strategy to address.

The commercial logic is straightforward: payers and HTA bodies evaluate products against the current standard of care. Teams that define that baseline with their own real-world evidence are better positioned to shape the comparison.

Early investment creates an empirically grounded narrative about the problem the product solves: who the patients are, how current treatments perform, what management paradigms cost the system, and where the care pathway breaks down. While this foundation does not guarantee favorable coverage, it does reduce the evidence gaps that often lead to restrictive payer coverage conditions.

Exhibit 5: Post-launch RWE

	Evidence Use	Commercial and Access Value
Post-Launch - Sustain, defend, and extend value		
6	Utilization and comparative effectiveness tracking	Monitors prescribing patterns, patient profiles, adherence, persistence, and real-world clinical outcomes from the date of first commercial sale. Enables early detection of divergence from trial-based expectations and supports field teams with current evidence.
7	Access negotiations and value submissions	Refreshes HTA dossiers, P&T committee submissions, and health economic models with current real-world utilization and outcomes data. Addresses the comparative effectiveness and budget impact questions that formulary reviews and managed access renewals generate.
8	Field tools and publication program management	Keeps payer-facing value tools, clinical education resources, and peer-reviewed publications current by integrating long-term outcomes data and comparative real-world effectiveness evidence as it accumulates post-launch.
9	Post-launch safety and outcomes monitoring	Detects emerging patterns in subgroup performance, safety profiles, persistence rates, and competitor activity from routine clinical practice data. Enables a proactive response to evidence developments with patient and thought-leader input before payers use them to propose coverage restrictions.
10	Lifecycle and indication expansion support	Provides the longitudinal real-world evidence base for label expansion submissions, indication sequencing, and line extension strategy, identifying opportunities in patient populations not included in the pivotal program.

RWE for Decision-Makers

WHAT REGULATORS, PAYERS AND HTA BODIES REQUIRE

Regulatory strategy affects access planning because it helps define the evidentiary baseline payers will later assess. Aligning regulatory and access evidence strategies can preserve budget, reduce duplication, and avoid parallel evidence programs.

This alignment is increasingly important because regulatory and payers and HTA bodies can collaborate directly and early (e.g., joint review). Additionally, each publishes their own frameworks and methodological standards for how RWE should be generated, evaluated, and applied.

THE FIVE QUESTIONS THAT SHAPE ACCESS DECISIONS

Payer systems and HTA frameworks differ by market, but their core access questions are consistent: What problem does the product solve, for whom, compared with what, at what cost, and with what degree of confidence? RWE programs structured around those questions create the clearest path to decision-grade evidence.

Exhibit 6: Five questions decision-makers ask

Decision-maker Questions	RWE must show and support
What is the real-world disease burden in the covered population?	Epidemiology, burden of illness, and care gap evidence calibrated to the payer's actual covered population, not the trial enrollment criteria.
Is the current standard of care insufficient?	Treatment pattern data, real-world clinical outcomes under existing therapies, persistence and adherence rates that reflect actual patient behavior, and comparative evidence on where current options fall short.
Will this therapy deliver clinical value in care?	Real-world effectiveness evidence, comparative effectiveness in the populations the payer covers, and subgroup outcomes in patients with comorbidities or clinical profiles that pivotal trials did not represent well.
Is the budget impact sustainable?	Budget impact models grounded in real life clinical practice (i.e., utilization rates, resource use, discontinuation) and total cost of care, not projection models built from trial population assumptions that may not transfer to the covered population.
Is the cost-effectiveness acceptable?	HTA-aligned economic evaluations that justify non-trial assumptions with real-world treatment patterns, provide sources for missing rates, and reduce uncertainty in parameter estimates

STANDARDS FOR DECISION-GRADE EVIDENCE

As regulators and payers review more RWE submissions, their methodological expectations continue to rise. Submissions that fall short can undermine credibility with reviewers who are increasingly able to identify weaknesses in study design, data quality, and reporting. In some cases, weak RWE may create more risk than submitting no evidence at all.

Exhibit 7: Standards for decision grade evidence

Standard	What it requires in practice
Fit-for-purpose	The data source, study population, outcome definitions, and follow-up period match the specific regulatory or coverage decision the evidence is meant to support. Evidence designed for one purpose does not automatically serve another.
Pre-specification	Protocol and statistical analysis plan are finalized before any data lock or analysis. Reviewers at FDA, NICE, and CADTH now explicitly flag post-hoc specification of hypotheses, endpoints, or analytical approaches as a credibility risk.
Bias identification and mitigation	Study teams identify confounding by indication, selection bias, immortal-time bias, and misclassification, and address them through design choices including target-trial emulation, active comparator designs, and new-user frameworks. Prespecified sensitivity analyses document the bounds of uncertainty.
Transparency and reproducibility	Study teams fully document methods, data provenance, variable definitions, and the handling of missing data and competing risks according to applicable reporting standards, including RECORD-PE, HARPER, and STaRT-RWE.
Timing relative to the decision	Evidence reaches the decision-maker when the regulatory, HTA, or formulary review is underway. Evidence that arrives after a coverage determination is too late and must wait for the next decision cycle, which may be 12 months or longer.

HOW MAJOR DECISION-MAKERS EVALUATE RWE

RWE requirements vary across payer and HTA systems, but several structural patterns hold across major markets. HEOR and Market Access teams need to understand these patterns when building global evidence programs.

Exhibit 8: Payer and HTA expectations by body

Body	Where RWE creates the most value	Key expectation
NICE (England)	Resolving uncertainty about long-term outcomes, external validity in patient populations, managed access evidence collection, and real-world comparison with funded alternatives.	Transparent data provenance, pre-specified protocol and analysis plan, evidence delivered on the committee's review timeline.
ICER / US Commercial Payers (United States)	Comparative effectiveness in covered populations, real-world utilization and persistence, budget impact calibrated to patient volumes, and post-approval evidence updates.	Payer-relevant endpoints, credible comparative framing, and evidence addressing the specific population the payer covers — not the clinical trial enrollment criteria.
G-BA / IQWiG (Germany)	Contextual and transferability evidence where RCT-based comparative data are unavailable or where real-world practice differs materially from trial conditions. Germany applies exceptional-case standards to non-randomized comparative evidence at launch.	Germany applies the highest standard in Europe for comparative causal evidence. Observational evidence qualifies only in justified exceptional cases and cannot substitute for RCT-based benefit assessment at launch.
CDA-AMC (Canada)	Post-market evaluation, oncology reassessment, and evidence supporting reimbursement reviews where real-world outcomes data are available. National reporting guidance provides explicit standards for both regulatory and HTA submissions.	Transparent reporting aligned with Canadian national standards, evidence that serves post-market review as well as initial reimbursement assessment.
CMS (United States)	Coverage with Evidence Development, where Medicare coverage is conditioned on enrollment in approved clinical studies. Post-marketing evidence generation is a condition of access for products with insufficient evidence at approval.	Evidence collection infrastructure, registry design, data capture workflows, and governance, must be in place at the time of the CED agreement.

Case Studies: RWE Outcomes



EVIDENCE PLANNING PRODUCES MEASURABLE ACCESS OUTCOMES

The case for early, structured RWE investment is clear. NHS patient access data, FDA approval records, and peer-reviewed analyses of label expansion patterns show that RWE performs best when treated as a planning discipline. Across these examples, stronger access outcomes appear linked to earlier, more specific evidence design.

1 >

Earlier access for patients: NHS Cancer Drugs Fund

Patients can reach treatment sooner when evidence is planned before decision-makers ask for it.

The NHS Cancer Drugs Fund in England demonstrates how structured RWE planning can support patient access. Since the program was redesigned in 2016, approximately 102,114 patients have received cancer treatments through the Fund, spanning 279 indications and 116 drugs. (NHS 2024)

More than 63,000 patients accessed treatments through managed access arrangements while evidence collection was ongoing. An additional 23,500 received interim access because a credible, pre-specified evidence plan gave NICE's appraisal committee confidence that remaining uncertainty would be actively addressed.

The lesson is that structured planning, credible governance, and decision-linked evidence generation can make access possible before final evidence is available.

2 >

Reaching patients that trials missed: Label expansion through RWE

Longitudinal real-world data can reveal patient segments that benefit from a therapy beyond its original label. When those signals are credible, they can inform evidence generation for label expansion and help extend access to patients who would otherwise remain outside the approved population.

Palbociclib's 2019 supplemental approval for male patients with hormone receptor-positive, HER2-negative advanced or metastatic breast cancer illustrates how RWE can support access for populations underrepresented in pivotal trials. Because breast cancer is rare in men, the clinical trial evidence base was limited. Real-world data helped show that men were receiving and responding to palbociclib in routine practice, contributing to the evidence package for label expansion.

This pattern is becoming more common. A 2024 review of FDA supplemental approvals from January 2022 through May 2024 found that RWE contributed, or likely contributed, to 25.2% of label expansions. (Cohen et al. 2024) That figure may understate RWE's role because FDA public documentation does not always disclose which data sources influenced the final decision.

3 >

Shaping lifecycle opportunity: Insights to protect portfolio

RWE can generate lifecycle management insights for both strategic brands and the broader portfolio. Regular reviews of real-world data can surface unanticipated risks or benefits, identify early trends, reveal shifts in care patterns, and support competitive threat mitigation.

Established RWE programs also provide a strategic buffer when assets face unexpected challenges. When concerns emerge around a perceived class effect, suspected safety signal, or competitive threat, teams with mature RWE infrastructure are better positioned to mobilize relevant data quickly. That evidence-based response may help address uncertainty and reduce the risk of restrictions driven by incomplete information.

These examples create a strong investment rationale. Real-world data can contribute meaningfully to lifecycle strategy, portfolio protection, and global revenue potential. For that reason, RWE infrastructure should be built early in the commercial planning cycle. With the right planning, RWE becomes a strategic asset in its own right: a source of insight and evidence that protects the portfolio, supports expansion, and helps mitigate clinical and commercial risk.



Building an RWE Program That Wins Access



FIVE DISCIPLINES OF ACCESS-READY RWE

1 >

Plan backward from access decisions

Many RWE programs fail structurally because they plan forward from study initiation instead of backward from the anticipated access decision.

Backward planning reverses the sequence. Teams first identify the likely timing of HTA submissions, formulary reviews, managed access renewals, contracting milestones, and reimbursement reassessments that will determine access. They then specify the evidence each decision requires and calculate the latest start date that still allows results to be available before the decision occurs.

The exercise consistently reveals that critical evidence activities must begin earlier than teams expect, particularly when studies depend on data partnerships, registry governance, or longitudinal follow-up.

The output is a decision-linked evidence map: each access event is tied to a submission deadline, required deliverable, accountable owner, and initiation date.

2 >

Build cross-functional integration around one evidence plan

Evidence programs usually fail at the boundaries between functions. Market Access builds arguments from the evidence available. HEOR designs studies around scientific priorities. Medical Affairs manages publications and scientific exchange. Commercial builds value narratives from clinical data. Each workstream may be sound in isolation, but without a shared organizing framework, the portfolio becomes fragmented and misaligned with the access objectives it was meant to serve.

Effective evidence programs start with an integrated plan. The plan defines the proof domains required for launch and lifecycle management, including burden of illness, treatment patterns, comparative effectiveness, persistence and adherence, budget impact, and long-term safety and durability. Each activity is then tied to a named decision milestone and a primary stakeholder audience.

3 >

Match study design to the decision question

Designing a study around a relevant question sounds obvious. Yet many RWE studies start with the available dataset rather than the decision the evidence must support.

Payer claims are often the default choice under time pressure, but they were not designed for research. They can support credible evidence only with careful curation, fit-for-purpose assessment, and transparent methods. Payer and HTA reviewers can recognize studies designed for convenience rather than decision relevance, and they discount the evidence accordingly.

For causal questions, target trial emulation offers a defensible observational approach because it makes the comparison explicit before analysis begins. Investigators define the randomized trial they would have run, then design the observational study to emulate it as closely as possible. That means specifying eligibility criteria, treatment strategies, time zero, outcomes, and follow-up before analyzing the data. NICE's RWE framework recommends this approach for non-randomized comparative effectiveness studies. (NICE 2022)

4 >

Build the infrastructure that makes evidence possible

Evidence infrastructure must exist before access depends on it. Registries, longitudinal follow-up programs, and structured data collection in clinical workflows cannot be commissioned at the moment a payer or regulator asks for evidence.

They require data access agreements, governance frameworks, workflow integration, fit-for-purpose registry design, quality oversight, validation, monitoring, and escalation processes for data quality issues. These components take longer to build than the window between a payer request and a formulary review. Reactive infrastructure planning therefore creates a predictable failure: the evidence is not ready when the decision will be made.

5 >

Apply the Executive Readiness Checklist

An evidence portfolio can be individually reasonable and still collectively misaligned. That is one of the most expensive failures in commercial planning.

Evidence Program Readiness

FUNCTIONAL LEADER CHECKLIST

Leaders in Market Access, HEOR, Commercial, and Medical Affairs can use this checklist to assess whether their evidence program is ready to support access and lifecycle strategy. It focuses on the planning, governance, methodological, and infrastructure requirements that help prevent scientifically sound RWE programs from becoming commercially ineffective.

Exhibit 9: Executive readiness checklist

Readiness question	What good looks like
Is there one integrated evidence plan covering regulatory and access needs?	All major RWE activities map to one evidence strategy with designated owners and decision-linked milestones.
Is each planned study connected to a named access decision / strategic imperative and decision date?	Every major evidence activity supports a specific regulatory, payer, HTA, or contracting milestone — not a general publication objective.
Has landscape RWE started prior to pivotal trials (e.g., alongside Phase II or early Phase III)?	Pre-launch evidence generation is underway and actively informing positioning, endpoint strategy, and launch sequencing.
Are decision-grade evidence standards defined for each priority market?	Teams have explicit agreement on data quality thresholds, bias mitigation requirements, reporting standards, and submission timing for each key market.
Is the evidence infrastructure in place prior to Phase III?	Data partnerships, registry governance, provider workflow integration, and quality oversight are operational at launch, not in development at launch.
Is the evidence plan reviewed and updated on a defined cycle?	Evidence priorities are reassessed as policy environments, competitive dynamics, and payer evidence expectations evolve.
How is RWE input supporting lifecycle strategy?	Program leads across related assets and lifecycle management have visibility to RWE goals and infrastructure decisions.

Call to Action:

To turn RWE into access impact, we call on leaders across Market Access, HEOR, Commercial, and Medical Affairs to build RWE into access strategy.

Priority 1:
Integrate Evidence
Planning Now

BUILD RWE INTO DEVELOPMENT STRATEGY

Evidence planning aligned with payer and HTA requirements should begin in Phase II planning. Endpoint selection, patient-reported outcome specification, real-world data partnerships, and study design choices can shape pivotal development and future access in ways that are difficult to correct later.

Strategic Question

"How do we build the evidence foundation needed to establish burden, unmet need, and differentiated value before the market is defined without us?"

Priority 2:
Anticipate
Policy-Driven
Evidence Needs

SCENARIO PLAN PROACTIVELY

Every major commercial decision carries an access policy dimension. MFN, IRA negotiation timelines, orphan exemption structures, coverage with evidence development requirements, and HTA joint clinical assessment frameworks all create strategic inflections with evidence implications that should be addressed before launch.

Strategic Question

"How do we engage payers, HTA bodies, and policy stakeholders early enough to shape evidence expectations before access decisions are framed without our input?"

Priority 3:
Prepare for
Continuous
Value Defense

DEMONSTRATE ONGOING VALUE

Sustained reimbursement requires evolving evidence. Post-launch RWE programs that demonstrate long-term clinical and economic outcomes, support outcomes-based contract performance, and provide evidence for lifecycle expansion are now core commercial capabilities.

Strategic Question

"Are we continuously generating and communicating comparative value in ways that protect price, preserve access, and support future growth?"

Summary

RWE planning that begins in clinical development creates a stronger launch position. By approval, disease burden can be characterized, the care pathway mapped, and data infrastructure prepared to generate real-world outcomes from day one of commercialization. That foundation does not appear at launch; it must be built years before approval.

Turning RWE into access impact requires one integrated evidence plan across HEOR, Market Access, Medical Affairs, Marketing, and Commercial. The plan should connect evidence activities to shared objectives, decision milestones, and access timelines. It should run backward from formulary reviews, HTA submissions, and managed access renewals, not forward from the research team’s preferred schedule. When a payer raises a question, the team should already know what evidence exists, where it sits, and how it will be used.

The access environment is becoming less forgiving. Payer scrutiny is rising, HTA expectations are tightening, and the window between regulatory approval and commercial traction is narrowing. RWE cannot remain a publication exercise or a post-launch contingency plan. The commercial advantage will belong to teams that build RWE before the market asks for it.



Appendix

RWE REFERENCE GUIDE: KEY TERMS AND CONCEPTS

This guide is designed to be used independently of the paper, as a reference for teams working across evidence planning, payer submissions, and HTA dossier development. Definitions are written for functional leaders who work with evidence strategy but may not have a formal research methods background.

FOUNDATIONAL CONCEPTS

Term	Definition
Real-World Data (RWD)	Patient health and care delivery data collected outside the controlled conditions of a clinical trial. Sources include electronic health records, medical claims, disease registries, and patient-generated data from digital devices. RWD reflects what happens in routine clinical practice rather than in a protocol-defined study population.
Real-World Evidence (RWE)	Clinical evidence derived from analysis of real-world data. RWD is the input; RWE is the analyzed output. The same dataset can produce different RWE depending on the study design, the question, and the analytical methods applied. Not all RWD analyses meet the threshold required to support a coverage or reimbursement decision.
Landscape RWE	Evidence generated before product launch to characterize the disease environment a therapy will enter. Landscape RWE covers disease burden, patient epidemiology, care pathway gaps, treatment patterns, and provider behavior. It draws on existing data sources and is available to any team regardless of approval status. Most organizations underinvest here.
Product RWE	Evidence generated after product launch to measure how an approved therapy performs in routine clinical practice. Covers utilization, adherence, persistence, comparative effectiveness, total cost of care, and safety in broader populations than pivotal trials enrolled. Infrastructure for product RWE (data partnerships, registries, outcome capture) must be in place before launch.
Decision-Grade Evidence	Evidence that meets the fitness-for-purpose, methodological, and reporting standards required for a specific coverage, reimbursement, or regulatory decision. A rigorous study that answers the wrong question, covers the wrong population, or arrives after the review has concluded does not qualify. Decision-grade evidence requires fit-for-purpose data, a pre-specified protocol and analysis plan, explicit bias mitigation, transparent reporting, and timing aligned with the decision.

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Best Practice Resources

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