Generation and Utilization of Real-World Evidence by US Payer Organization Stakeholders to Support Formulary Decisions

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Introduction

The capabilities of real-world evidence (RWE) continue to expand enabling the support of a wide range of needs. This RWE may span from understanding disease burden in specific populations or therapeutic areas, to real-world treatment patterns, to comparative effectiveness and complementing data from clinical trials. The acceptance and role of RWE is also rapidly evolving, with broader use in US regulatory and reimbursement environments. As the application of RWE continues to broaden, US commercial payers' ability to generate and consume these data for formulary decision-making is beginning to progress

Objective

The objective of this primary research study was to obtain perspectives on the generation and use of RWE from diverse US payer organizations.

Methods

In May 2024, we recruited experienced stakeholders from US payer organizations via our Petauri Payer Network, inviting them to participate in an online quantitative and qualitative survey. Inclusion criteria for the survey included: Currently based in US, current or former US payer, at least 5 years of experience as payer or actuary, and a current or former voting member or participant on their organizations' Pharmacy and Therapeutics (P&T) committee. Within the survey, we explored 12 key themes, consisting of 53 questions. We conducted descriptive statistics and contextual analyses. Participants were provided with an honorarium for participation in the 30-minute survey based on fair market value.

Results

The survey included 20 participants (4 medical directors, 11 pharmacy directors, 4 industry/trade relations professionals, and 1 actuary), who represented national and regional Managed Care Organizations (MCOs), Pharmacy Benefit Managers (PBMs), and Integrated Delivery Networks (IDNs). Overall, 80% of participants reported 15 or more years at payer organizations, with 75% currently in role. Most (87%) of the pharmacy and medical directors were voting members in their organization's P&T committee, with the remaining 13% serving as non-voting P&T members.

Most payers (80%) reported using RWE from their own institution to inform treatment coverage decisions. Looking at the reasons for utilizing their own institution's RWE (Figure 1), the most common uses were to evaluate current drug utilization (80%), analyze current treatment patterns (70%), track healthcare resource utilization (60%), assess current economic burden (60%), and assess clinical disease burden (45%).

Figure 2 summarizes the value that payers place on internal analyses of cost burden by therapeutic area. The highest weighted ratings (very or extremely valuable) for cost burden analyses were noted for diabetes (weighted average: 4.19/5.0), cardiovascular disease (3.94), obesity (3.73), and oncology (3.73). This clearly reflects payer focus on high prevalence, high expenditure areas. Therapy areas for which internal cost burden analyses are of notably lower value included digital therapeutics (weighted average, 2.50/5.0), diagnostics (2.85), and infectious disease (3.0). These likely capture payers' inability to directly manage some therapeutic areas, and relatively low budget impact of digital therapeutics and diagnostics.

Whilst payers placed a high value on in-house RWE across several use cases, their perceptions of the utility of RWE provided by manufacturers varied substantially across uses (Figure 3). Areas of RWE from manufacturers with the highest perceived value were identified as real-world post-launch clinical effectiveness (weighted average: 3.50/5.00), real-world post-launch clinical safety (3.40), real-world post-launch clinical effectiveness (3.35), and real-world post-launch economic impact of specific therapeutic (3.35).

Post-launch RWE is clearly important to payers, as nearly two-thirds (65%) of payers stated that post-launch RWE has had an impact on product coverage and formulary changes in their health plan (Figure 4). Examples of this impact included more favorable coverage when positive impacts on healthcare resource utilization could be demonstrated.

US payers have conventionally placed greater value on clinical trials data versus RWE when considering expanding coverage to additional patient populations. Our results do not overturn that conventional wisdom, but do suggest a potential moderation of views. When asked about their receptiveness to using RWE, in order to support label extensions following initial Food and Drug Administration (FDA) approval, most payers were slightly or moderately supportive (75%) of this, but only 10% were extremely or very supportive (Figure 5). A common reason cited for this continued hesitancy was that RWE comes with limitations and can be a great supplement to clinical trial data, but cannot replace these data entirely. Others took a more nuanced view, noting that the value of RWE here depends on the disease state, evidence collected, and type of label extension.

Figure 1: Use of RWE from institution to inform coverage decisions

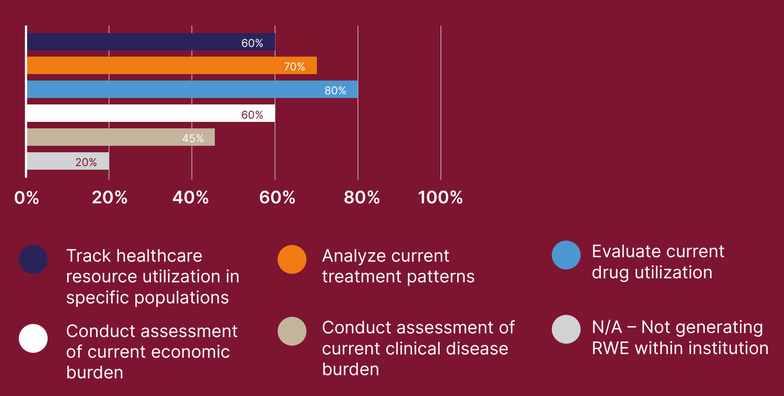


Figure 3: Perceived value of RWE from manufacturers

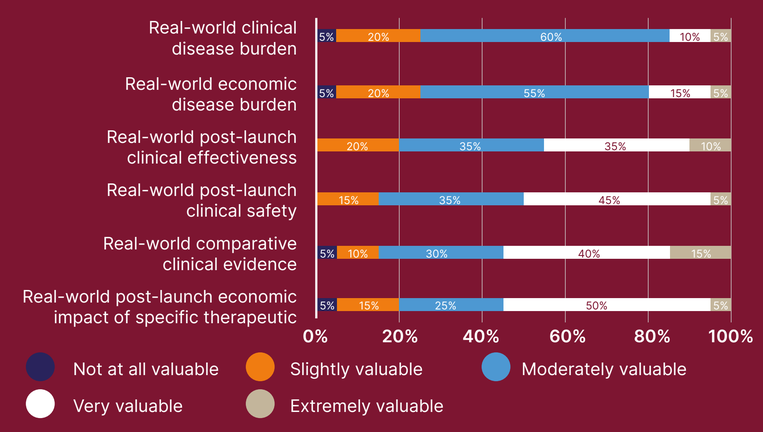


Figure 2: Weighted average of in-house cost-burden analyses in terms of therapeutic area

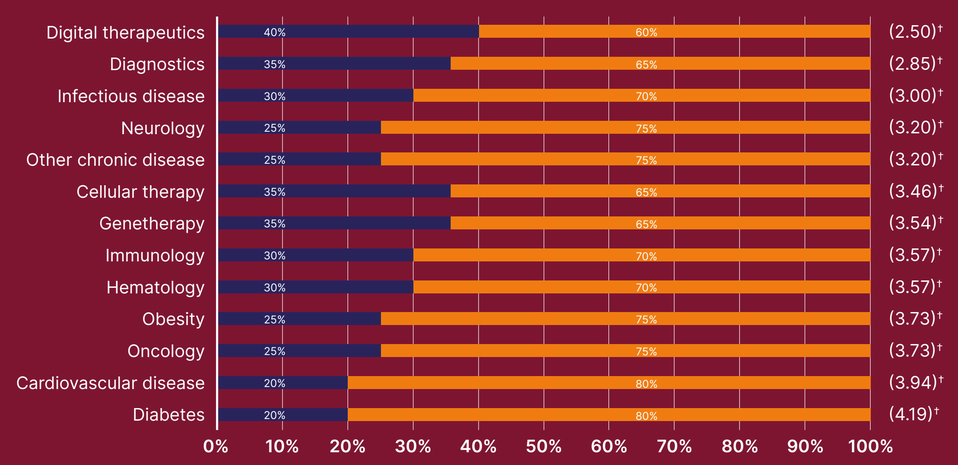


Figure 4: Impact of post-launch RWE on formulary changes

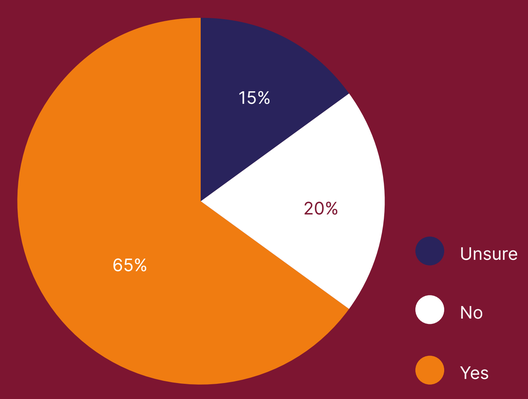
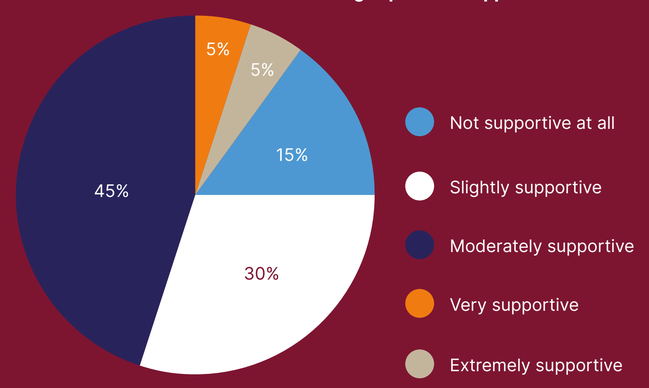


Figure 5: Receptiveness of using RWE, rather than clinical trial evidence, to substantiate label extensions or changes post-FDA approval



Conclusion

The role and influence of RWE in formulary decision-making continues to evolve in the US. Whilst clinical trial evidence remains the gold standard for decision-making at launch, RWE offers strategic advantages for payers and other stakeholders in numerous scenarios, enhancing and supporting the formulary decision-making process. There is a growing interest in the use of RWE to understand real-world clinical effectiveness/safety and other real-world outcomes post-launch.

From a value evidence perspective, launch is just the start of investment in RWE, as it drives patient access and commercial success. Payer evidence needs must be vetted and planned pre-launch, so that deep data sources from providers and health systems can be put in place to avoid potential delays with administrative claims datasets. With the availability and application of RWE rapidly expanding in the US, payers and manufacturers alike must work together to align on RWE needs, with a goal of improving patient outcomes.

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Abbreviations

CVD: Cardiovascular Disease
FDA: Food and Drug Administration
IDN: Integrated Delivery Networks
MCO: Managed Care Organizations
PBM: Pharmacy Benefit Managers
P&T: Pharmacy and Therapeutics
RWE: Real-World Evidence