The Role and Importance of Evidence in US Payer Assessment and Formulary Decisions

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Introduction

Payer evidence preferences in the US continue to evolve with an evolving biopharmaceutical industry. In the US, there is no formal health technology assessment (HTA) or systematic set of requirements for reimbursement and coverage determinations unlike many other markets around the world. As such, insights around evidence needs and preferences for US payers need to be uncovered on an ongoing basis through payer research and insight generation. Understanding payer preferences for and importance placed on evidence in therapeutic assessment is critical to ensure generation of meaningful and impactful evidence that supports the formulary decision-making process.

Objective

The objective of this primary research study was to obtain perspectives around the role and importance of evidence in therapeutic assessment to inform the formulary decisionmaking process by diverse US payer organization stakeholders.



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Methods

In May 2024 we recruited experienced stakeholders from US payer organizations via our Petauri Payer Network 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 organization(s) 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 an honorarium for participation in the 30-minute survey based on fair market value.

Results

Figure 1: Evidence to support product agnostic disease burden

Figure 3: Importance of H2H clinical evidence for a new therapy in select therapeutic areas

Rare disease space

The survey included 20 participants, including 4 medical directors, 11 pharmacy directors, 4 industry/trade relations professionals, and 1 actuary. Participants 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% of participants were 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.

Within the survey, US payer evidence needs and preferences and the sources of information used to address these evidence needs were explored. Regarding the evaluation of product disease burden in preparation for formulary decisions, the most frequent sources used by payers were published literature reviews (weighted average: 4.25/5.0) and RWE from their organization (4.0) (Figure 1).

For the evaluation of product-specific clinical evidence, the most frequent sources were peer reviewed literature (weighted average: 4.55/5.0), published clinical trials (4.45), professional guidelines (4.20), and ICER reviews (3.90) (Figure 2). While manufacturer-provided materials were not the most frequently used sources for productspecific clinical evidence (weighted average: 3.65), 60% of payers frequently or always use manufacturer provided evidence and only 5% stated they never use manufacturer-provided evidence.

Looking at specific preferences for clinical evidence, it is well established that payers highly value head-tohead clinical evidence if/when appropriate based on current treatment and standard of care in a therapeutic area. More specifically, payers perceived head-tohead clinical trials as the most important in competitive disease states with many therapeutic options (weighted average: 4.55/5.0) and for a new mechanism of action that challenges the standard of care (4.20) (Figure 3). When head-to-head clinical trial evidence is not available, payers and other stakeholders often look to other methods to assess comparative clinical evidence. The most common method cited was payers conducting their own informal indirect comparison, with 75% of payers surveyed using this method. Other common methods utilized included conducting their own network meta-analysis or other indirect comparison (40%), using manufacturer provided network meta-analysis or other indirect comparison methods (35%) (Figure 4).



Figure 2: Sources of data/information for product-specific clinical evidence



with no existing	15%		30%	15%	15%	25%	25%	
therapeutic options								
Rare disease space								
with existing	15%	5% 20%		35%		30%		
therapeutic options								
First therapeutic	100/	20%		25%	10%			
ption in therapeutic area	10%	20%		33%	10%	25%		
New MoA that								
challenges standard of	15%		50%			35%		
care guidelines								
New MoA in								
somewhat crowded	25%	;		50%		25%		
therapeutic area								
·								
Competitive disease								
states with many	15%	15%			70%			
therapeutic options	1070	1370						
	0%	20%	4() %	60%	80%	100%	
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Figure 5: Sources of data/information to determine product or class-specific economic impact



Of evidence used to evaluate economic burden, the most frequent sources were ICER reports (weighted average: 3.60/5.0), own institution-built economic models (3.55), and AMCP dossiers (3.25) (Figure 5).

Overall, payers surveyed perceived the most important sources of evidence for support of formulary decisions to be clinical guidelines (weighted average: 4.45/5.0), peerreviewed publications (4.20), and internal claims data (3.60) (Figure 6).





Figure 6: Importance of evidence in supporting formulary decisions



Conclusion

As shown by the results of this payer study, there are a wide range of sources that US payers leverage to inform and support formulary decisions. While some of these evidence types are preferred, US payers find some level of utility for most evidence types and sources. Payers had a strong preference toward published evidence whenever possible to support the formulary decision making, including peer-

reviewed literature and clinical guidelines. Payers also shared insights that they tend to favor their own internal data, analyses, and models whenever possible, but this does not discount the importance and impact of manufacturer-provided evidence. Despite varied perspectives around specific evidence types, there was a consistent preference toward scientifically robust and transparent evidence.





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Abbreviations

AMCP: Academy of Managed Care Pharmacy H2H: Head-to-head HTA: Health technology assessment ICER: Institute for Clinical and Economic Review IDN: Integrated Delivery Networks MCO: Managed Care Organizations MoA: Mechanism of Action P&T: Pharmacy and Therapeutics PBM: Pharmacy Benefit Managers RWE: Real-world evidence