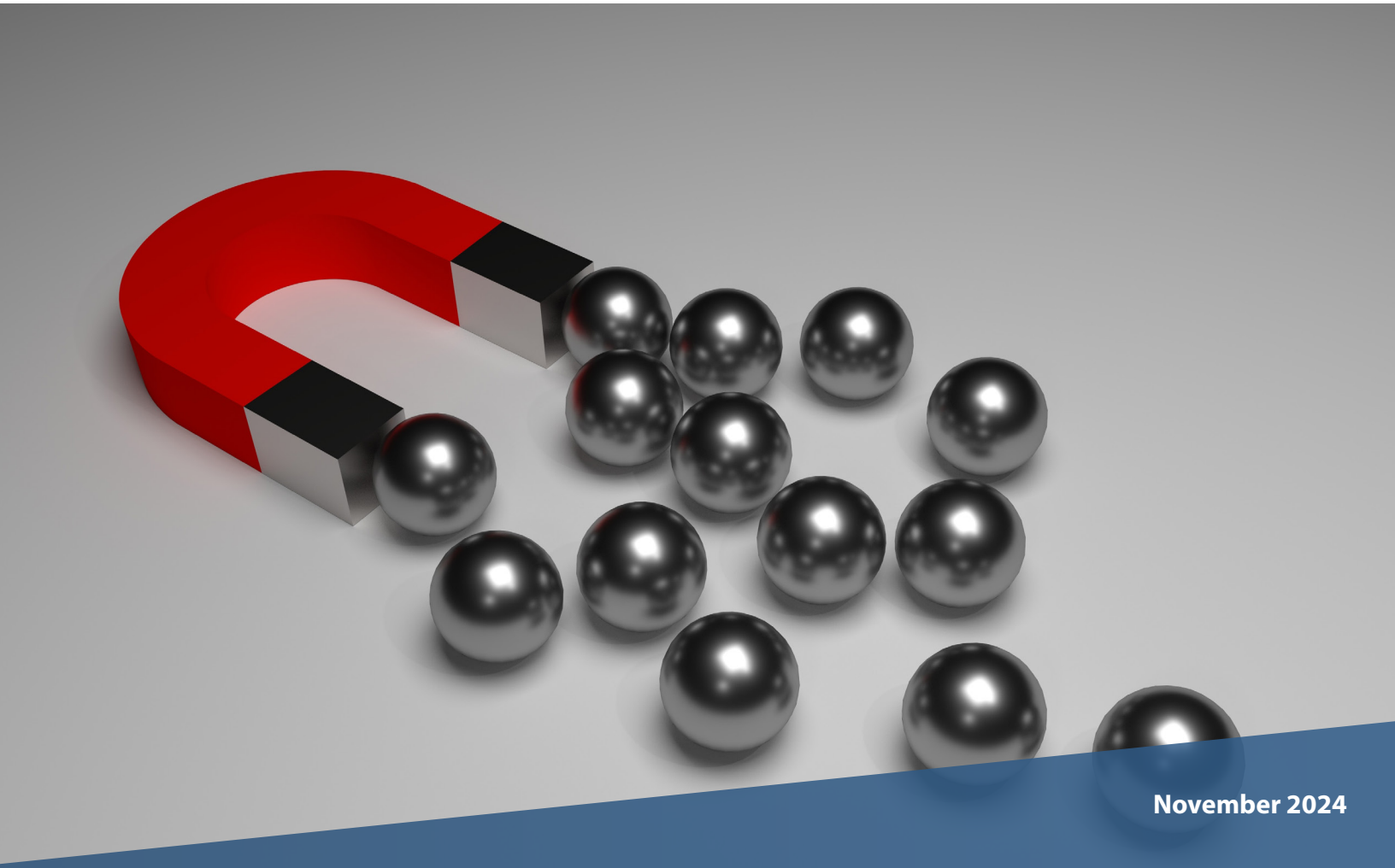
TRINITY

ADVISORY BRIEF

Assessing the Influence of ICER Reports on U.S. Payer Decision Making

Mary Fletcher-Louis • Sam Feng • Nandini Hadker • Sophie Doran • Andrew Gould

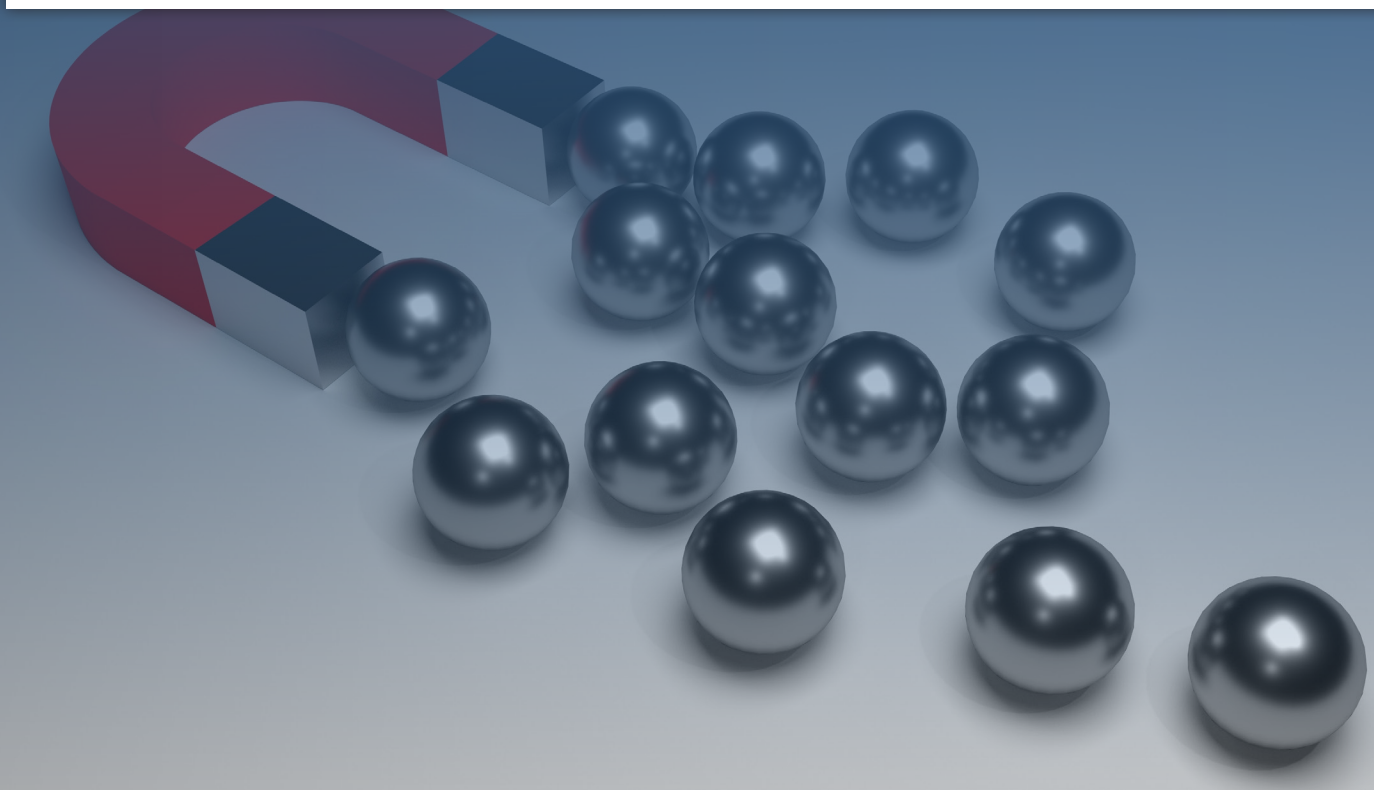


November 2024

Introduction

The Institute for Clinical and Economic Review (ICER) is an independent, non-profit research organization dedicated to assessing the value of healthcare interventions, including diagnostics, devices, digital therapeutics and prescription drugs. Although ICER's assessments do not represent a mandate for product funding or pricing in the U.S. in the same way as, for example, National Institute for Health and Care Excellence (NICE) assessments in the U.K., public reports on a product's value are bound to influence discussions and negotiations between payers and manufacturers. However, the extent to which payers consider ICER assessments in their evaluation of therapies remains unclear.

To address this uncertainty, Trinity Life Sciences conducted a payer survey to better understand how U.S. payers really use ICER assessments. The goal was to shine a light on the impact ICER assessments and other third-party economic assessments truly have on the formulary decision making process and perceptions of price. In this advisory brief, we share key findings that emerged from our research to provide guidance for life sciences manufacturers.



Background

Based on ICER's Value Assessment Framework (VAF),[1] assessments involve a review of all available evidence, comparative clinical effectiveness analyses, long-term effectiveness analyses, economic analyses and consultation with patients and clinical experts. For each intervention assessed, ICER publishes:

- A rating of the comparative net health benefit of the intervention (ranging from negative net benefit to substantial net benefit)
- A cost-effectiveness evaluation
- An evaluation of potential other benefits/disadvantages (including health equity)
- ICER's view of a "Health Benefit to Price Benchmark" (HBPB) (defined as the price range that would achieve incremental cost-effectiveness ratios between $100,0000 and $150,000 per quality-adjusted life year (QALY) or equal value of life years (evLY) gained*
- The probability of cost effectiveness at thresholds ranging from $50,000 per QALY/evLY and $200,000 per QALY/evLY

Since its inception in 2006, ICER has published up to 12 reviews each year.[2] Of the 10 reviews conducted in 2023 and up to September 2024 (Table 1, see next page) in which an HBPB was published,† four assessments provided a HBPB that broadly aligned with the annual wholesale acquisition cost (WAC) (the higher end of the range for the HBPB estimate was ≥100% of the annual WAC), suggesting that the product would represent value for money at the proposed price. For the remaining six assessments, the higher end of the range for the HBPB estimate ranged from 9 to 80% of the annual WAC, suggesting that the product would not represent value for money at the proposed price.

As ICER has grown in prominence, pharmaceutical manufacturers, patient advocacy groups and physicians have raised concern about its methods of assessment, specifically the use of cost-effectiveness analysis and QALYs.[3] Similar methods have long been used by some countries' health technology assessment (HTA) bodies (e.g., NICE in the UK) as QALYs assume that health improvement is equally valued between individuals, thereby allowing comparison across disease areas to support resource allocation.[4] However, cost effectiveness and QALYs (and similar metrics) may miss some of the elements of value that matter to patients and society.[3,5] Furthermore, there is concern that the use of these metrics discriminates against older adults, those with disabilities and those with rare or more complex diseases, for whom treatments are less likely to be deemed cost effective compared with treatments for younger individuals in good health.[6]

* QALY: A quality-adjusted life year is a metric that measures the value of health outcomes by combining length of life and quality of life into a single number; evLYG: equal value of life years gained is a metric used to measure the value of a medical treatment's ability to extend a patient's life.

† An additional report on post-traumatic stress disorder was published in 2024 but HBPBs were not provided as the economic analysis was exploratory due to insufficient evidence availability.

Table 1: ICER Recent Summaries

Indication	Publication Date	Annual WAC	ICER HBPB	HBPB % of WAC
Anemia in myelodysplastic syndrome	July 2024	$365,197	$94,800 to $113,000	26 – 30%
Chronic obstructive pulmonary disorder	June 2024	$35,400	$7,500 to $12,700	21 – 36%
Paroxysmal nocturnal hemoglobinuria*	March 2024	Iptacopan (with C5 inhibitor as a comparator): $550,377 per year	$178,000 to $180,000	32 – 33%
		Danicopan + C5 Inhibitors (with C5 inhibitor as a comparator): Placeholder price: $150,000 per year	$12,300 to $13,100	8 – 9%
Schizophrenia	February 2024	Placeholder price†: $20,000	$16,000 to $20,000	80 – 100%
Pulmonary arterial hypertension	January 2024	Placeholder price†: $400,000	$17,900 to $35,400	5 – 9%
Metachromatic leukodystrophy	October 2023	Placeholder Price†: $2,800,240	$2.3M to $3.9M	80 – 140%
Sickle cell disease	August 2023	Placeholder price†: $2M	$1.35M to $2.05M	67 – 100%
Non-alcoholic steatohepatitis	May 2023	$19,011 or $85,111	$32,600 to $40,400	40 – 171%
Alzheimer's disease	April 2023	$26,500	$8,900 to $21,500	34 – 80%
Multiple sclerosis	February 2023	$59,000 to $102,128‡	$16,500 to $34,900	28 – 35%

Green rows indicate products for which ICER-reported HBPB broadly aligned with the annual WAC (the higher end of the range for the HBPB estimate was ≥100% of the annual WAC), indicating that they would provide value for money at the proposed price.

Abbreviations: FSS, federal supply schedule; HBPB, Health Benefit to Price Benchmark; ICER, Institute for Clinical and Economic Review; SSR, suggested sales reimbursement; WAC, wholesale acquisition cost.

* Treatment-experienced patients on a Stable C5 Inhibitor Regimen with Clinically Significant EVH.

† Placeholder price: In cases where the WAC and FSS or SSR net prices are not available, ICER searches for and uses an average of investor analysts' opinions on launch price, if available. If no estimates of launch price are available and there are other drugs in the same class with similar characteristics, ICER uses the average price for the class as a placeholder for launch price.

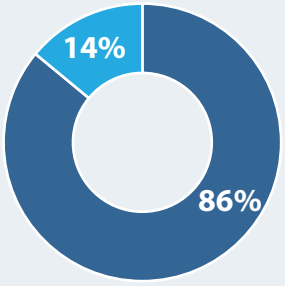
‡ These threshold prices do not include any provider-administered mark-up, which was assumed to be 6% in the cost-effectiveness model used to generate these estimates, where applicable.

Research Methodology

Based on our curated panel of U.S. payers, Trinity Life Sciences surveyed 20 U.S. formulary decision makers. Participants were required to be involved in pharmacy and therapeutics (P&T) decision making for a U.S. managed care organization (MCO), integrated delivery network (IDN) or pharmacy benefit manager (PBM) covering at least 10 million lives. The sample of MCOs included a mix of regional and national plans and provided a geographic mix. Participants were required to hold the title of Pharmacy Director or Medical Director and be involved in formulary decision making. The screening criteria were designed to include a wide variety of payer organizations, ensuring the results are applicable and generalizable across different types of payers.

Payer Demographics

Organization Type		Lives Covered by Organization Type (in the U.S.)
Large National Plan/Managed Care Organization (MCO)	40%	136M
Regional Plan/Managed Care Organization (MCO)	40%	31M
Integrated Delivery Network (IDN)	10%	11M
Pharmacy Benefits Manager (PBM)	10%	32M



Role

- Pharmacy Director – **86%**
- Medical Director – **14%**

Key Findings

Key Finding #1: Most U.S. payers find ICER reports extremely or very helpful for making formulary coverage and access decisions

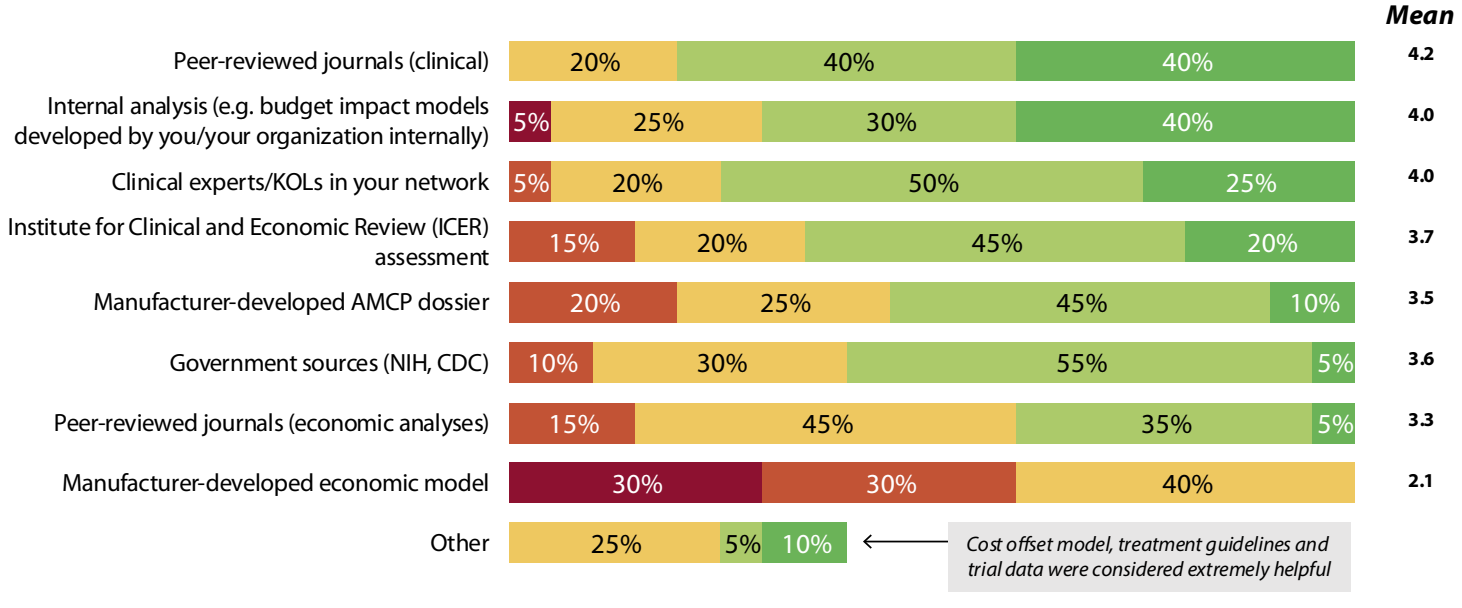
When making formulary coverage and access decisions, ICER reports are considered extremely or very helpful by the majority (65%) of U.S. payers, and somewhat helpful by a further 20% of payers. As may be expected, the most highly valued external evidence sources for making formulary coverage and access decisions were those from peer-reviewed journals with a clinical focus (80% of payers considered them extremely or very helpful) and clinical experts/key opinion leaders in the disease area (70% of payers considered them extremely or very helpful).

Internal (payer-generated) economic analyses were also considered extremely or very helpful by 70% of payers, in contrast to manufacturer-developed economic models, which were not considered extremely or very helpful by any payers and were considered not helpful at all by almost one third (30%) of payers. This presents an opportunity for manufacturers to consider how they can develop models that are more useful to payers. Qualitative analysis of payer responses reveals that models that build comparative clinical data into the economic model are considered more useful than those based on placebo-controlled trials.

Below we showcase the data in two ways: distribution by respondents (% of surveyed payers) and by covered lives to account for plan size.

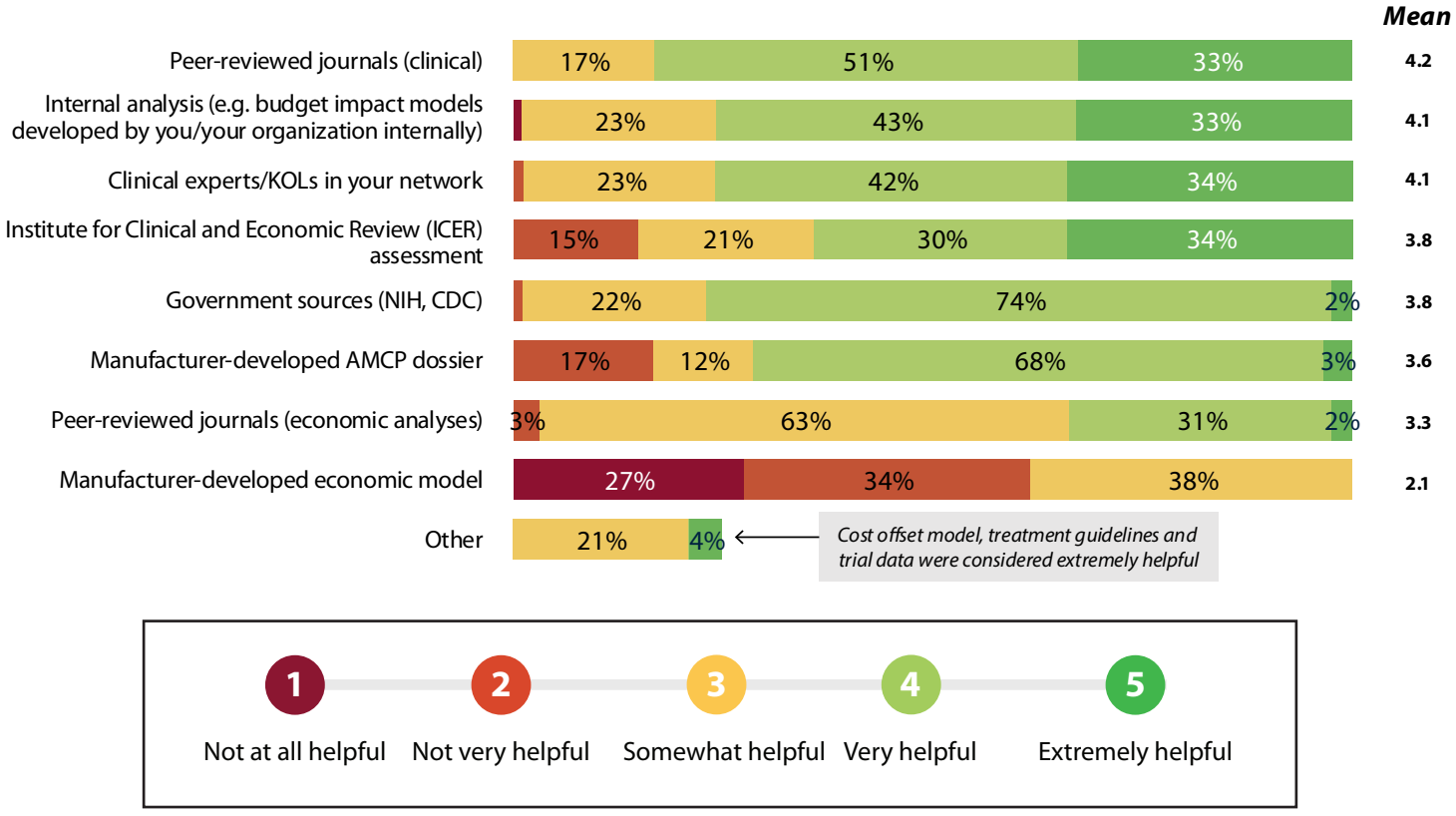
U.S. Payer Evaluation of the Value of Various Evidence Sources in Making Formulary Coverage and Access Decisions

% of Surveyed U.S. Payers (n=20)



U.S. Payer Evaluation of the Value of Various Evidence Sources in Making Formulary Coverage and Access Decisions

% of Surveyed U.S. Payers, Weighted by Covered Lives (n=20)



Key Finding #2: Over half of U.S. payers frequently use ICER assessments in their formulary decision making

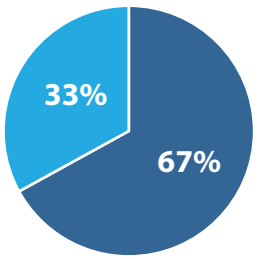
ICER assessments have gained substantial traction among U.S. formulary decision makers since the first ICER reports were published in 2007. All respondents report at least some familiarity with ICER assessments, with 67% stating they are very familiar. Furthermore, 56% of decision makers frequently use ICER assessments in their formulary decision-making processes, and 17% sometimes do so. These data suggest that ICER has established itself as a critical resource for payers, underscoring its growing influence in shaping coverage and access decisions.

Familiarity & Frequency of use of ICER Assessments by U.S. Formulary Decision Makers

% of Surveyed U.S. Payers, Weighted by Covered Lives (n=20)

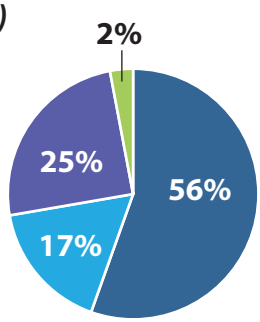
How familiar are you with ICER Assessments?

- Very – **67%**
- Somewhat – **33%**
- Not at all – **0%**



How frequently do you use ICER assessments in your formulary decision making?

- Frequently – **56%**
- Sometimes – **17%**
- Rarely – **25%**
- Never – **2%**

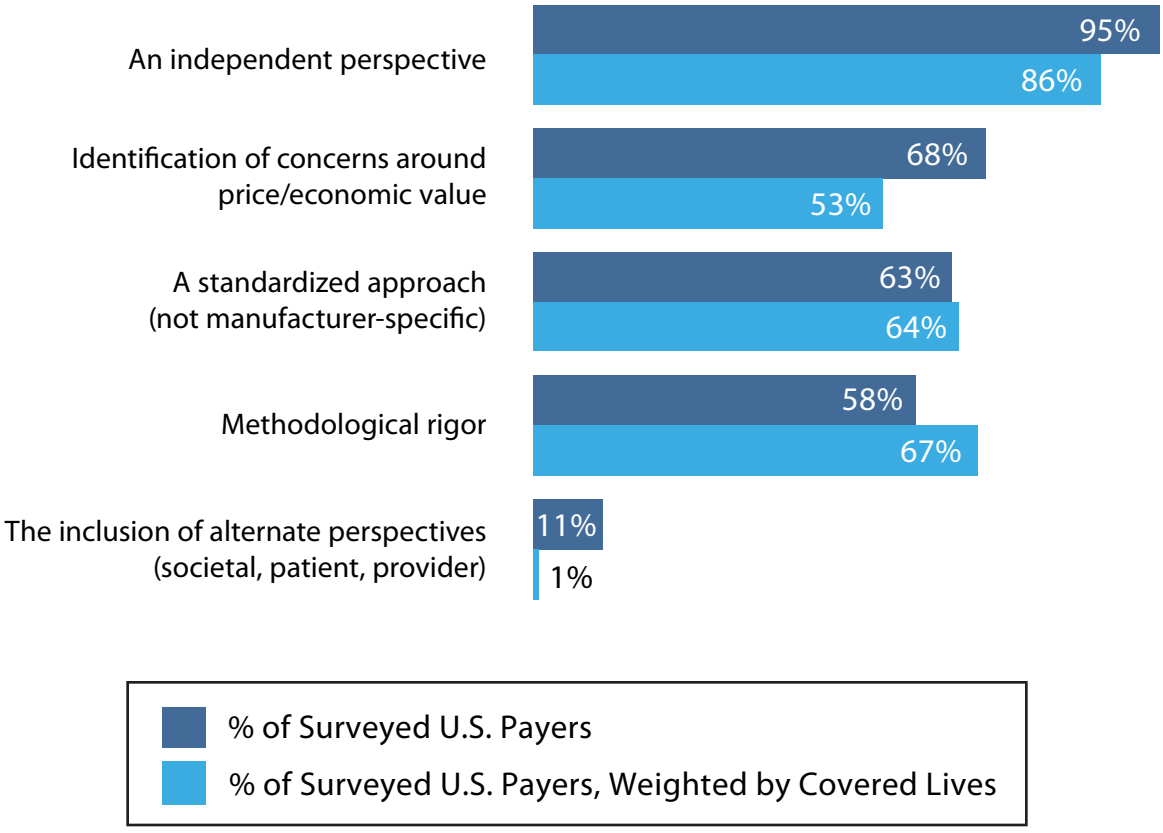


Key Finding #3: U.S. payers value ICER assessments primarily for providing an "independent perspective"

Surveyed U.S. payers value ICER assessments primarily because they see them as providing an independent perspective , with 95% of U.S. formulary decision makers recognizing this as a key element of value. Additionally, 68% appreciate ICER's role in identifying concerns around price and economic value. A further 63% find value in ICER's standardized approach. Only 11% of respondents value the inclusion of alternative perspectives such as societal, patient or provider views, indicating that these are not yet top of mind for U.S. payers.

ICER Assessments – Elements of Value for U.S. Formulary Decision Makers

(n=19)



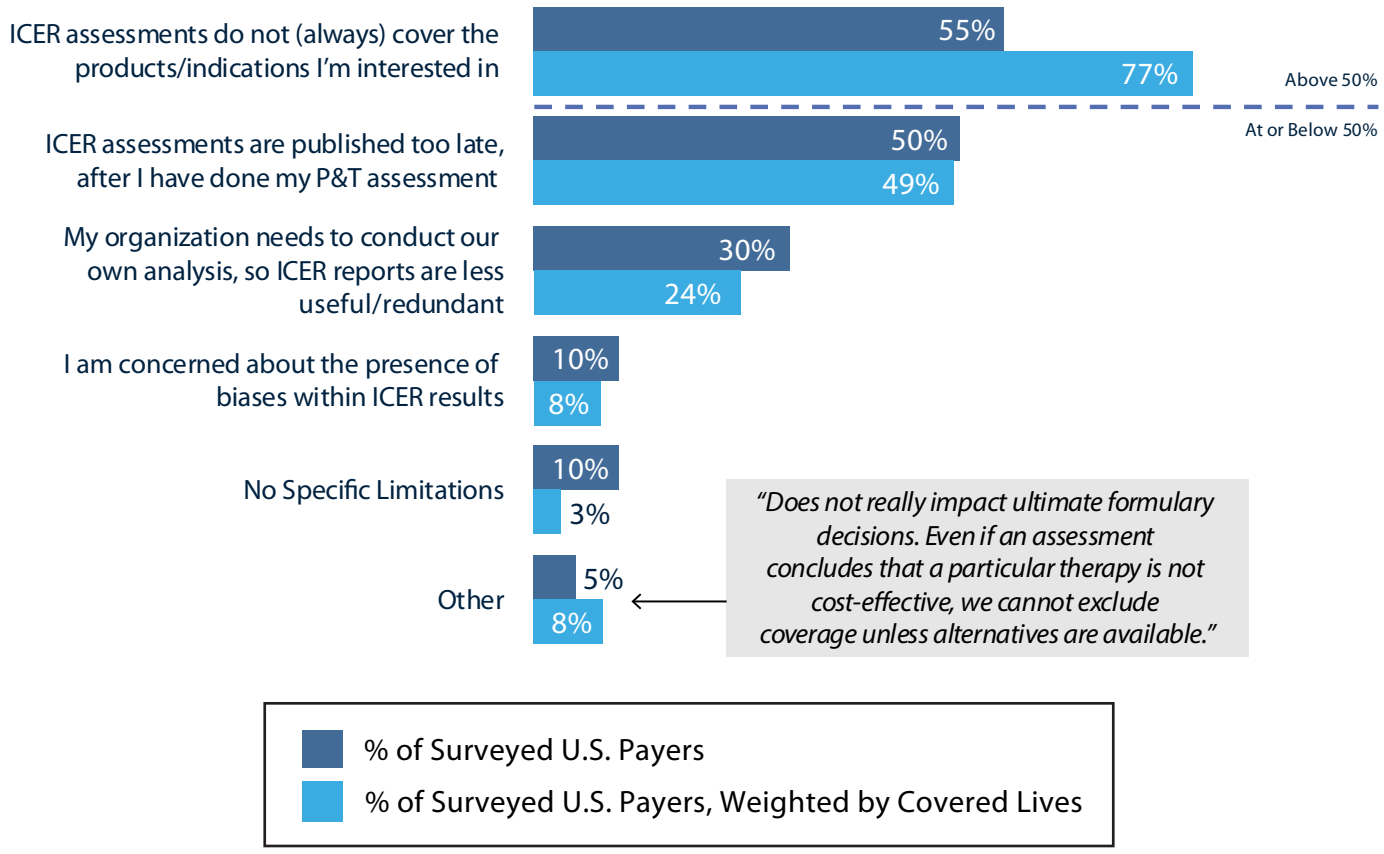
Key Finding #4: The value of ICER reports is limited by lack of coverage and timing

Reflecting the fact that ICER has published only five to eight assessments per year in the last three years,* survey participants highlight the lack of coverage of certain products or indications (55%) and the timing of ICER assessments as key factors that limit their value. One half of respondents say that ICER assessments are often published too late to influence formulary decisions.

Additionally, 30% of respondents indicate that they need to conduct their own analyses, making ICER reports less useful or redundant. Concerns about bias within ICER results and the lack of alternative treatments, even if ICER concludes that a therapy is not cost effective, play minor roles in limiting the assessments' perceived value.

Factors that Limit the Value of ICER Assessments for U.S. Formulary Decision Makers

(n=20)



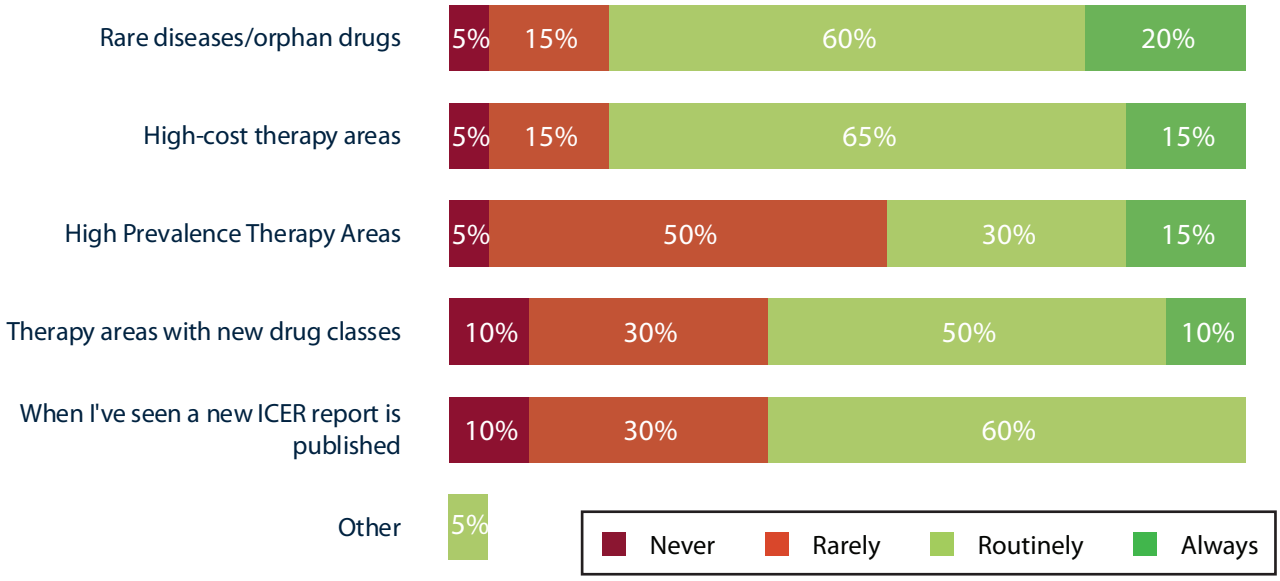
* As of September 2024, five reports had been published and one additional report was ongoing.

Key Finding #5: ICER assessments are most widely used in reference to orphan drugs and high-cost therapies

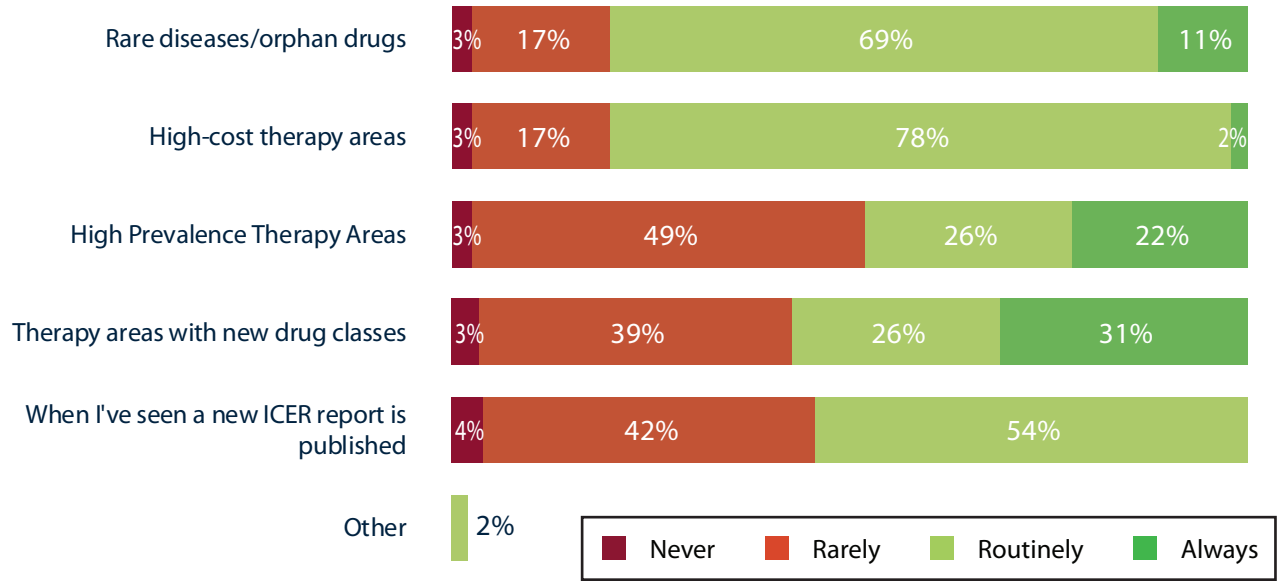
Our analysis highlights that ICER reports are widely utilized by payers, with 80% of respondents consulting them "always or routinely" for rare diseases/orphan drugs and for high-cost therapy areas, 60% consulting them "always or routinely" for therapy areas with new drug classes and 45% consulting them "always or routinely" for high prevalence therapy areas. In general, utilization of ICER reports is high, with 60% of payers surveyed referring to them when they see that a new report has been published.

Circumstances in Which U.S. Formulary Decision Makers Refer to ICER Assessments

% of Surveyed U.S. Payers (n=20)



% of Surveyed U.S. Payers, Weighted by Covered Lives (n=20)

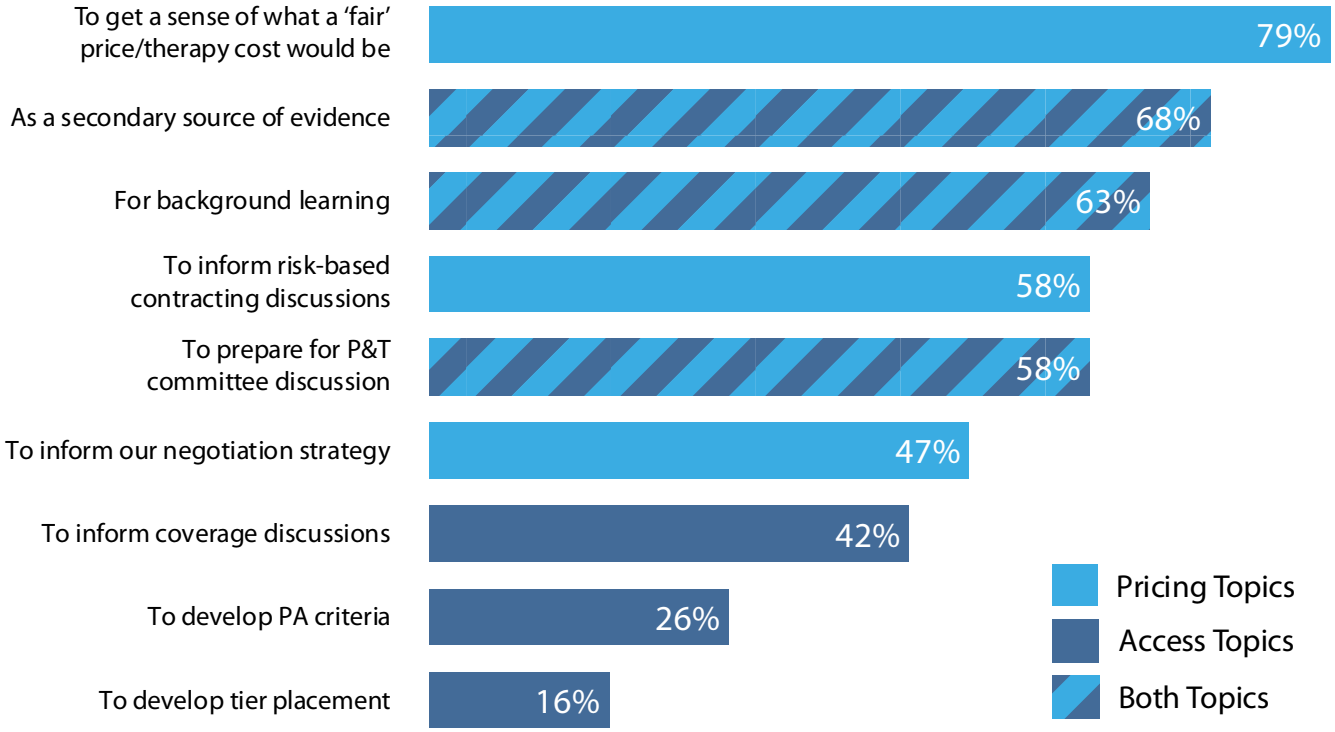


Key Finding #6: ICER assessments are most commonly used to understand "fair" pricing for a therapy

ICER reports are highly valued by payers for pricing-related topics, with nearly 80% of those surveyed using them to determine what a "fair" price for a therapy might be. They are also frequently used as secondary sources of evidence and for gaining background understanding. ICER reports are less frequently used for making standalone coverage decisions or developing prior authorization (PA) and tier placement guidance. Close to one half of respondents (47%) report using ICER assessments to inform negotiation strategy. When adjusted for covered lives, U.S. payers are slightly more inclined to use ICER reports for developing PA criteria (32% vs. 26% in the unadjusted data). However, they are slightly less likely to use these reports for informing negotiation strategy (35% vs. 47%) or for gauging a fair price (63% vs. 79%).

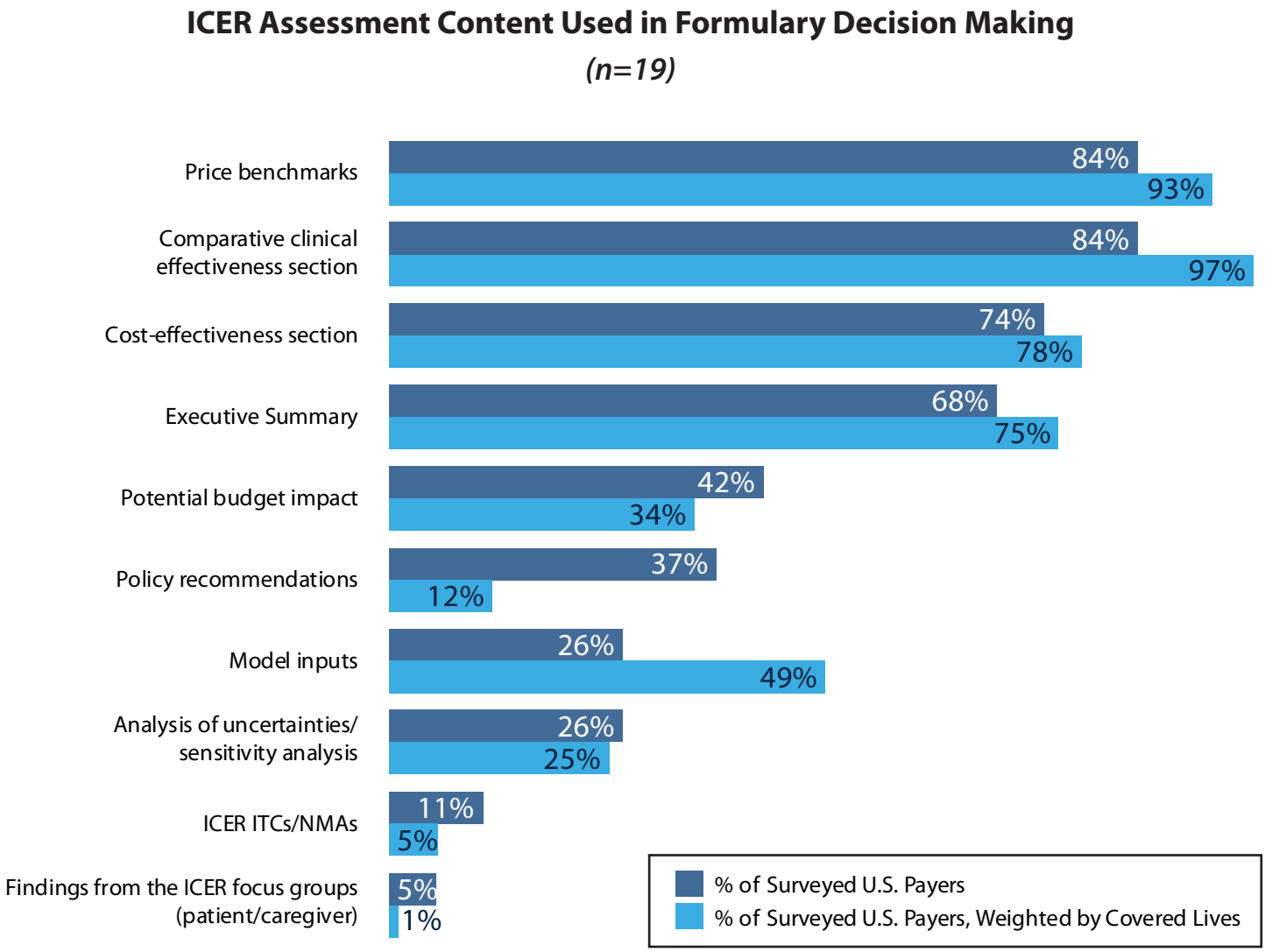
Use Cases for ICER Assessments by U.S. Formulary Decision Makers

% of Surveyed U.S. Payers (n=19)



Key Finding #7: Price benchmarks and comparative clinical effectiveness are the most commonly used content of ICER reports

When using ICER reports in their formulary decision making, payers predominantly utilize content related to price benchmarks and comparative clinical effectiveness, with 84% of payers surveyed using them in their decision making. The cost-effectiveness section and the executive summary are also highly utilized, by 74% and 68% of payers surveyed, respectively. Analyses of potential budget impact, policy recommendations and more detailed analyses (e.g., uncertainties, sensitivity analyses) are used by more than one quarter of surveyed U.S. payers.

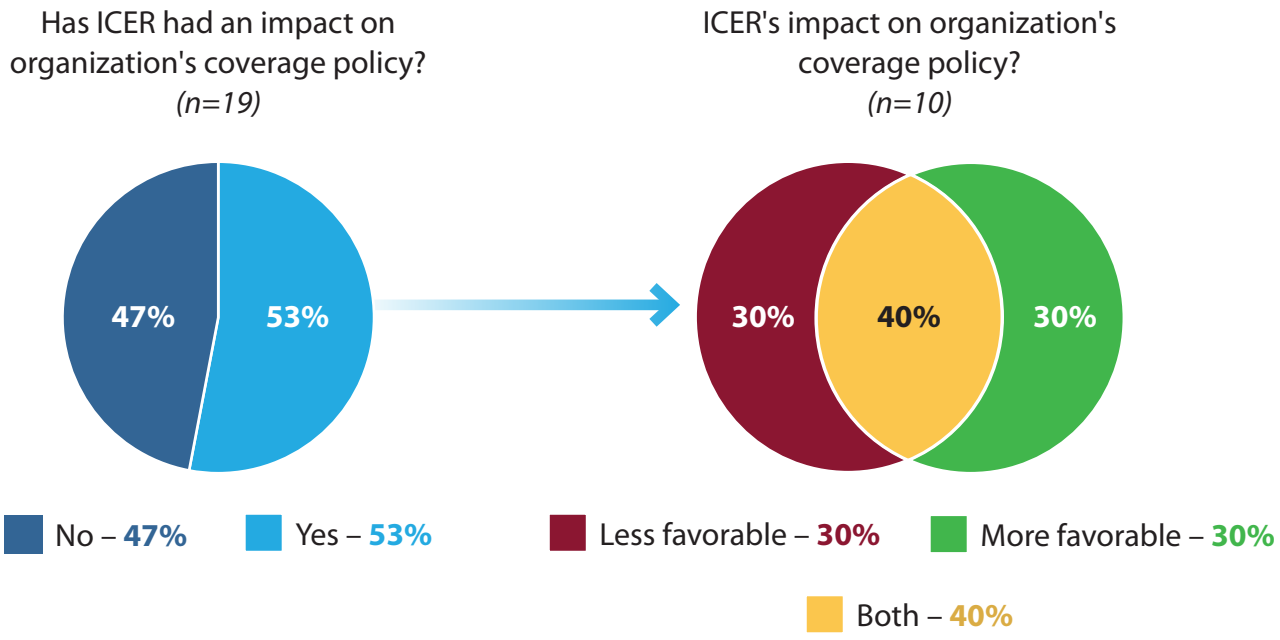


Key Finding #8: Over half of U.S. payers say that ICER reports have changed their formulary coverage decisions, either favorably or unfavorably, or both

More than half of surveyed U.S. payers say that ICER reports have changed their formulary coverage decisions. The influence of ICER assessments can swing both ways: 40% of these respondents say ICER reports have changed coverage decisions both positively and negatively, while 30% say ICER assessments have impacted coverage favorably, and another 30% say they have impacted coverage unfavorably. When the data are weighted by covered lives, surveyed U.S. payers are even more likely to say that ICER reports have had an impact on coverage policy; in this case, 57% of respondents report that they have done so.

Impact of ICER Assessments on Formulary Coverage Decisions

% of Surveyed U.S. Payers



Contrary to a commonly held industry perception, the HBPB that ICER provides is not always less than the manufacturer's WAC price. There are in fact instances where ICER's HBPB is aligned with the WAC. In three out of four of these recent cases (Table 1), the disease area is associated with lower socioeconomic status (schizophrenia, sickle cell disease and NASH).

Conclusion

Our survey finds that ICER assessments play a major role in how payers think about a therapy and the value it provides. The research finds broad utilization of ICER assessments by U.S. payers, reflecting their growing importance in the decision-making process for formulary coverage and contracting strategies. Most payers surveyed were familiar with ICER assessments, frequently using them for insights into what constitutes a "fair" price for new treatments, especially for orphan drugs and high-cost therapies. However, payers considered the value of ICER reports limited by the timeliness and range of assessments. Although ICER attempts to align reports with Food and Drug Administration (FDA) approval of a drug, assessments follow a standardized approach, including scoping, public consultation, modelling and reporting steps, and take approximately 8 months.[2] Furthermore, ICER has produced only five to eight reports per year in the last three years,[2] which means that many new launches have not had a corresponding ICER assessment.* Therefore, while ICER reports have a notable influence on payer strategies, particularly contracting negotiations, there remains an opportunity for ICER to address some of the barriers to broader and more consistent use.

There are concerns among stakeholders that ICER's methods for economic analysis do not adequately capture a therapy's holistic value and may even disadvantage some patient groups.[3,5,6] However, the findings of our survey suggest that payers value ICER and are paying attention to ICER assessments. As the healthcare landscape continues to evolve, ICER's role in shaping the economic value narrative for new therapies will likely grow, making it a critical tool for payers in balancing cost, access and value in their coverage decisions. There are opportunities for manufacturers to engage with ICER during the assessment process, ensuring that assessments reflect all available evidence and input.[7] Although some manufacturers may see engagement as legitimizing ICER's methods and findings, there is evidence to suggest a (non-significant) association between manufacturer engagement and improved cost-effectiveness ratios in both the draft and final ICER reports.[7]

Whether or not manufacturers choose to engage directly with ICER, it is increasingly important for them to prepare for potential downstream ICER reviews. It is key that manufacturers understand the types of analyses that ICER will likely conduct, the data and assumptions that will be used and the conclusions that may be drawn. This should be done as early as possible, ideally before a therapy is on ICER's list, since later, there is little time to respond—manufacturer evidence is due four weeks after it is formally requested and 13 weeks after topic selection. This planning and preparation will enable companies to maximize the value of a positive ICER assessment or to proactively and strategically mitigate any downstream consequences if the assessment is not positive, thereby protecting and optimizing the value of their new therapy.

At Trinity, we combine industry knowledge and experience with robust evidence strategy and payer-focused, evidence-based value communication. We can help our clients to prepare for and respond to external value assessments through evidence synthesis, economic modelling, dossier and response writing, payer value proposition and objection handler development, and mock negotiations.

* Relevant topics for ICER review are identified through public recommendation and independent analysis of the emerging drug pipeline. Interventions are prioritized based on criteria that include significant potential for improved patient outcomes, raising new questions about the comparative effectiveness of similar treatments, potential for significant financial impact, presentation of new opportunities to improve health outcomes and/or health system value through specific clinical or policy actions, being particularly relevant due to prevalence, severity, disparities, and cost, being likely to receive FDA approval within 1 year, potential over or underuse of treatments or tests, variation in approaches, potential to reduce health disparities, and potential to leverage current health reform initiatives.

Authors

Mary Fletcher-Louis | Managing Director & Head of Value Center of Excellence

Mary brings over 25 years of healthcare and consulting experience to Trinity. She has extensive experience in value strategy and in the development of value communication tools spanning diverse therapy areas. Mary has deep experience of the decision drivers of market access stakeholders across multiple countries and stakeholder types. Fostering a culture of innovation in all her endeavors, Mary is currently pioneering the integration of health equity into life science value strategy. As a senior thought leader in the industry, Mary has held leadership roles in various domains including global market access, HEOR, market forecasting and primary market research. For several years, Mary led DRG's Value Communication Center of Excellence. Her academic achievements include a master's in public health from Nottingham University and a BA from Oxford University.

Samantha Feng | Engagement Manager

Samantha has over 10 years of experience in the life sciences field, where she has led numerous engagements focused on portfolio commercial and P&MA strategies in the U.S. She brings extensive expertise in opportunity assessments, competitive landscaping, and lifecycle management strategy across multiple therapeutic areas. Sam holds a Ph.D. in Pharmacology and Toxicology from the University of California, Davis.

Nandini Hadker | Partner, Evidence Strategy

An economist by training, Nandini is a Partner at Trinity Life Sciences with over two decades of experience in custom research and strategy consulting. She is an expert at helping clients identify what real-world evidence will "move the needle" and drive their product's success, generating that evidence using publication-grade research, and pulling the insights through scientific dissemination. She is widely published in several highly rated journals as well as in industry forums.

Sophie Doran | Director, Evidence, Value, Access & Pricing

Sophie has over 10 years of experience in Value Communication. Her background is in specialist market access/health economics and outcomes research (HEOR) medical writing, developing high-quality materials, including (but not limited to) value stories, value and reimbursement dossiers, objection handlers, publications, training tools and health technology assessment (HTA) submissions. She has worked across a range of disease areas and has particular expertise in rare diseases, diabetes and obesity. Prior to joining Trinity, Sophie led a Value Communication team, comprising medical writers, graphic designers and programmers. Sophie holds a PhD in Biological Sciences and a BSc in Equine Science from Aberystwyth University, Wales.

Andrew Gould | Partner, Value, Access & Pricing

Andrew is a Partner in Trinity's Value, Access and Pricing practice and has over 15 years working within the Life Sciences arena. Prior to joining Trinity, he led CBPartners' U.S. Center of Excellence and focused on managed markets strategy engagements. His experience has emphasized the development of numerous pricing and access strategies for both pre-launch and in-line assets within the U.S. Specifically, Andrew supports asset development through various engagements, including: early stage/launch pricing, innovative and traditional contracting strategy, market access assessment, target product profile & optimized trial design review and value message development/testing.

References

1 ICER. Value Assessment Framework. Available at: https://icer.org/our-approach/methods-process/value-assessment-framework/ [Accessed October 2024].

2 ICER. Assessments. Available at: https://icer.org/explore-our-research/assessments/ [Accessed October 2024].

3 Ronquest, NA, Paret, K, Gopal Gould, I, Barnett, CL, Mladsi, DM. The evolution of ICER's review process for new medical interventions and a critical review of economic evaluations (2018-2019): how stakeholders can collaborate with ICER to improve the quality of ICER's reports. *J Manag Care Spec Pharm*. 2021;27(11).

4 Soares, MO. Is the QALY blind, deaf and dumb to equity? NICE's considerations over equity. *British Medical Bulletin*. 2012;101(1):17-32.

5 PhRMA. Value assessments should include all the outcomes that matter to patients and families. Available at: https://phrma.org/-/media/Project/PhRMA/PhRMA-Org/PhRMA-Org/PDF/G-I/ICER_Patients-Deserve-Value-Assessments.pdf [Accessed October 2024].

6 Alliance for Aging Research. ICER and drug price negotiation – what's at stake. Available at: https://www.agingresearch.org/icer-facts/ [Accessed October 2024].

7 Milstein Breslau, R, Cohen, JT, Ollendorf, DA, Neumann, PJ. Should drug companies engage with ICER? An empirical analysis of how often manufacturers engage with ICER and whether engagement may influence ICER's cost-effectiveness estimates. *Pharmacoeconomics*. 2022;6:893-895.

About Trinity

With almost 30 years of expertise, a best-in-the-business team and unrivaled access to data and analytics, Trinity Life Sciences is a modern partner to companies in the life sciences industry. Trinity combines strategy, insights and analytics to help life science executives with clinical and commercial decisionmaking. We serve over 300 pharmaceutical, biotech and medical device clients, helping them develop the right drugs and devices for today's market and optimize them once in market. We have a diverse staff of over 1200 people and 11 global offices across the U.S., Europe and Asia. Ultimately, we know that every decision our clients make impacts a life, and when we help our clients achieve their goals, the world benefits. To learn more about how Trinity is elevating the industry and driving evidence to action, visit trinitylifesciences.com

For more information, please contact us at info@trinitylifesciences.com.