CERTARA TRENDS **BRIEF**

The Rise of Cost Effectiveness Considerations in the US

Payer Views and Innovator Strategies

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Research background

- Using Certara's Compass research network, we conducted an online survey among active voting members of P&T committees in US managed care organizations (MCOs), followed by a set of semi-structured interviews for further interpretation and probing of key trends. In-depth interviews often help to clarify the gap between expressions in survey research and reality on the ground.
- A targeted literature review was conducted to contextualize the research in the current landscape of the specialty pharmacy category.
- Of 31 respondents, 19 were pharmacy directors and 12 were medical directors. These payers represent a total of 198.8M US lives (169.6M Commercial and 29.2M Medicare lives). All 31 respondents were responsible for Commercial lives and 25 of the 31 responsible for Medicare as well.
- Respondents were comprised of national (n=17) and regional health plans (n=14), pharmacy benefit managers (PBMs; n=7) and integrated delivery networks (IDNs; n=6).
  - MCOs were also categorized by size, based on the number of covered lives, into large, mid-sized and small health plans
  - Small plans: <920,000 lives; n=7 plans
  - Mid-sized/medium plans: (≥920,000 lives and <3.4M lives) (n=8 plans)
  - Large plans: (≥3.4M lives) (n=11 plans)
  - PBMs: 59.5M lives (6 unique organizations)
  - IDNs: 24.4M lives (6 unique organizations)
  - Small plans: 3.4M lives (7 unique plans; includes PBMs and IDNs)
  - Mid-sized/medium plans: 13.5M lives (8 unique plans; includes PBMs and IDNs)
  - Large plans: 182M lives (11 unique plans; includes PBMs and IDNs).
- Parts of the analysis presented in this report have been accepted for publication as posters by the American Academy of Managed Care Pharmacy (AMCP) in 2020, one of which has been awarded a prestigious gold ribbon in professional review.[1,2]

Limitations

The estimates and findings in this report are based on a qualitative research methodology. Throughout this report, the numbers of covered Commercial and Medicare lives per health plan type are reported to illustrate, and provide a deeper understanding of, the research findings. The numbers of reported lives are approximations provided by research respondents.

Certara has not accounted for any overlap in covered lives between health plans. As such, findings on the trends we document should be considered indicative rather than conclusive.

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[1] *Bangia I, Garcia Padilla J, Neumann U, Vargas M. A Survey-Based Analysis of Formulary Decision Making and Utilization Management Trends Across Managed Care Organizations. In Journal of Managed Care and Specialty Pharmacy; April 2020; Houston, TX.*

[2] *Neumann U, Bangia I, Garcia Padilla J, Vargas M. A Systematic Comparison of Status Quo and Future Expectations for Novel Drug Financing Strategies Across Managed Care Organizations. In Journal of Managed Care and Specialty Pharmacy; April 2020; Houston, TX.*

FIGURE 1
Payer readiness to employ ICER in P&T

We are integrating ICER assessments directly into the formulary evaluation process of our P&T committee. It has helped us improve the quality of our value assessments.

REGIONAL PLAN

We used the ICER report in our negotiations. Did we receive the ICER price? The answer is no, we didn't..

NATIONAL PBM

It will be necessary to include new bases of clinical and financial review... such as comparative effectiveness and QALYs, as the drugs are too expensive to pay for if they don't deliver enough either to individuals or populations.

NATIONAL PBM

People are finding the QALY concept to be more and more acceptable. As these kinds of approaches get adapted... pharma will have to change its view on what best pricing is.

NATIONAL PBM

I think everyone would welcome, including pharma and payers, a value-based pricing mechanism ... In the UK, they have NICE and in the United States we have ICER .

REGIONAL PLAN

I don't know what the right threshold is. But the fact that there is no agreement doesn't mean that the threshold is unlimited. The Brits actually get this, while we pretend it's an unlimited budget.

REGIONAL PLAN

Information from ICER on complex disease states has been helpful... using their report saves us roughly $10-30k per P&T meeting.

REGIONAL PLAN

ICER is responding to real needs in the marketplace, which is why it has become so visible.

IDN

...an important component to drug coverage decisions, helping to ensure the most clinically appropriate and cost-effective medications are preferred in drug formulary.

NATIONAL PBM

Market context

- The public debate around drug pricing has spurred demand for standardized value assessment in the US. A venture-funded think-tank called "ICER" (Institute for Clinical and Economic Review), has made its name as America's "drug price watchdog", selecting pharmaceutical products for review under cost-effectiveness criteria. The incremental health gains are measured in quality adjusted life years and equal value of life years gained, as complimentary method the organization suggests for life extending treatments (Figure 2).
- 97% of reports the organization published online in 2018 found that developer WAC prices do not match the value the products provide, requesting discounts beyond 60% in nearly half of all reviews. To further address affordability concerns, "ICER" also projects a budget impact of interventions on the basis of a population-level back of the envelope calculation for the US healthcare system, as shown in Figure 3.

FIGURE 2
(Simplified) components of ICER's value framework and cost-effectiveness calculation

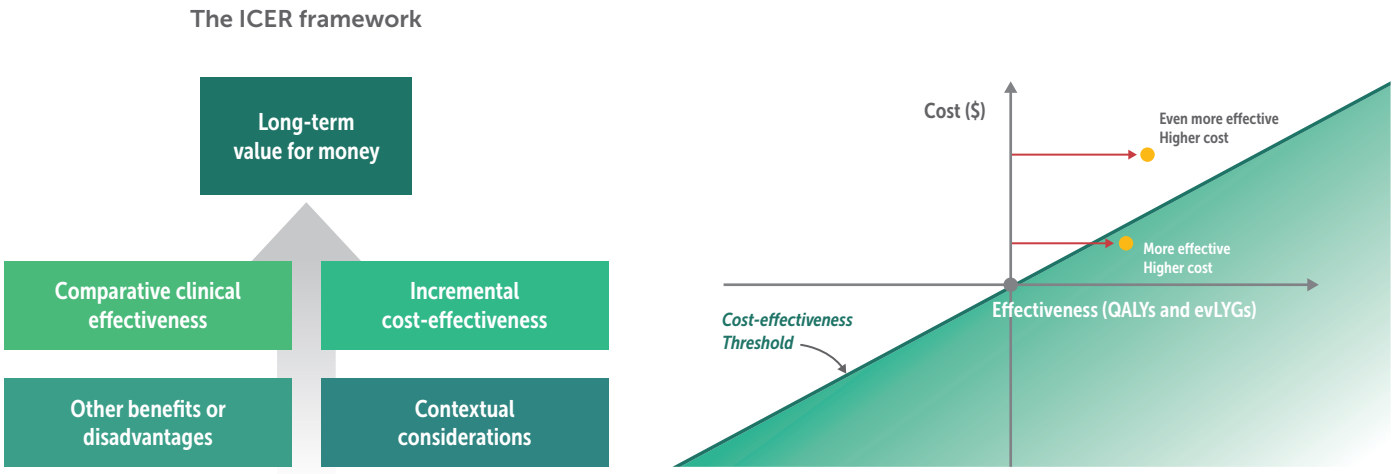


FIGURE 3
Assumptions, ICER budget impact calculation

Source: ICER

Item	Parameter	Estimate	Source
1	Growth in US GDP +1%	3.5%	World Bank, 2019
2	Total personal medical care spending, 2018 estimate	$2.95 Trillion	CMS National Health Expenditures, 2019
3	Contribution of drug spending to total health care spending (%) (Row 4 + Row 2)	16.9%	Calculation
4	Contribution of drug spending to total health care spending, 2018	$498.6 Billion	CMS National Health Expenditures, 2019; Altarum Institute, 2018
5	Annual threshold for net health care cost growth for ALL drugs (Row 1 x Row4)	$17.4 Billion	Calculation
6	Average annual number of new molecular entity approvals over 5 years (2014-2018)	42.6	FDA, 2019
7	Annual threshold for average cost growth per individual new molecular entity (Row 5 + Row6)	$409.6 Million	Calculation
8	Annual threshold for estimated potential budget impact for each individual new molecular entity (doubling of Row 7)	$819 Million	Calculation

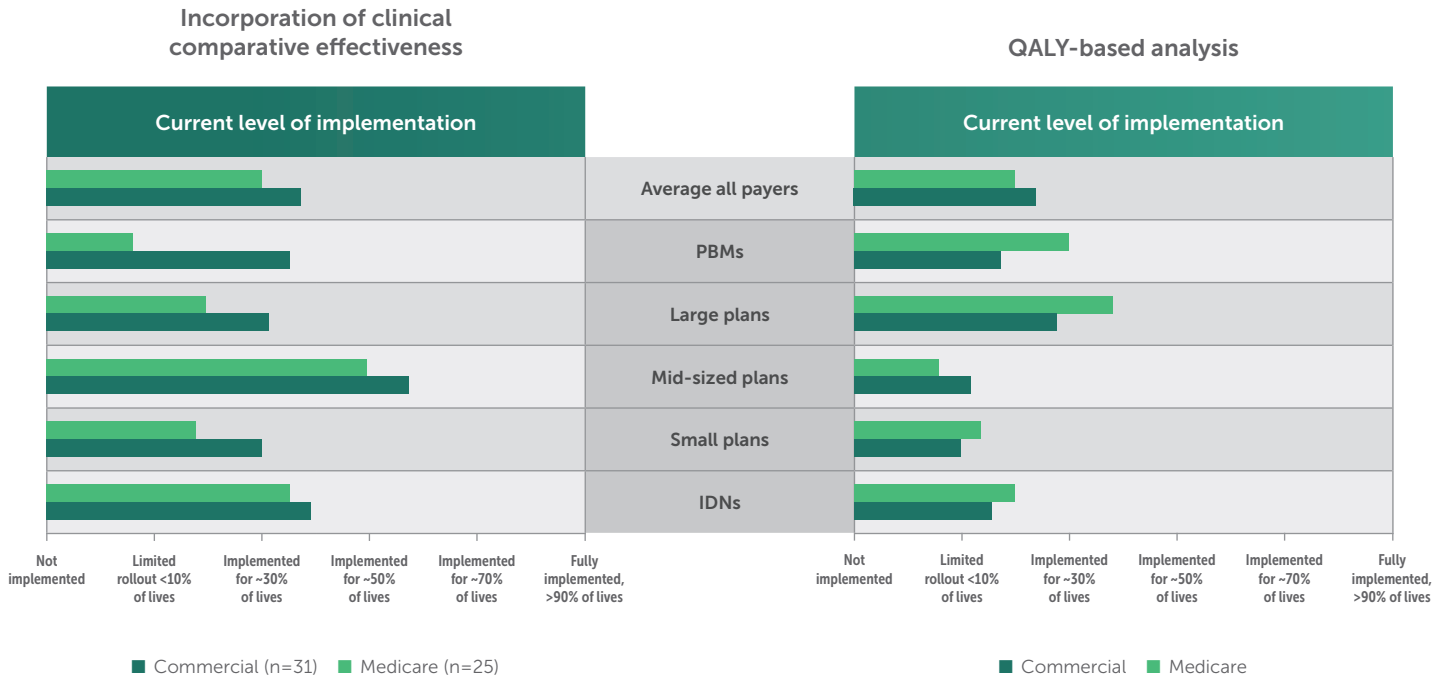
Key trends

CURRENT STATE

- Public payer statements (as shown in figure 1) for a broader adoption of value-based pricing and numerous recent research surveys have shown the growing desire to see the appraisal of pharmaceuticals based on QALYs. Some recent surveys indicate that 9 out of 10 payers would see a need for a US HTA, with 64.5% saying they are 'likely' and 'extremely likely' to follow ICER's cost-effectiveness thresholds.[3]
- In contrast, we see very limited use of QALY-based cost-effective analyses today among the surveyed payers for this research. The approach is reported to guide formulary inclusion/ exclusion with an estimated implementation of less than 10% of Commercial and Medicare lives. Clinical comparative effectiveness analyses see a higher level of implementation in about 40% of Commercial lives. Too often ICER reports do not get published in time for the initial P&T committee discussion.
- Follow-up interviews with our experts reveal that from an actuarial perspective, ICER offers limited value as a budgetary decision-framework to most US insurers who cannot easily translate their final pricing recommendations into coverage. ICER models are US population (vs. specific plan)-based and may differ on key assumptions from the back-of-the-envelope assumptions shown in figure 3. They are not replicable and partly non-transparent, and often come with a high degree of uncertainty. As a concept, QALYs are still largely intangible to US payer decision-making and a life-time horizon isn't useful for actuarial realities and short-term insurance windows in the US (considering frequent beneficiary plan switching).
- At the same time, we can report that ICER reviews are widely respected as an "independent" arbiter and a signal on overall product value, consistently used for background information on the evidence base and specifically for economic data points and key assumptions that enable the economic value story.
- Our research shows that an estimated 20% of payers incorporate QALY-based analyses into their price/rebate negotiations with developers for Commercial and Medicare plans.

FIGURE 4

Current level of incorporation of clinical comparative effectiveness or QALY-based analyses into formulary decisions/ QA



[3] *Pharma Exec Trends.*

FUTURE EXPECTATION

- 50% of payers[a], report that they are likely to use QALY-based assessments like ICER in decision-making. This contrasts with a higher share at 70% of payers[b], who are likely to use comparative effectiveness research in formulary decision-making in the next three years. They expect QALY-based cost-effective analyses to guide formulary inclusion/ exclusion for about 30% of lives in both Commercial and Medicare.
- Establishing an official, independent US HTA is payors' most preferred of all major recent policy proposals. While receiving average level of "somewhat" support, it still ranks roughly 20% in preference above drug Importation and POS rebate pass-through legislation, and even 4% higher than 'External Pricing Indexing,' such as introduced by HHS.[4] Payers managing 63.7M lives and 73.8M lives strongly favor or somewhat favor having an official cost-effectiveness body in the US, respectively.

FIGURE 5

Adoption of cost and clinical effectiveness research by payers

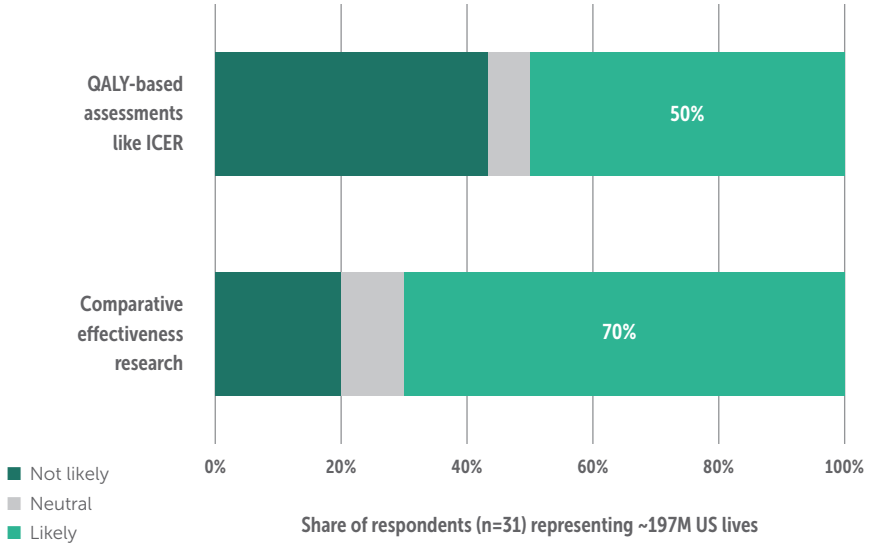
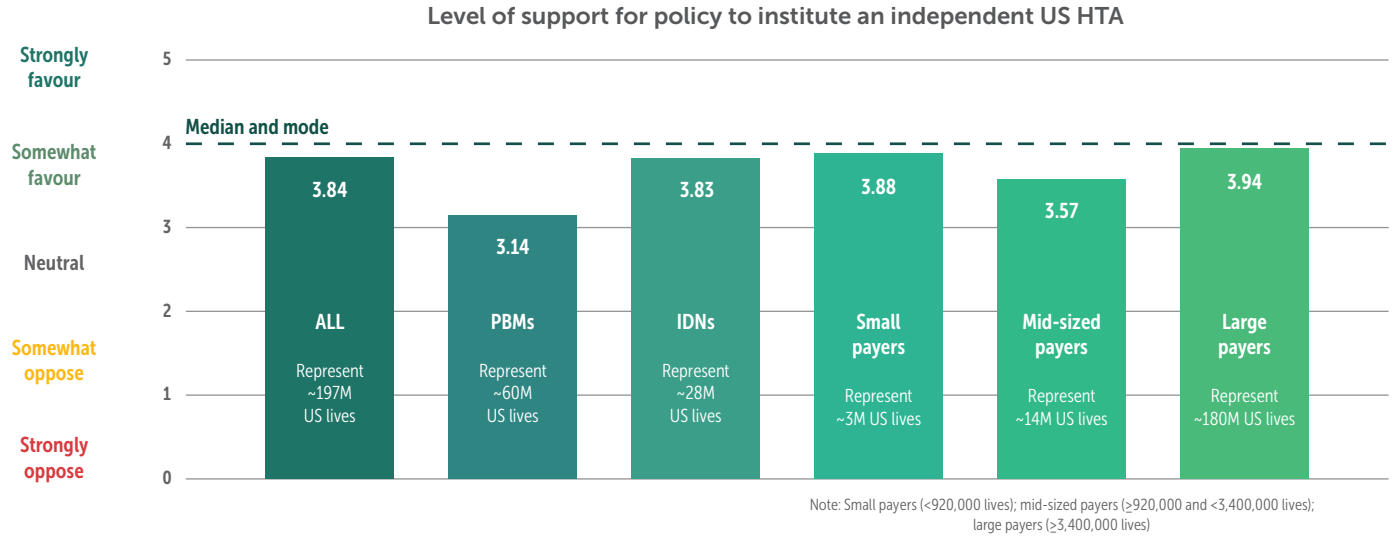


FIGURE 6

Level of payer support for policy proposal to institute an independent US HTA body which appraises drug value through QALY-based cost-effectiveness methods



Note: Small payers (<920,000 lives); mid-sized payers (≥920,000 and <3,400,000 lives); large payers (≥3,400,000 lives)

[a] *n=16, representing 136M lives*

[b] *n=21, representing 166M lives*

[4] *US Department of Health and Human Services. HHS Advances Payment Model to Lower Drug Costs for Patients. HHS.gov. https://www.hhs.gov/about/news/2018/10/25/hhs-advances-payment-model-to-lower-drug-costs-for-patients.html. Published October 29, 2018. Accessed June 1, 2020.*

DEVELOPER TAKEAWAYS

- While QALY-based approaches like that of "ICER" do not render themselves for easy adoption for payer decision-making, they have become an important element in negotiations and most payers today acknowledge considering such reports at some point during the drug evaluation process.
- Given the opportunity to use utilization management tools as outlined in previous sections, payers are keen to look for assumptions to define eligible patients when considering coverage, limiting PA to label and/or trial, and opportunities for coverage with evidence development and/or outcomes-based deals. Additional collection of clinical evidence may be required for re-authorization when coverage is re-evaluated.
- "ICER" does not currently follow a standardized selection process for its review of therapies. Getting involved with the process during the review window is critical but engagement doesn't equate to influence over shaping the report findings. Analyses show that contributions rarely results in major amendments in terms of the conclusion but may significantly influence the revision of model assumptions which may matter to US payers.
- "ICER" generally acknowledges industry comments per table response and tends to address specific methodological considerations with varying levels of robustness. As long as specific alternatives have been offered by the developer, roughly 1/3 of suggestions make their way into final reports, thereby modifying the final evidence report. However, significant variation exists and not all changes are desirable from a developer perspective.
- Developers should explain systematically why they might find specific "ICER" assumptions to be problematic and illustrate the materiality of these concerns towards the value determination more definitively wherever possible (e.g. are these concerns leading to a required shift in value category?). We reiterate that a strong need remains for developers to provide greater specificity and determination in their comments and interaction with ICER.

"ICER"– ready?

FIGURE 7
Targeted publications as part of a strategic ICER defense

SITUATION	SOLUTION
Tesaro's niraparib in ovarian cancer was selected for inclusion in ICER review; ICER findings suggested discount rates of 57-90%	*Certara published "Budget impact of niraparib as maintenance treatment in recurrent ovarian cancer following platinum-based chemotherapy" demonstrating the use of niraparib could result in significant cost savings compared with other maintenance treatment options included in the ICER report* Neeser K, O'Neil WM, Stern L, Harrow B, Travers K. Budget impact of niraparib as maintenance treatment in recurrent ovarian cancer following platinum-based chemotherapy. Journal of Comparative Effectiveness Research. 2019;8(8):577-587. doi:10.2217/cer-2018-0069
A client in the rare disease space required counter-publication as part of their public affairs strategy in light of the new ICER rare disease framework	*Working with world-renowned, independent cost effectiveness experts, including Paul Kind and Michael Schlander, Certara presented a guiding publication which argues against use of cost/QALY (ICER) in the realm of rare disease and regenerative therapies* Wagner M, Samaha D, Casciano R, et al. Moving Towards Accountability for Reasonableness – A Systematic Exploration of the Features of Legitimate Healthcare Coverage Decision-Making Processes Using Rare Diseases and Regenerative Therapies as a Case Study. International Journal of Health Policy and management. 2019;8(7):424-443. doi:10.15171/ijhpm.2019.24
UCB's certolizumab pegol was chosen for an ICER's review in rheumatoid arthritis. Project goal was to contextualize the positive results ("more effective, less costly" versus adalimumab) in a more payer friendly context for market impact (rather than using cost/QALY)	*Certara prepared model for publication showing certolizumab pegol treatment associated with lower one-year and two-year costs per low disease activity (cost per response) compared to adalimumab* Chua A, LeeE, Ralston P, etal. Cost Per Response of Certolizumab Pegol Versus Adalimumab Among Biologic-Naïve Patients With Moderate or Severe Rheumatoid Arthritis from the US Payer Perspective. Value in Health. 2018;21:S194. doi:10.1016/j.jval.2018.04.1321

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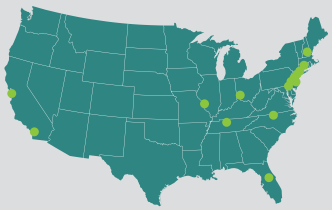

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