RTI (h)(s) Health Solutions

US Payer Perspectives on Value and Comparisons Outside the US: An Interview Study

Andrew P. Brogan, Josephine Mauskopf

RTI Health Solutions, Research Triangle Park, NC, United States

BACKGROUND

- The current challenge for the United States (US) health care system is to simultaneously meet the goals of sustained innovation, cost control, and improved quality.
- The International Society for Pharmacoeconomics and Outcomes Research has spearheaded a US Value Assessment Framework initiative and released a series of Task Force reports in February 2018 in Value in Health, providing a timely and thorough review of value in US and global health care decision making.[1]

OBJECTIVE

- To understand the current US payer landscape for achieving value in the use of pharmaceuticals and how it differs from payers outside the US.

METHODS

- In-depth phone interviews with 13 executive-level US managed care representatives and 6 health technology assessment (HTA) advisors (1 each in Australia, Canada, France, Germany, Italy, and the United Kingdom [UK]) were conducted in September–November 2017. Interviewees were provided with discussion questions and background information on value frameworks.

RESULTS

US Payer Definitions of Value

- US payers surveyed as part of this research did not have consistent definitions of value and were skeptical of the concept of achieving value within the current health care paradigm in the US.

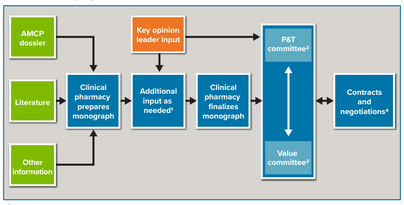
> "Clinical value: cost offsets…resource utilization (hospitalization, shorten hospitalization, reduce adverse events). Not looking at patient-reported outcomes. Manufacturers don't give us the information we need. Pharma is charging what the market will bear not the incremental benefit over other available options."
>
> US Medical Director F

> "This whole value framework/what is value thing is a conundrum to us…we do the best medicine. We just have to figure out a way to underwrite all this stuff, which in today's world is just shoveling risk around."
>
> US Pharmacy Director J

General Process for Payer Decision Making in US Health Plans

- Based on feedback from the US payers in this study, we developed the flow chart outlined in Figure 1. This flow chart outlines the general process for decision making in a typical US health plan.[2]

Figure 1. General Process for Payer Decision Making in US Health Plans



[1] Additional input may include internal analytical input and medical review.
[2] P&T committee focused on efficacy and safety and makes coverage decision based on medical necessity.
[3] Value committee focused on appropriate use (e.g., tiering, step edits, prior authorization).
[4] Provided with remit and boundaries for contracts and negotiations by P&T and value committees.

US Payer Use of Value Assessment Frameworks

- Value assessment frameworks are used as a reference by most US health plans, often to support decisions that US payers have already made.
  - While not formally considered by most US payers, the Institute for Clinical and Economic Review (ICER) and the National Comprehensive Cancer Network (NCCN) are the most commonly referenced value assessment frameworks among payers surveyed.

> "ICER trying to be like NICE but not that effective because we don't have a single-payer system. We can't control pharmaceutical prices like they can in Europe. We can't pay for indirect costs. We can't use QALYs."
>
> US Medical Director A

> "We use them when convenient for use…example is PCSK9…tell physicians that ICER doesn't think this drug is a good value so you shouldn't request it. Our only control over pharmaceutical pricing is in public shaming…this does have some impact…not a great management technique, but it works somewhat."
>
> US Medical Director B

> "ICER hasn't really released a report that says a drug is worth the money and nothing is changing…pharma hasn't lowered their price."
>
> US Pharmacy Director J

US Payer Approaches to Achieving Value in Pharmaceutical Decision Making

- US payers are limited in what they can do to achieve value in pharmaceutical decision making.
  - If a treatment is Food and Drug Administration (FDA) approved and there is no therapeutic alternative available, then US payers must cover the treatment in most cases.
  - Due to Centers for Medicare and Medicaid Services (CMS) requirements, payers must make a coverage decision within a set time period after market entry.
  - US payers are restricted in the formal use of incremental cost per quality-adjusted life-year and have limited or no control over pharmaceutical pricing.

Mechanisms US Payers Use to Achieve Value

- ***Coverage:*** Covering only treatments that are medically necessary.
- ***Tiering:*** Placing the treatment in a higher tier with a higher copay to try to limit use by increasing the financial burden to the patient.
- ***Step edit:*** Failing on one or more less expensive treatments before the health plan will cover a more expensive treatment.
- ***Prior authorization:*** Requiring the health plan's approval before access is given to the treatment.
- ***Preferred product(s):*** Competitive contracting for therapeutic equivalents.
- ***Public opinion and media mobilization***

Payer Perspectives on Outcome-Based Contracting

- Payers both inside and outside the US indicated that outcomes-based contracts can be very difficult to operationalize, and they are unsure of savings and achieving value.
- Implementing an outcomes-based contract adds a new burden to health care professionals and payers in order to manage, track, and implement the terms of the contract properly.

> "We are a national leader on [outcomes-based contracts]… Dozens of agreements in place. Not sure if this is the solution, despite being a national leader…jury is out. Not the solution for everything. A tool but not for every drug (e.g., hep C because know the drug works). Outcomes-based contracting is only valid for about 25% of drugs."
>
> US Medical Director E

> "With great skepticism. These are great for pharma so they can keep their high price. Very expensive and difficult to operationalize…no clear definition of success. Gene therapy and CAR-T… Millions of dollar cures…these would be areas where we would consider outcomes contracts because very high cost and clear measure of success = cure."
>
> US Medical Director C

> "Has been used somewhat. Minimally used in the future. Brilliant idea, but difficult to operationalize…large data requirements."
>
> France HTA Advisor

> "Too much hassle and administrative work… Look at Italy…not working great."
>
> Germany HTA Advisor

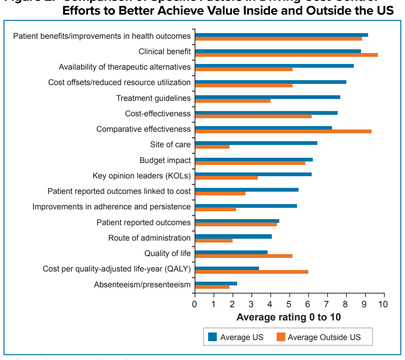
> "Pricing negotiations get delayed because can't reach an agreement, then finally managed-entry scheme is considered basically because of delay. Outcome defined in the contract. Difficult to manage. Outcomes contracts dependent on physicians entering data into the system. Managed by a web-based system in Italy. Can be onerous… This is a criticism of managed-entry schemes. Drug agency proposes managed-entry scheme after numerous delays. It is a way to maintain the price, and the budget impact is limited by volume controls. Still out of money in Italy so expected to increase."
>
> Italy HTA Advisor

- Outcomes-based contracting is not viewed as a broadly applicable solution by payers, but it could have some utility in specific cases where the treatment has a particularly high cost and there is a clear outcomes measure that can be readily measured.
- As part of this study, several US payers discussed the idea of discontinuation-based contracting as an outcomes-based contract with a simple, easily trackable outcome.
  - The rationale is that the patient discontinued the product, so the outcome was not achieved and the pharmaceutical manufacturer should provide a rebate to the health plan.

Factors Considered as Part of Value Determinations for Pharmaceuticals

- Payers inside and outside the US were asked to rate the importance of various factors to their value determinations on a scale of 0 to 10, where 0 is not important at all and 10 is extremely important (Figure 2).
  - Clinical benefit and patient benefits/improvements in health outcomes were rated high by all payers.
  - The availability of therapeutic alternatives, improvements in adherence and persistence, treatment guidelines, key opinion leaders, patient-reported outcomes linked to cost, route of administration, and site of care are all factors that are most important to US payers in their value determinations.
  - Payers outside the US placed a higher importance on cost per quality-adjusted life-year and comparative effectiveness.

Figure 2. Comparison of Specific Factors in Driving Cost-Control Efforts to Better Achieve Value Inside and Outside the US

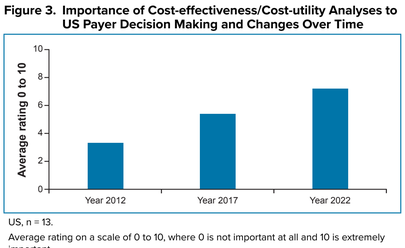


US, n = 13; outside US, n = 6.
Average rating on a scale of 0 to 10, where 0 is not important at all and 10 is extremely important.

Cost-effectiveness/Cost-utility in the US: Past, Present, and Future

- Several countries outside the US are well established in their use of cost-effectiveness/cost-utility analyses in value determinations, particularly Australia and the UK.
- As part of this study, US payers were asked how important cost-effectiveness/cost-utility analyses are to their health plan's decision making for pharmaceuticals on a scale of 0 to 10, where 0 is not important at all and 10 is extremely important, and how this is expected to change over time (Figure 3).
  - Cost-effectiveness/cost-utility analyses have increased in importance to US payer decision making over the last 5 years and are expected to continue to increase over the next 5 years.

Figure 3. Importance of Cost-effectiveness/Cost-utility Analyses to US Payer Decision Making and Changes Over Time



US, n = 13.
Average rating on a scale of 0 to 10, where 0 is not important at all and 10 is extremely important.

Achieving More Value for Pharmaceuticals in the US: Potential Lessons Learned From Other Countries

- US payers surveyed were largely open to learning from the health care systems of other countries; however, differences in how the system is set up (e.g., employer-based system) remain a challenge in the US.
- All other developed countries have price control mechanisms that the US does not, which severely limits the ability of US health plans to control costs.
- The UK has strict cost-effectiveness thresholds, and Australia, Canada, France, and Italy also consider cost-effectiveness as part of the decision-making process.
- Italy and France have effectively used price-volume agreements (i.e., clawbacks) to manage pharmaceutical costs and achieve value.
  - Price-volume agreements require pharmaceutical manufacturers to pay back all or a prespecified percentage over budget-impact estimates as part of providing access.
  - Price-volume agreements are typically negotiated behind closed doors, and the savings and details of these arrangements are not transparent.
  - US payers surveyed in this study did not consider price-volume agreements to be a viable mechanism for managing pharmaceutical costs and achieving value in the current health care environment in the US.
- The German health care system was cited by several US payers as having pharmaceutical cost-control measures to better achieve value.
- The central element for the German system is the requirement of patient-relevant benefit compared to a G-BA–approved comparator.
  - Patient-relevant benefit is defined as mortality, morbidity, quality of life, and safety.
  - Morbidity endpoints must be considered patient-relevant and exclude surrogate endpoints like progression-free survival in oncology and lowering HbA1c in type 2 diabetes mellitus.
  - Patient-relevant morbidity endpoint examples considered in Germany include reductions in cardiac events, avoiding stroke, and reductions in hospitalizations.

> "[For Germany] must be patient-relevant benefit: mortality, morbidity, quality of life and safety. Only consider improvements versus active, G-BA–approved comparator. Morbidity—only patient-relevant benefit. For example. in cancer, endpoints PFS and imaging are not considered. HbA1c not considered in type 2 diabetes. No lipid level improvements considered, no lowering of blood pressure endpoints. Endpoint must be patient relevant: avoid stroke, avoid neuropathy, avoid hypoglycemia, etc. Completely rely on the manufacturer studies…onus on the manufacturer [to provide this data]."
>
> Germany HTA Advisor

> "Payback or clawback rule has had an impact…only thing that is known to work. If budget impact is higher than anticipated, then claw back money from manufacturer. [Italy has had success with] flexibility of solutions. Empowerment of AIFA is crucial…power to negotiate with manufacturer."
>
> Italy HTA Advisor

> "Transparent, legal [system] that can be appealed to the highest court. Process is also fairly timely. Internal NICE appeals take 4-6 months. Manufacturer still can appeal to high court. If no government backing, then dead in the water. Have been contacted by US ICER to do a pilot…if no teeth, it will fail. This is why the first cancer drug fund [in the UK] failed."
>
> UK HTA Advisor

> "Yes. We could learn from [other countries]. These are real-world trials. UK, France, Germany use slightly different systems to determine value. Germany has a budget, and it must be managed to that budget."
>
> US Medical Director B

> "Germany: requiring certain comparators and patient-relevant benefit…make specific demands. Clawbacks like in France would be difficult to implement.
>
> CMS and FDA could be doing more…they have more leverage then they are willing to use…create realistic requirements to make value-based decision. CMS and FDA could push back on pharma."
>
> US Medical Director F

> "Rare and specialty…look closely at European data…used to be 'we are the US, and we want US data,' but there is good data in these single-payer systems that are applicable to the US, especially in rare and specialty. Look at value, start/stop, treat and not treat, subpopulation identification…all will be evaluated."
>
> US Pharmacy Director J

CONCLUSIONS

- Providing access to the ever-increasing number of innovative treatments at higher prices remains a challenge for US health plans to underwrite all risks associated with provided health coverage.
- Cost and value will increasingly be part of the discussion. The use of value assessment frameworks, cost-effectiveness/cost-utility analyses, comparative effectiveness, strictly defining patient-relevant benefit or requiring specific outcomes measures, and competitive contracting and preferred status for therapeutic equivalents are all expected to increase in the US.
- US payers consider health systems outside the US to have valuable lessons that could have roles in the US, many of which would require government intervention (e.g., a national health system covering everyone, pricing negotiations, and strictly defining patient-relevant benefit or requiring specific outcomes measures as part of regulatory approval or reimbursement by CMS) to be properly implemented.

ACKNOWLEDGMENT

Renu Vekaria and Doreen McBride contributed to the development of this poster.

REFERENCES

1. Neumann PJ, Willke RJ, Garrison LP Jr. A health economics approach to US value assessment frameworks–introduction: An ISPOR Special Task Force Report [1]. Value Health. 2018;21:119-23.
2. Hogue SL, Brogan AP, Earnshaw SR, Khan SB, Nelsen SH. Academy of managed care pharmacy (AMCP) dossiers: use in health care decision making. Poster presented at the ISPOR 19th Annual International Conference; June 2, 2014.

FINANCIAL SUPPORT

This research study was sponsored by The Pew Charitable Trusts, Washington, DC.

CONTACT INFORMATION

Josephine Mauskopf
Vice President, Health Economics
RTI Health Solutions

Phone: +1.919.541.6996
Fax: +1.919.541.7222

E-mail: jmauskopf@rti.org