Applying Cost-Effectiveness Thresholds to the Real World

Implications on Access for Medicare Beneficiaries
Summary of Findings

- Xcenda analyses have found that if Medicare Part B were to apply the cost-effectiveness thresholds utilized by the Institute for Clinical and Economic Review (ICER) as the basis for coverage policy, 62% to 93% of patients with serious, complex conditions would face access barriers to clinically important physician-administered treatments.

- The findings quantify the disconnect between the variable, evidence-informed, real-world needs of patients and the one-size-fits-all judgments made in ICER’s (and similar) cost-effectiveness calculations.

- The findings suggest that:

  Medicare reliance on a cost-effectiveness standard would ultimately harm patient access.
  
  Cost-effectiveness standards ignore the real-world needs of providers and patients.

Introduction

Continued concerns about rising healthcare costs have prompted renewed proposals for use of a single value threshold (eg, cost-effectiveness threshold or similar approaches) as the basis for setting coverage or payment policy. For example, in March 2018 the Medicare Payment Advisory Commission (MedPAC) considered the use of cost-effectiveness in Medicare coverage and payment policy. Similarly, a 2016 Medicare proposal (which was ultimately withdrawn) suggested use of value assessments performed by ICER, which employ cost-effectiveness thresholds, as the basis for setting policy in the Medicare program. To further inform debate about the role and implications of value standards in healthcare policy, Xcenda examined the potential impact if ICER’s value assessment framework was applied across 4 conditions in the Medicare Part B program. Xcenda selected ICER’s framework because it appears to be designed for use at the policy level; ICER was referenced in MedPAC deliberations and was cited by the Centers for Medicare & Medicaid Services (CMS) in its controversial 2016 Part B payment demonstration.

Xcenda’s analysis found that if the Medicare program drove patients toward treatments deemed “cost-effective,” according to ICER’s framework, nearly 140,000 of the 225,000 (62%) patients included in the analysis could lose access to their physician’s treatment of choice and could be forced to switch to a therapy deemed cost-effective. As a result, many patients would likely receive treatments that are less safe or effective for them due to their different clinical circumstances or that fail to address their specific life circumstances and preferences.

These findings quantify the ways that cost-effectiveness standards are disconnected from care that is valued by patients and fail to reflect important differences in perspectives on value, both among stakeholders and within a typical patient population. As a result, use of cost-effectiveness analysis (CEA) as a rigid standard for policy has the effect of replacing the doctor and patient decision-making process with that of a single value judgment based on population averages and not the individual.

Our results underscore the importance of ensuring that value assessments are used in ways that do not prevent patients and doctors from making informed choices about care options and the need for alternatives that are more physician- and patient-centered. Ideally, current or alternative tools that encourage informed, individualized decisions at the physician and patient level could be tailored and supported.
Evolution of Health Technology Assessment (HTAs) in the United States

Some countries, such as the United Kingdom (UK), France, and Germany, have public healthcare systems designed around centralized bodies that develop HTAs. These entities have the regulatory authority to impose their decisions and, as a result, have been used to limit patient access to treatments and therapies. These denials have led to outcry from stakeholders, particularly patients and providers, on several occasions.

In the US, development of both privately and publicly funded HTAs has expanded since 1972 through the Agency for Healthcare Research and Quality, the Oregon Drug Effectiveness Review Project, and the Center for Clinical Effectiveness (formerly the Blue Cross and Blue Shield Association Technology Evaluation Center). HTAs increased in prominence again in 2015, as multiple organizations offered their versions of value assessment frameworks. One of the more visible of these organizations is ICER, which relies on traditional cost per quality-adjusted life-year (QALY)-based cost-effectiveness methods. According to ICER, the organization is focused on using evidence to “provide a foundation for a more effective, efficient, and just healthcare system.” Some of the institute’s reviews have been controversial, and ICER has faced significant pushback on its methods and the potentially negative implications for patient access.

While CEA studies are considered by payers in the US, their context and application is different from their use in other countries such as the UK. These differences, which involve CEAs being tailored to different decision-maker needs and used much more judiciously as one of many data points rather than a rigid policy standard, could make approaches such as ICER’s poorly suited to the US context.

Implications of Introducing Value Thresholds Into Medicare

Americans have resisted the use of CEAs in public insurance programs, particularly QALYs. Critics of QALYs, including patient and disability advocates, argue that QALYs fail to incorporate patient-centered care and systematically disadvantage patients with disabilities and severe illness. Even representatives from ICER have acknowledged that “attempting to capture and quantify quality of life” is a politically thorny issue. In fact, opposition to QALYs is so high in the US that when Congress created the Patient-Centered Outcomes Research Institute as part of the Affordable Care Act, it explicitly forbade the use of QALYs in cost-effectiveness research and the use of comparative or CEAs to restrict or limit patient access in Medicare.

In 2016, the Center for Medicare & Medicaid Innovation (CMMI) proposed a demonstration project that would have radically changed the way Medicare pays for drugs under the Part B program. A planned phase 2 had envisioned use of value frameworks such as those developed by ICER to implement reference-based pricing (a policy where products with similar health effects are reimbursed equally, often at the lowest available price) and indications-based pricing (a policy where reimbursement for the same product is set differently depending on the condition it is used to treat). (CMMI was able to overcome the prohibition on use of QALYs in determining Medicare coverage due to its waiver authority.) Republicans and some Democrats in Congress objected to the proposed demonstration, arguing that it could limit patient access to certain drugs; was too large in scope; and could harm independent, small, and rural physician practices. A wide range of stakeholders strongly opposed the proposal, with many patient and provider groups expressing opposition to phase 2, fueling a debate about whether Congress should consider placing checks on the broad authority of CMMI.

Determining which treatments are ‘therapeutically similar’ is fraught with pitfalls, as treatments that work for the average at the population level may not work for the individual patient. This model homogenizes the patients, but in reality, disease severity levels and stages demand treatment variations.
– American Academy of Dermatology Association

The American Lung Association’s concern with the value-based payment tools is that the generalization of clinical effectiveness and health outcomes tied to drug payment does not consider personalized care for the patient and will negatively impact the patient’s quality of care.
– American Lung Association

Value-based payment programs can be a force for improved patient care but are often crafted without the patient’s voice in determining what clinical and personal outcomes matter most and how they can be achieved. The use of CEA, in particular, is concerning.
– COPD Foundation
In late 2017, CMS withdrew the Part B drug payment model, citing “the complexity of the issues related to the proposed model design and the desire to increase stakeholder input.” 

If CMS radically altered the Medicare fee-for-service (FFS) payment approach to incorporate a cost-effectiveness-based formulary rather than covering all Food and Drug Administration-approved, physician-administered drugs, beneficiary access and provider choice for necessary treatments could be constricted. To illustrate the impact of such a change, we estimated the number of Medicare Part B patients with 4 conditions who would theoretically be forced to switch to alternative drugs should the program adopt an ICER-based formulary tailored to include drugs deemed most cost-effective in their evaluations.

Methodology

The 2016 Medicare Part B 5% Standard Analytic Files were used to estimate the number of patients utilizing ICER-evaluated physician-administered drugs for rheumatoid arthritis (RA), multiple sclerosis (MS), non-small cell lung cancer (NSCLC), and/or multiple myeloma (MM). Data were weighted to national FFS estimates. The 4 disease areas examined by Xcenda are all marked by multiple treatment options, considerable variability in patients’ clinical needs and preferences, frequent sequencing of patients on a series of different treatments until the optimal regimen is identified, and a relatively strong base of clinical evidence and availability of multiple, rigorous tools for clinical decision support (such as the National Comprehensive Cancer Network guidelines and the American College of Rheumatology clinical practice guidelines).

The analysis included patients with at least 1 administration of a drug with the appropriate diagnosis code on the claim, and they were categorized into 3 groups:

- Patients receiving physician-administered drugs deemed most cost-effective by ICER
- Patients receiving physician-administered drugs not deemed most cost-effective by ICER
- Patients receiving at least 1 physician-administered drug deemed most cost-effective and 1 physician-administered drug not deemed most cost-effective

The assessment compared only physician-administered, ICER-evaluated medicines that were available in 2016. Therefore, Part D medicines that have been reviewed by ICER were not included in this evaluation to estimate the impact of patients shifting from Part B to Part D treatment. Additionally, this analysis spans only 1 calendar year; thus, the impact could be larger for patients who may need to shift therapies over a longer period of time and would have limited options for access.
Results and Key Findings

In 2016, over 200,000 Medicare Part B FFS beneficiaries with RA, MS, NSCLC, and/or MM used a physician-administered product evaluated by ICER. If the Medicare program was to adopt an ICER-based formulary, 59% to 93%, or nearly 140,000 of these patients, could lose access to their physician’s treatment of choice and could be forced to switch to a therapy deemed cost-effective.

Percentage of Patients Who Could Lose Access to Current Treatment With an ICER-Based Part B Formulary

- **RA** (N=151,685):
  - Would stay on treatment: 41%
  - Could be forced to alternative treatment: 59%

- **MS** (N=13,000):
  - Would stay on treatment: 7%
  - Could be forced to alternative treatment: 93%

- **NSCLC** (N=45,001):
  - Would stay on treatment: 38%
  - Could be forced to alternative treatment: 62%

- **MM** (N=15,257):
  - Would stay on treatment: 33%
  - Could be forced to alternative treatment: 67%

Discussion

It is widely acknowledged among all stakeholders that patients differ in their clinical characteristics, such as genetics and comorbidities, and their priorities and preferences. For example, recent analysis shows wide variability among outcomes that MS patients view as important and variability between what MS patients value and what payers and other stakeholders value. Such variability would make it exceptionally challenging to reach accurate, appropriate judgments about the comparative effectiveness of treatment options at the population level. This is clearly shown in the above-described results.

Due to the significant heterogeneity among patients, it is difficult for a population-level CEA to accurately predict the impact of preference for a specific treatment on an individual patient. In some disease areas, such as RA and MS, it can be nearly impossible for a clinician to determine what treatment will work best for a patient—unless the patient can try different treatments. For example, patients with RA respond differently to treatments in the same therapeutic category, and treatments may be used sequentially if patients become resistant to or fail to respond to a treatment. Medicare Part B currently covers 7 drugs that treat RA, and it is common for patients with RA to try multiple treatments before they find the one that works well for them. Even once patients are stable, they can stop responding to their treatment and may need to switch medications to continue to effectively manage their condition.
Cost-effectiveness determinations that drive patients toward a single, high-value treatment also may not reflect the reality of treating certain diseases and conditions. Recent analysis has confirmed that cost-effectiveness analyses may not reflect aspects of a treatment’s value that matter to patients. A study found that assessments like ICER’s that rely on CEAs do not accurately reflect the value that patients place on specific health gains because they are focused on the payer’s perspective. The study found that “patients with metastatic colorectal cancer placed a greater value on survival gains than has previously been recognized by health regulators and payers,” and concluded that “ignoring patients’ preferences for life extension might be a major misstatement of value in healthcare.”18

Physicians have similarly expressed concern that ICER’s assessments do not reflect how physicians care for their patients. For example, the American Society of Hematology (ASH) wrote to ICER that “the scope of ICER’s analysis is far too narrow because it does not represent the realities of clinical practice.” ASH’s analysis concludes that many of the treatments ICER designated as “high value” are older, less-effective treatments that very few patients are being prescribed.19

If an ICER-like standard were imposed for Medicare Part B, many patients could potentially lose access to the treatments that they and/or their physicians had determined were best for them based on their individual needs and preferences and after careful consideration of the nuances of their condition.

Relying on standards like those generated by ICER to limit patient access to a single treatment has been met with strong opposition in other countries. In 2008, the UK’s National Institute for Health and Care Excellence imposed significant restrictions on patient access to treatments for RA, forcing patients to try only 1 of 3 highly effective RA drugs called anti-tumor necrosis factors (TNFs). Patient organizations voiced strong opposition to the decision, stating that having the flexibility to tailor their care was essential to finding the most effective treatment. One expert stated, “It’s almost impossible to know which anti-TNF will work for a patient at the outset. Before this decision, we could try patients on each of the 3 treatments in turn to find that was effective for them. Now we will only have 1 shot at success.”20

It is possible, of course, that Medicare could incorporate safeguards into a cost per QALY standard, such as an exceptions or appeals process. However, such processes in the past deterred patients from specific products and have been found to be overly burdensome for patients and healthcare providers, significantly delaying access to the treatment the patient ultimately needs.21