INSTITUTE FOR CLINICAL AND ECONOMIC REVIEW

Value Assessment and International Reference Pricing: Distinctive Strengths and Weaknesses as a Foundation for Medicare Drug Price Negotiation

An ICER Policy Perspective Produced for the ICER Policy Leadership Forum

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Based on a webinar featuring:

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Introduction

The need to improve drug affordability remains one of the few areas of bipartisan agreement in the US, with polling showing that a majority of both Democrats and Republicans want the federal government to act to lower prescription drug prices.[1] Policymakers from both parties have advanced proposals to rein in drug costs, but there is no consensus on the primary problem – patient out of pocket expenses versus costs to Medicare and other insurers – or on root causes and best approaches to address costs while maintaining the right amount and focus of incentives for future innovation.[2-4]

Many policymakers, however, have suggested that giving Medicare the ability to "negotiate" prices for both Part D and Part B drugs will be a necessary step to remedy one of the primary reasons that costs for drugs in the US are so much higher than in other countries.[5,6] Although the life science industry and some policymakers remain staunchly against the idea of Medicare negotiation, the Trump administration advanced specific proposals for drug price regulation and the Biden administration has announced its strong support. In the runup to the 2021 Congressional budget legislation, debates about the best approach for Medicare negotiation have flourished in anticipation that Democrats will seek to advance some version this year.[7-11]

Among policymakers favoring Medicare negotiation, most have suggested that legislation adopt one of two primary mechanisms to determine "fair" or "reasonable" price targets: international reference pricing (IRP), or value assessment.[12,13] There has been some mention of "domestic" reference pricing as a third option, but the details of this approach have not been explained clearly enough to enable a clear understanding of how pricing would be determined.[14] IRP is based on the creation of an international pricing index that would identify the prices paid for a drug by a set of countries with advanced economies relatively comparable to the US. The average (HR3) , or the lowest (Trump administration "most favored nation" proposal), of these prices would be used as the maximum fair price that Medicare would pay. In 2019, the Democratic majority in the House of Representatives passed the Elijah E. Cummings Lower Drug Costs Now Act (HR3), which called for Medicare to negotiate drug prices for certain high-cost drugs using a maximum fair price of 120% of the average price paid in six other countries (Australia, Canada, France, Germany, Japan, and the UK).[15] Medicare would be empowered to negotiate prices lower than this maximum fair price, but this price remains an important conceptual and practical anchor for negotiation. Although HR3 was not taken up in the Senate, it has been re-introduced in the current Congress as a prelude to the current consideration of Medicare negotiation in budget legislation.[16]

Separately, the Biden administration has in the past signaled interest in tying US drug prices to those in other countries, although the President in a recent personal statement has called for Medicare negotiators to be "provided a framework for what constitutes a fair price for each drug," suggesting he is open to approaches other than IRP. Other policymakers have also called for fair pricing to be based solely or in part on a homegrown approach to determining how

well a drug performs compared to other options, an approach often called "health technology assessment," or "value assessment." For example, Senator Ron Wyden of Oregon, Chair of the US Senate Committee on Finance, issued a statement calling for Medicare price negotiation in which he describes aligning price and value through the commissioning of studies to be carried out by independent entities.[14,17]

In the context of this ongoing national policy discussion on Medicare negotiation, the Institute for Clinical and Economic Review (ICER) hosted a webinar on July 21, 2021 to explore the potential advantages and disadvantages of IRP and value assessment as mechanisms for informing or determining the fair price sought by Medicare. Given time constraints, the discussion was not intended to include broader consideration of whether Medicare negotiation was a desirable national policy, and therefore only proponents of Medicare negotiation were invited to participate. The discussion was moderated by ICER's President, Dr. Steven Pearson and featured the following panelists:

- Richard G. Frank, PhD, Margaret T. Morris Professor of Health Economics, Department of Health Care Policy, Harvard Medical School
- Aaron S. Kesselheim, MD, JD, MPH, Professor of Medicine, Brigham and Women's Hospital / Harvard Medical School
- Rachel Sachs, JD, MPH, Professor of Law, Washington University in St. Louis.

Comparing the Potential Advantages of International Reference Pricing and Value Assessment

Within the webinar discussion multiple dimensions of IRP and value assessment were discussed. Table 1 on the following page presents a general summary of relative advantages that panelists described for each approach. Importantly, panelists emphasized that the two approaches are not mutually exclusive, and that either one could serve as the backstop for Medicare negotiation that provides a concrete element necessary for the CBO to calculate potential savings, with the other serving as a piece of information to guide a broader negotiation. Key differences in relative advantages between IRP and value assessment are described following the Table.

Table 1. Relative Advantages and Disadvantages of IRP and Value Assessment as Mechanisms for Guiding Fair Price Considerations Within Medicare Negotiation

	International Reference Pricing	Value Assessment
Savings for Medicare	HR3 scored by CBO* as saving approximately $450B over 10-year period	Has not been scored but extrapolation from ICER reviews suggests $350B-$600B depending on cost-effectiveness methods used
Impact on incentives for high-priced therapies, including cell and gene therapies	May reflect lower prices paid for breakthrough therapies in Europe	May scale pricing higher for high-impact treatments, especially one-time cell and gene therapies
Political advantages	Simple to describe to lay public; no reliance on measures that can be framed as putting a price on life; no reliance on "experts" who may be mistrusted	A "homegrown" US approach, which can be touted as reflecting US goals and values; can be based on cost-effectiveness measures other than the QALY
Implementation advantages	Ready for implementation from day 1; could be used as a short-term bridge to value assessment	Methods can be adapted to reflect domestic public health priorities. May prove less vulnerable to strategic shifts in timing of drug launches overseas and special arrangements with other countries that would obscure true net pricing
Implementation disadvantages	Public prices available from overseas often do not reflect additional secret bargains that countries negotiate and therefore could be overestimates. Over time, drug makers may choose to adapt by slowing drug launches in certain overseas markets.	Time to finalize methods and scale to provide assessments for 25-50 drugs may take 1-2 years. Creation of new value assessment infrastructure may not meet conditions required for inclusion in reconciliation legislation.

*CBO = Congressional Budget Office

Savings for Medicare

Both IRP and value assessment can be scaled to create different levels of cost savings for Medicare and, potentially, private payers. HR3 was scored by the Congressional Budget Office (CBO) as leading to $456 billion in savings over 10 years.[18] But this amount could be made larger or smaller depending on the number and type of drugs included in negotiation, the countries included in the international pricing index, and the premium above the average price that would serve as the maximum fair price. Similarly, value assessment could be applied to any number of drugs, and cost savings would also depend on the cost-effectiveness threshold applied or on other techniques selected that might be used to determine a "value-based price."

Impact on incentives for high-priced therapies

The impact of Medicare negotiation on manufacturer profits and the output of new drugs is one of the most hotly debated elements in policy discussions, no matter what mechanism is proposed to identify maximum fair prices. The two CBO analyses of HR3 have suggested that the lower profit margins expected with HR3 would lead to between two to eight fewer drug approvals over the first decade, but arguments have persisted over the validity of this estimate and whether the "lost" drugs would be more likely to be me-too or high-impact drugs requiring more risky development courses.[18,19]

Panelists argued that there is no strong rationale to believe that life science companies would tilt investment away from riskier assets under Medicare negotiation using either IRP or value assessment. However, value assessment using cost-effectiveness scaled to the health care costs and opportunity cost threshold in the US would likely assign higher prices to treatments that provide a substantial benefit over prior options. In particular, one-time or short-term treatments, such as cell and gene therapies, would likely receive higher price recommendations through value assessment than through IRP. Although it is not clear that the therapies targeted for negotiation would include newer high-cost products with limited budget impact, some would argue that value assessment has a relative advantage in being able to scale pricing in a way that encourages high-value drug development and more easily retains incentives for treatments requiring greater time, risk, and investment.

Political advantages

Panelists shared differing views of the relative advantages of IRP versus value assessment when considering the political aspects of describing the mechanism to the American public and gaining their support. IRP is simpler to describe and benefits from a visceral response among many Americans to the perceived unfairness of paying far more in this country for the same drugs than people in other countries. In addition, IRP has the advantage that its approach

requires the creation of no "experts," no new group within the US government that needs to make difficult judgments about fair pricing; that task is handled by other countries.

Value assessment has a contrasting set of political advantages. Most centrally, it can be positioned as a "homegrown" or "American" solution to a problem that has distinctive features in the American health care landscape. American values – and not foreign ones – can be honored in the foundation of a value assessment approach, whereas IRP requires Americans to accept the judgments of foreign governments. One panelist noted, however, that this critique of IRP would hold true only if IRP were used to set prices, rather than being one element of broader negotiating platform.

Another relative advantage of value assessment may live in its ability to frame fair prices without direct or indirect reference to cost-effectiveness analyses based on quality-adjusted life years (QALYs). Some other countries link drug prices to patient benefit through application of standard cost-effectiveness analyses which use QALYs as the central measure of health improvement. But certain groups in the US have long advocated against use of the QALY in health policy as leading to discriminatory rationing of care, complicating the political path for both IRP and value assessment. In value assessment methods developed by ICER, non-QALY alternative measures can be used instead to avoid any connection to the QALY, thereby offering politicians the option of supporting an American version of value assessment that more strongly avoids attacks that its methods are discriminatory.

Implementation advantages

IRP has the major advantage that it could be implemented rapidly without great elaboration of additional methods. Drugs that are high cost in the US system are almost all approved for use in other countries, and thus the international pricing index could be calculated and used in negotiation without any delay. Legislation using value assessment as the primary mechanism for establishing a maximum fair price would require some period of time in order for assessment methods to be determined in detail and for the necessary infrastructure to be created to support the development and deliberation of value assessments. ICER has established strong precedents for technical and procedural methods for value assessment in the US context, but it seems likely that the government would require additional time to formalize the intended content of assessments, identify the organization(s) that would conduct them, and establish an infrastructure inside Medicare for applying the results of assessments within negotiation.

Panelists commented that IRP could be used as a short-term bridge pending the full development of a value assessment approach, and also noted that value assessment had an important potential advantage in the long run because it is not dependent on the prices paid for drugs in other markets. One concern with IRP is that it may be more susceptible to "gaming" by drug makers. To obscure the true net price of a drug from the figures reported to the US

government, drug makers almost certainly would revise their strategy for launching drugs in certain markets depending on the impact it would have on the IRP process in the US. Some drugs might never be launched overseas, might be launched in different formulations or delivery mechanisms, or might come forward only with complex pricing and payment mechanisms lacking transparency through to the international pricing index. The impact on patients in international settings, and the repercussions more broadly should drug makers shift away from those markets, are unknown, but represent concerns specific to IRP. Value assessment would face none of these vulnerabilities and thus has some important long term potential implementation advantages.

Conclusion

Whether Medicare negotiation makes it through the legislative process in the coming months, the momentum behind the idea has triggered deeper consideration of the specific mechanisms that would be needed to support the government in determining a fair price. IRP and value assessment appear the current dominant paradigms for providing Medicare with a "framework" for fair pricing, but as pointed out earlier, they are not mutually exclusive, and may also be integrated alongside information about drug development costs, eligible patient population size, and many other practical or conceptual factors that may be relevant to a drug's pricing. The panelists in the ICER webinar all strongly support Medicare negotiation, and thus do not represent the full spectrum of perspectives on these issues. But they agreed that Medicare negotiation is needed to correct an underlying imbalance in the US health care system. They each saw certain advantages and disadvantages in selecting IRP or value assessment as the mechanism for establishing maximum fair prices in the US market. They advocated for Medicare to have the benefits of the information derived from both methods as cornerstones of negotiating with drug makers. To achieve the right balance of fair pricing, fair access, and future innovation, the US may be able to learn from the experience in other countries while also creating an approach tailored to meet the unique aspects of the US health care system and of US society.

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Webinar Speakers

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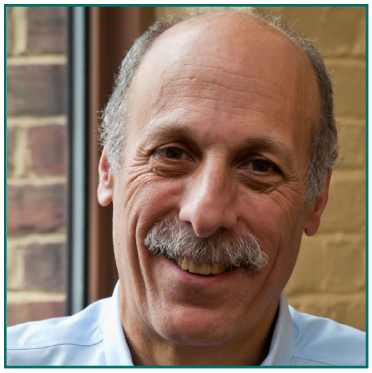

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