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The ICER Value Framework: Integrating Cost Effectiveness and Affordability in the Assessment of Health Care Value

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ABSTRACT

What should be the relationship between the concepts of cost effectiveness and affordability in value assessments for health care interventions? This question has received greater attention in recent years given increasing financial pressures on health systems, leading to different views on how assessment reports and decision-making processes can provide the best structure for considering both elements. In the United States, the advent of explicit value frameworks to guide drug assessments has also focused attention on this issue, driven in part by the prominent inclusion of affordability within the value framework used to guide reports from the Institute for Clinical and Economic Review. After providing a formal definition of affordability for health care systems, this article argues that, even after using empirical estimates of true health system opportunity cost, cost-effectiveness thresholds cannot by themselves be set in a way that subsumes questions about short-term affordability. The article then presents an analysis of different approaches to integrating cost effectiveness and budget impact assessments within information to guide decision making. The evolution and experience with the Institute for Clinical and Economic Review value framework are highlighted, providing lessons learned and guiding principles for future efforts to bring measures of affordability within the scope of value assessment.

Keywords: affordability, budget impact analysis, cost-effectiveness analysis thresholds, drug pricing.

Introduction

Introduced for use in 2013, the drug Sovaldi (Sofosbuvir, Gilead Sciences, Inc., Foster City, California) forced health systems around the world to confront a stark contrast between the concepts of "cost effectiveness" and "affordability." The drug was effective: most patients treated were effectively cured of the virus that causes chronic hepatitis C. And, even at an initial list price of $84,000 in the United States, cost-effectiveness analyses by academics and health technology assessment (HTA) agencies concluded that it represented good value for money over the long-term [1,2]. But even though competitive entry and insurer negotiating brought the price down significantly, the large numbers of infected patients needing treatment forced health systems to confront a potentially crushing short-term budget impact. As a result, patient access to Sovaldi in many countries was delayed, and even when made available it was often funded only for patients with advanced disease [3,4]. Apparently, Sovaldi was cost-effective but unaffordable.

This divergence between a drug's cost effectiveness and its perceived affordability will have come as no surprise to those health economists who have long criticized the use of incremental cost-effectiveness ratios as a guide to decision making [5]. They would argue that Sovaldi's incremental cost effectiveness at the time of entry provides little insight into whether Sovaldi represents a wise use of available resources for health care. Other health economists and policymakers, however, believe that cost effectiveness is a useful input to resource allocation decisions and are more likely to see in the case of Sovaldi a demonstration that current thresholds for decision making are unrealistically high, untethered from what should be their empirical source: the true marginal opportunity cost of new expenses within the health system [6]. A final set of observers views Sovaldi as demonstrably cost-effective and therefore a good value for the health system, the problems paying for it being nothing more than a demonstration of the failure of health system managers and governmental leaders to plan ahead so that they can eliminate low-value services and re-allocate the resources to effective new services [7].

Views on the lessons to be taken from Sovaldi therefore differ widely. All observers, however, would agree that since the worldwide economic crisis of 2007 to 2008, the debate over the relationship between cost effectiveness, affordability, and a broader concept of value in health care has greatly intensified. Before that time many health systems in the developed world had become accustomed to adding resources year after year, with budgets usually rising faster than the underlying rate of growth of national economies [8]. With growth assumed, questions about

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whether health systems could afford to fund all new "cost-effective" interventions were relatively muted. But over the past 8 years the landscape has shifted, with health systems around the world now facing strict dictates to shed costs or at best to grow no faster than the national economies in which they are embedded. As a consequence, substantial additional funding can no longer be assumed to be forthcoming to pay for new services. It is within this chastened fiscal environment that the question of whether all cost-effective interventions are affordable has become much more acute.

As a reflection of this new reality, Germany, Japan, and England are among many countries that have recently proposed measures to help moderate spending on new drugs—even those with favorable long-term cost effectiveness—if they exceed or threaten to exceed specific budget impact thresholds [9–11]. In the United States, concerns about rising drug expenditures have triggered a vigorous public policy debate and highlighted the question of how the value of drugs should be assessed. In the absence of a centralized governmental HTA agency in the United States, clinical specialty societies and independent groups have developed several "value frameworks" intended to guide decisions by individual patients and clinicians or to inform price negotiations and coverage determinations by insurers [12,13]. Most of these value frameworks include multiple domains of value, but only one, the framework developed by the Institute for Clinical and Economic Review (ICER), includes a measure of affordability for new drugs within the conceptual core of an assessment of value to the health system. To guide deliberation on affordability, ICER performs a potential budget impact analysis at the national level with a suggested threshold that, if exceeded, signals to policymakers that the amount of added health care costs associated with a new service may be difficult for the health system to absorb over the short-term without displacing other needed services or contributing to unsustainable growth in health care insurance costs [14].

Does this approach create more problems than it attempts to solve? How should information on cost effectiveness and budget impact be considered and by whom? Pressure groups representing drug companies and some health economists have criticized ICER's approach by asserting that information on budget impact should be held apart from any process of evaluating a treatment's value to avoid creating a bias against the long-term perspective on value [15,16]. Given the debate over the ICER value framework, which is set amid the growing global concern regarding drug costs, the goal of this study was to examine the conceptual and practical relationship between cost effectiveness and affordability as components of the assessment of the value of drugs and other health services. Ultimately, this analysis will argue that a measure of affordability is necessary and complementary to the information provided by cost-effectiveness analysis, and that both should be included in any deliberative process intended to support sustainable access to high-value care for all patients.

A Definition of the Affordability of New Health Services

Affordability is not a simple concept, whether within or outside health care settings. As long as the cost for a good or service does not outstrip the entire amount a potential buyer has available, then what constitutes an "affordable" cost cannot be determined empirically [17]. But this does not make it impossible to define affordability nor to design empirical measures of affordability that can inform deliberations on the value of new health services.

The affordability of a new service can be defined most usefully through a negative framing in which affordability is assumed unless funding would cause a net harm through one or more of the following four mechanisms:

1. By requiring cuts or other changes to existing health care services that cause a greater loss of benefits, to the same or different patients, than the corresponding gains anticipated from the new service;
2. By absorbing available new resources that could be spent on other identifiable services that would provide greater overall benefit to the same or different patients;
3. By increasing the amount that must be paid by individual patients to an extent that would require unreasonable individual sacrifice;
4. By requiring the re-allocation of spending from other social goods (e.g., education) or an increase in new revenues (e.g., taxes) that would cause a greater loss of benefits to society than the benefits anticipated from the adoption of the new service.

At the root of all these criteria lies the basic idea of opportunity cost: a new service that adds costs to the health system to help some patients always means that there are benefits foregone to the same or other patients, or to society more generally. The resources available for health are never a truly fixed amount given the ability to shift resources from other spending over time. Decision makers trying to judge affordability need to consider this temporal flexibility but must still make judgments of whether the benefits foregone from spending on a new service are likely to be greater than the benefits to be gained; if so, the service should be considered unaffordable. This does not mean that a health system could not pay for it, just that the health system (or the broader society) would end up "poorer" for having done so. There is some affinity in this approach with the medical maxim of "first, do no harm." We will return later in this article to consider empirical measures that can help identify when the potential budget impact of a new service raises a significant risk of excessive opportunity cost. First, however, we must address the question of whether cost-effectiveness thresholds themselves can serve this purpose.

Can Cost-Effectiveness Thresholds Subsume Questions about Affordability?

There are four basic ways of thinking about how to identify the most appropriate threshold for incremental cost-effectiveness ratios: 1) the willingness to pay (WTP) of a health system (or society) demonstrated by past positive and negative funding decisions; 2) societal WTP based on normative ideals of what a society should be willing to pay for health care; 3) societal WTP based on extrapolation from the answers individuals (either healthy or ill) give when asked hypothetically how much money they would be willing to give up from their own salary to avoid a certain health loss or receive a health gain; and 4) the opportunity cost based on empirical efforts to estimate the cost effectiveness of services already in the health system that would be displaced at the margin if new services are introduced.

Can any of these methods for setting an incremental cost-effectiveness ratio threshold eliminate the potential tension between cost effectiveness and affordability? It may seem self-evident that the first three methods are particularly insensitive to the dynamic between cost effectiveness and affordability. Whether derived from past funding decisions, normative ideals, or individual trade-off decisions, single incremental cost-effectiveness ratio thresholds, or even incremental cost-effectiveness ratio ranges, cannot reflect the scale of spending on different health services given the number of patients involved. Therefore,

to say that a service with an incremental cost-effectiveness ratio below the threshold established through one of the WTP methods is affordable by definition is illogical. Thresholds set by WTP methods cannot serve as a proxy for affordability.

But what about the attempt to actually measure opportunity cost at the margin and use that as the method for setting an incremental cost-effectiveness ratio threshold? Some health economists believe that an empirical effort can identify a threshold (lower than that suggested by WTP methods) that truly aligns with affordability [18]. This approach has strong theoretical roots in the idealized circumstances under which the league table approach was originally proposed to create a basket of funded services for a health system constrained by a certain budget [19]. The major barrier to using opportunity cost approaches to set thresholds has always been the difficulty in actually identifying what is foregone with the addition of spending for new services, and measuring the cost effectiveness of the impact of this opportunity cost. Real-world decision makers certainly do not know the incremental cost-effectiveness ratios of all the services they might reduce or discontinue to make room at the margin for a new service. Harder still is to capture and measure the opportunity cost when resources are made available for new spending through actions such as reducing staff, delaying updates to infrastructure, or lengthening waiting times.

Nonetheless, intensive efforts have now been made in the English National Health Service (NHS), with efforts ongoing in other countries, to estimate the opportunity cost of marginal services by examining trends of spending and health outcomes across jurisdictions that have had different budget constraints [6]. For England, empirical results suggest that the current threshold used by the National Institute for Health and Care Excellence (NICE) is too high, and that a true opportunity cost threshold would be closer to £13,000 than the general £20,000 to £30,000 range commonly cited (Claxton et al. [6]). This work is underpinned by numerous assumptions linking measurable outcomes to marginal spending, a number of which have been challenged as unrealistic by Barnsley et al. [20], and some commentators have concluded that it is premature to use this methodology to change the cost-effectiveness threshold for NICE [21]. But for the purposes of this article, the question remains: Would a cost-effectiveness threshold that accurately mirrored the opportunity cost of services at the margin tell us whether a new service is affordable?

I believe the answer is still "no." Even an empirically robust study of displaced services and their impact on health outcomes would provide only a rough average of the opportunity cost at the margin, an average that would fail to reflect the scale of the budget impact of a specific new service. As Birch and Gafni [22] have argued, the opportunity cost at the margin of any health system is dynamic: as new interventions are funded and others replaced, the marginal funded intervention changes, and therefore the incremental cost-effectiveness ratio of the marginal funded intervention is context-specific. The chances that an opportunity cost threshold would promote funding for an unaffordable service would be low when the potential budget impact is small, but could become considerable for a new drug such as Sovaldi or other treatments that would require substantial new spending. The greater the added cost, the greater the risk that it would displace spending for a package of other services that, in the aggregate, would have provided greater overall benefit to the same patients or patients somewhere else in the health system. Thus, no matter where a cost-effectiveness threshold is set, even using empirical estimates of average opportunity cost, by itself it cannot determine whether the funding of a specific new service would fail the affordability challenge.

Options for Integrating Considerations of Cost Effectiveness and Affordability

If incremental cost-effectiveness thresholds cannot adequately subsume questions of affordability, how should the two concepts be linked in some way to inform decision making? One approach, of course, is to keep them separate by having one deliberative process involved in the determination of value and the other one involved in which affordability is considered. In Australia, for example, evaluations of cost effectiveness are performed by the country's HTA group and then sequentially passed on to government leaders for consideration of affordability before final funding decisions are made [23]. Since its inception, NICE has also had its mandate limited to cost effectiveness [24]. In October 2016, however, with the stress caused by NICE's approval of Sovaldi top of mind, NHS England and NICE jointly proposed a new system through which a projected budget impact for a cost-effective new drug of more than £20 million per year would trigger a "dialogue" between the manufacturer and health system officials, the chief intention being to seek further price concessions and/or delay the timescale for the funding requirement should the budget impact "risk disruption to the funding of other services" [25]. If this proposal is agreed upon, it will represent a major shift by integrating considerations of affordability directly into the NICE methodology of using cost effectiveness to guide funding decisions in the NHS.

Another way to integrate cost effectiveness and affordability is to replace a single cost-effectiveness threshold with a categorical sliding scale, varying the threshold in some proportion to the potential budget impact of a new service [26]. The larger the potential budget impact, the lower the threshold would be so as to reflect the greater amount of current spending that would be displaced to fund the new service.

This recommendation was presented in a briefing paper to NICE in 2008 and deserves further consideration, but to my knowledge it has never been implemented in a national HTA program. This may be because, although in principle it could help align the incremental cost-effectiveness ratio threshold with the potential opportunity cost, several important challenges would remain. First, it would likely prove impossible to determine empirically how much the cost-effectiveness threshold should vary with different levels of potential budget impact. Knowledge of the entire set of services that would be available for trade-off at the margin would be required, and their own cost effectiveness and budget impact determined. Any simple system through which ranges of potential budget impact would be tied to explicit gradations in cost-effectiveness thresholds would be open to attack as completely arbitrary. In addition, the implication of such a system would be that patients with a common illness, one for which a treatment would have a substantial budget impact, would have their "lives" valued at a lower level than patients with less common conditions. Imagine, for example, the problematic ethics and politics of defending a core methodology that implies that the health system is willing to spend $100,000 per added life-year for some patients but only $75,000 for other patients—just because one condition is less common than the other. This is the end result, of course, of many positive funding decisions regarding ultra-orphan drugs, but the challenges of defending what would likely be perceived as an arbitrary sliding scale across all conditions may supersede the theoretical goal of maximizing health benefits within a given health budget.

Although a stark categorical sliding scale for incremental cost-effectiveness ratio thresholds in relation to potential budget impact may not be easy to defend, in theory it remains possible to use broad cost-effectiveness ranges to accomplish the same goal. Thus, potential budget impact (i.e., implied opportunity

cost) could be one of several contextual factors in a value framework used to modulate the effective cost-effectiveness threshold across a broad range, through either a qualitative methodology or a quantitative multicriteria decision analysis technique. Even these approaches, however, would raise difficult questions about whether using budget impact to set the effective cost-effectiveness threshold is equitable and can be done reliably across conditions.

The ICER Experience: Integrating Affordability into Value Assessment

At ICER we have included potential budget impact estimates as part of value assessments of new services, including tests, treatments, and delivery system interventions, since 2010, when public and private insurers began requesting the information to help inform their decision making and to broaden awareness of the challenge of affordability that payers were grappling with.

From 2010 to 2014, our approach was to present hypothetical potential budget impact assessments evaluating the net budget impact if all eligible patients were treated with a new care option. No specific budget impact thresholds were implied, and the independent appraisal committees convened by ICER were not advised specifically on how to integrate the information on long-term cost effectiveness (and other domains of value) with potential budget impact estimates. Committees were asked to verbalize their thinking before taking a single vote on the "comparative value" of a care option using their own judgment on how to weigh different elements of the information presented in the reports and contributed by stakeholder comments during public deliberation.

We began to reconsider our methods after our report and public meeting on Sovaldi and other new drugs for chronic hepatitis C. At this meeting, when given a single voting option on the comparative value of Sovaldi versus the previous standard of care, 11 out of 14 committee members voted "low" value, and their expressed rationales made it clear that the opportunity cost of the potential budget impact was the dominant factor, perceived to be so significant that it overrode what was acknowledged to be excellent long-term cost effectiveness.

To us it appeared that this vote on a single conceptual term representing "value" had not elucidated well the tension between long-term value and short-term affordability. This was part of the motivation behind our decision to launch in early 2014 a formal multistakeholder workgroup to help inform the development of a new and more explicit value framework to underpin ICER reports and public deliberative meetings. In this article I will focus only on the evolution of our thinking on how to measure and incorporate affordability within the value framework.

There was a stark divide within the multistakeholder group from the beginning: manufacturers did not believe that budget impact assessment should be incorporated at all. They viewed consideration of short-term affordability as an exercise that should be done separately by individual payers in the US health care system, each of which would have a different underlying financial structure and circumstances that would give each a unique sense of what was and what was not "affordable."

Consumer groups and insurers, however, were strongly in favor of having affordability made a core element of the ICER value framework. Payers argued that budget impact was central to their efforts to manage value in the health system, and that to leave it out was inconsistent with the reality of the way that value should be assessed. Simply stated, they believed that no intervention that added substantial new costs to the health system should be considered "high value."

Payers and consumer groups also supported introducing a threshold for short-term added costs that would help suggest when the costs were substantial enough to warrant concern about affordability. They fully acknowledged that the true judge of affordability is the individual payer, but they strongly recommended that ICER develop a budget impact threshold. Adopting a threshold was envisioned as a way to signal publicly whenever the potential uptake of a new drug could bring new costs substantial enough to warrant closer consideration of its introduction, whether through tighter utilization controls, distinctive payment mechanisms, or further efforts to reduce the price. There was no illusion that a single threshold should be applicable across different payers, nor was it ever envisioned as a cap on spending for individual drugs. But a budget impact threshold was viewed as helping to convey to the public that affordability concerns were a legitimate part of decision making, even for treatments with good long-term value. And, second, it was hoped that a transparent method for identifying a threshold would prove more defensible than a general argument that a drug's potential budget impact seemed "unsustainable."

ICER Value Framework 1.0

After considering the input from the workgroup, ICER released its value framework in early 2015. This "version 1.0" of the formal ICER value framework retained assessment of affordability as a core element, but, similar to the Australian system, placed it as a separate step after consideration of all the elements required to make an initial judgment of what was termed long-term "care value." The voting on value by the appraisal committees was to be split into two votes, the first being a judgment of care value. After this vote, consideration of potential budget impact—the information with which to judge short-term affordability—was integrated into a second vote on what was termed "health system value." The idea of specifying two different kinds of value was intended to keep the appraisal of long-term value separate and identifiable from that of affordability, while making it clear that no overall judgment on value to the health system could be made without consideration of both.

As part of the potential budget impact assessment, the ICER value framework adopted for the first time a specific threshold. We had explored two different options for how to determine an affordability threshold. The first can be termed "payer ability to pay." We performed a confidential survey of multiple payers in the United States, asking them a question meant to capture the essence of excessive opportunity cost: How much added cost from an individual new treatment could they absorb without requiring an "unsustainable" short-term increase in insurance premiums and/or the displacement or deferment of some other service of greater value? Like other efforts to survey payers to estimate opportunity cost thresholds, we found that payers lacked a clear context for this question and struggled to answer it [27], and we also came to believe that basing a budget impact threshold on private payer's self-reported ability to pay would be unlikely to receive an adequate level of trust in the United States.

The other option we explored, and ultimately settled on, was to set a threshold linked to budgetary growth targets for the entire health care system. With the exception of the Veterans Affairs health care system, no private or public insurance system in the United States operates under a set budget for health care. Nevertheless, two states, Massachusetts and Maryland, had recently passed laws requiring that policy actions be taken to control health care costs should they be found to be growing faster than growth in the state gross domestic product (GDP) [28,29]. Similarly, at the federal level, the Affordable Care Act had called for an independent advisory board to take action to reduce costs in Medicare if annual costs grew faster than the national

Table 1 – Calculations deriving a potential budget impact threshold for new drugs in the US during 2015-2016.

1	Growth in US GDP, 2017 (est.) +1%	3.75%	World Bank, 2016 [33]
2	Total personal medical health care spending	$3.08 trillion	CMS NHE, 2016 [34]
3	Contribution of drug spending to total health care spending	13.3%	CMS NHE, 2016 [34]; Altarum Institute, 2014 [35]
4	Contribution of drug spending to total health care spending	$410 billion	Calculation (Row 2 x Row 3)
5	Annual threshold for net health care cost growth for ALL drugs	$15.4 billion	Calculation (Row 1 x Row 4)
6	Average annual number of new molecular entity approvals	34	FDA, 2016 [36]
7	Annual threshold for average cost growth per individual new molecular entity	$452 million	Calculation (Row 5 ÷ Row 6)
8	Annual threshold for estimated potential budget impact for each individual new molecular entity	$904 million	Calculation (doubling of Row 7)

GDP plus an additional 1% [30]. This general approach of setting an affordability threshold consistent with growth in the national economy was favored by outside health economists we spoke with and supported by insurers and consumer groups. We selected the more generous target of GDP + 1% and constructed a set of calculations on the basis of publicly available data to set a threshold for the potential budget impact of individual new services that would be likely to contribute to overall health care cost growth exceeding the GDP + 1% target (see Table 1). Our value framework had targets associated with both drugs and devices, but for the purposes of this article only the details related to drugs will be discussed.

As described earlier, the express purpose of this threshold was not to suggest a budget cap for an individual new drug, nor for spending on all drugs as a proportion of overall health care spending. Instead, the goal was to have a consistent and transparent method for determining when policymakers should consider the possibility that special measures—such as prioritizing patient access, re-allocating other resources, price reductions, or extended payment options—would be needed to manage the tension between a new drug's long-term value for money and its short-term affordability. To serve this purpose, the following assumptions and methods were used to guide the calculation of a specific threshold for an individual new drug (see Table 1):

1. New spending for health care should not exceed the anticipated growth in the national economy (GDP + 1%) and will be evenly shared among existing categories of spending; that is, the contribution of health care spending attributable to drugs will remain the same as expenditures for health care increase.
2. Potential budget impact analysis will examine net budget impact, including consideration of cost offsets in all areas of health spending, and will reflect the net difference in spending for patients switching from previous care options to the new drug.
3. The entire growth in spending on drugs to meet the GDP + 1% target will come from spending on new drugs; that is, all "background" spending on existing drugs, with its mix of increased expenditures for some and decreased expenditures for others, will have a net neutral budget impact.
4. The number of new drugs anticipated in the next 1 to 2 years will be an average of the preceding 2 years.
5. Uptake assumptions used as part of an estimate of potential budget impact analysis will be based on the "unmanaged" uptake of new drugs. Because the goal of the analysis is to suggest when the budget impact could be substantial enough to warrant policies such as tight utilization management, this assumption of unmanaged uptake would be based on estimates of clinician and patient demand and not be constrained by potential insurer policies.
6. The time horizon for potential budget impact would be 5 years, with the peak uptake assumption reached at the completion of year 5. Payers in the United States operate under situations requiring 1- to 2-year budget horizons, but manufacturers sought a longer time horizon to capture more potential cost offsets from new treatments.
7. On the basis of these assumptions, an average net budget impact can be calculated for each new drug that would absorb all new drug spending within the GDP + 1% target. This average net budget impact would not, however, be the most helpful threshold for policymakers because too many drugs would have a potential budget impact greater than the average. Instead, because the goal is to focus policy attention on those drugs with the greatest chance of excessive opportunity cost, a more useful threshold is one set at twice the average budget impact that would consume the entire amount of new spending.

For 2015 to 2016 our calculations produced a threshold for potential net budget impact of $904 million per year, annualized over the 5-year time horizon. Adopting this kind of single number would be more relevant in a single-payer system, and the threshold is obviously not the specific potential budget impact figure that would be used by any individual payer in the United States. Payers will differ in their budgetary flexibility and in the spectrum of employers and other purchasers they represent, all of whom may have different views of affordability. Another important limitation of designing a budget impact threshold for individual drugs is the problem of accounting for the ebb and flow of the number of drugs within calendar years. If in a single year there happen, by chance, to be multiple drugs with potential budget impacts of $900 million, none would exceed the threshold, and yet cumulatively they could pose a substantial risk to affordability. Other years with fewer blockbuster drugs might have more room in health care budgets. All these limitations highlight that any budget impact threshold used by an HTA group to signal the need to consider affordability must be considered within a broader context and not applied to pricing in an automatic fashion.

Experience and Revisions for ICER Value Framework 2.0

ICER's value framework using this new approach to potential budget impact analysis with a threshold was first applied simultaneously to reports produced in September 2015 on two new drugs for high cholesterol and one new drug for heart failure [31,32]. Including these first reports, as of February 2017 the ICER has used its first value framework to assess a total of 21 new drugs used for 11 different conditions, including expensive new drugs for multiple sclerosis, lung cancer, asthma, and diabetes. As it has turned out, only the first three drugs evaluated for high cholesterol and heart failure had potential budget impact estimates that exceeded the threshold. For example, the cholesterol drugs, known as proprotein convertase subtilisin/kexin type 9 (PCSK9) inhibitors, were found to have poor long-term cost effectiveness, requiring approximately a

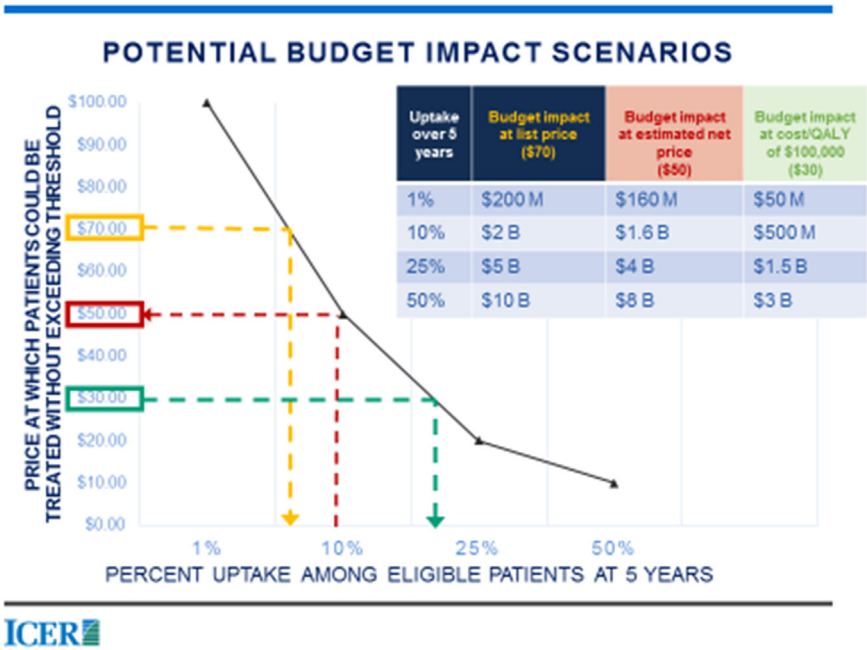

Fig. 1 – Revised ICER value framework version 2.0 approach to potential budget impact analysis. The black line indicates when the potential budget impact threshold is reached at each combination of price and percent uptake among eligible patients at 5 y. The figure allows stakeholders to use their own assumptions for price and uptake to determine the estimated net budget impact of a new drug in relation to the threshold, which for 2017–2018 is $915 million per year annualized over the 5-y time horizon. ICER, Institute for Clinical and Economic Review; QALY, quality-adjusted life-year.

50% discount from their average wholesale acquisition cost to achieve a WTP of $150,000 per quality-adjusted life-year. At the wholesale acquisition cost, and assuming a 5-year uptake assumption of 20% of eligible patients, the drugs would have required an even deeper 85% discount in order not to exceed the potential budget impact threshold.

Despite the fact that only the first three drugs evaluated were estimated to exceed the potential budget impact threshold, experience using the value framework and public comment from stakeholders identified two key issues:

1. The label of "health system value" as a term representing an integrated consideration of long-term cost effectiveness and short-term affordability was resisted by some health economists and adamantly opposed by manufacturers. Despite support from insurers and some policymakers, many felt that the incorporation of potential budget impact as an element in health system value seemed to give it precedence over long-term cost effectiveness.
2. The concept of the "unmanaged" uptake assumption was proving difficult to communicate, leading many stakeholders to confuse our (higher) estimates with what might be assumed to be "real-world" uptake figures when factoring in insurer coverage limitations. Manufacturers felt that unmanaged uptake assumptions falsely inflated potential budget impact figures in a way that unfairly stigmatized new drugs.

Given these concerns, as part of a planned update to the ICER value framework for 2017 to 2019, we decided to revise our framework to replace the term "health system value" with a graphic representation showing that two elements—"long-term value for money" and "short-term affordability"—must be considered to achieve a common goal: "sustainable access to high-value care for all patients." In addition, we will no longer attempt to estimate the uptake of a new drug as part of a specific estimate of budget impact. Instead, going forward, ICER will present information in a graphic format that will allow stakeholders to ascertain the potential budget impact of a new service according to a wide range of assumptions on both price and uptake. A general example is shown in Figure 1. Importantly, as part of this new approach, ICER will continue to use a threshold for potential budget impact linked to growth targets for the national economy so that policymakers will be able to know when a certain combination of price and uptake would lead to a heightened concern regarding affordability.

ICER will note in its reports the percent uptake of a new intervention, at its estimated net price in the US market, that would produce a potential budget impact that exceeds this threshold. In addition, ICER will include as part of its final report an "affordability and access alert" if discussion among stakeholders at ICER's public deliberation meetings suggests that utilization driven by clinical need, at estimated net pricing, would exceed the budget impact threshold without active intervention by insurers and others to limit access to the treatment. The purpose of an ICER affordability and access alert will remain consistent with the overall purpose of the potential budget impact analysis: to signal to stakeholders and policymakers that the amount of added health care costs associated with a new service may be difficult for the health system to absorb over the short-term without displacing other needed services or contributing to rapid growth in health care insurance costs that threaten sustainable access to high-value care for all patients.

Conclusions: Why Both Cost Effectiveness and Budget Impact Belong in a Value Assessment

In the United States and in many other countries, the assessment of the value of drugs and of nearly all health care services is dominated by considerations of safety, effectiveness, and budget impact, and not by formal measures of long-term cost

effectiveness. There is a logic to this perspective but also obvious perverse outcomes.

Budget impact is a reasonable consideration for private insurers because they work in rapid cycles with purchasers and individual subscribers, translating short-term cost projections into planned insurance premiums for the coming year. Rapid cost growth in the short-term, especially when it increases beyond anticipated inflation rates, pushes quickly upstream to purchasers and policymakers who have to make their own short-term decisions about how to find the needed resources.

Even public insurers, who among all payers should take the long-term view, often face short-term budget realities that dominate decision making. In the United States, for example, state governments faced with high short-term costs for a new treatment such as Sovaldi, with excellent long-term value for money, might be forced to consider unpalatable options such as raising taxes or reducing the education budget to find the funds to pay for the new treatment. The "affordability imperative" can be paramount.

Certainly, the perverse influence of an undiluted focus on budget impact cannot be overstated. A narrow short-term perspective blinds policymakers and insurers to the need to forge efforts to reshape the delivery system and reframe payment mechanisms to make room for new and potentially expensive interventions that will help patients and pay off in the end. Therefore, if an economic analysis of new interventions is focused only on the short-term, relying solely on budget impact estimates, patients and the health care system will be the ultimate losers.

But health systems and nations cannot make advances in managing the potential tension between long-term value for money and short-term budget impact by keeping budget impact assessment out of value frameworks and value assessment reports. The idea that having analyses of long-term value and budget impact in the same report will somehow taint decisions can only be imagined if budget impact were not already dominating the playing field. The ICER value framework includes budget impact analysis because leaving it out would only reinforce its silent power over too many decisions.

Thus, the ICER value framework carries in its basic structure the conviction that keeping budget impact considerations off the table, to be factored in only post hoc by insurers or delegated clinical systems in ways unknown, would be a mistake. There is no empirical formula by which budget impact can modulate considerations of long-term value for money, but keeping budget impact analyses too far from value assessments would rob nations of the chance to bring the public directly into the critical discussions about health care and health insurance that are needed to achieve sustainable access for all patients to the kind of innovative new tests, treatments, and delivery system interventions that add value to their lives.

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