

Seminar Briefing 16

A Framework for Payer Assessment of the Value of New Technologies: A US Approach

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1. Background

In the US, nearly all drugs are funded for all relevant FDA indications. Prices for these drugs are set at the discretion of manufacturers with standard percentage discounts for a number of public insurers. Clinicians have little accountability for the quality of treatments they provide and even less accountability for their financial impact.

To protect against overuse, insurers manage access to medicines in a number of ways. They employ patient cost-sharing methods (such as co-payments), using tiered formularies in which tier placement is determined largely by price and not by value for money. Often, they also delegate the drug delivery side to third-party administrators called pharmacy benefit management companies, which are responsible for negotiating discounts and rebates with manufacturers.

In addition, insurers may require prior authorisation as a means to restrict use of medicines beyond FDA indications. Insurers also use step therapy ("fail-first") policies to guide therapy, where a less expensive drug must be tried first, and only if the clinical response is inadequate will coverage be provided for a second, more expensive, option.

Despite these measures, payers in the US are becoming less able to pass on cost increases – from

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any source – to patients or purchasers. The most important payer, Medicare, is prohibited from considering costs and no dominant approach to judging value exists between private payers or state Medicaid programmes.

This has resulted in an ongoing disconnect regarding the view of value between payers and manufacturers. The current scientific and business model for manufacturers is trending towards more high-cost drugs. Payers often do not believe that these drugs offer good value to the health care system, whereas manufacturers worry that payers will tighten their unclear evidence standards even further and use existing policy tools to restrict access.

There is political and policy gridlock in the US: comparative effectiveness research is hobbled by exclusion of the consideration of costs. Early efforts of physician specialist societies, such as the American College of Cardiology and the American Society of Clinical Oncology, have been met with some resistance. There has also been an attempt at public shaming over the cost of Sovaldi (sofosbuvir), a highly effective but high-cost drug for hepatitis C.

In order to address this conflict of interest, the Institute for Clinical and Economic Review (ICER) has developed a “value framework”. The long-term goals of the framework are to improve the reliability and consistency of value determinations by payers and to provide the basis for more transparent dialogue between manufacturers, payers and other stakeholders over considerations of value.

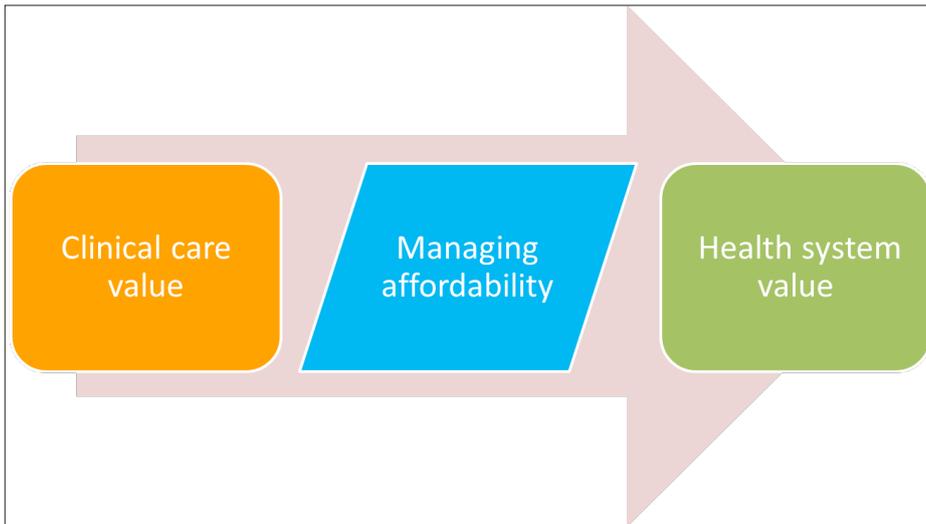
2. The ICER Value Framework project

The framework as presented here today is very much still a work in progress, and I expect the final version will differ, perhaps substantially, from the model I will present today. The framework includes a list of value elements to consider, methods to measure or judge each element, and a process by which to integrate these measurements and other information into an assessment of overall value.

It was informed by input from the ICER Policy Development Group, which involves representatives from insurers and pharmacy benefit management companies (including OmedaRx, Kaiser Permanente and WellPoint), patient organisations (FamiliesUSA), purchasers (Marriott and Maine Health Management Coalition) and manufacturers (including Lilly, GSK, Amgen and Merck).

The framework splits the conceptual idea of “value” into two components: clinical care value and health system value – which are discussed individually below. Once clinical care value has been established, payers and manufacturers can work together to *manage affordability* and thus obtain health system value, as depicted in Figure 1.

Figure 1. Elements in a payer assessment of value

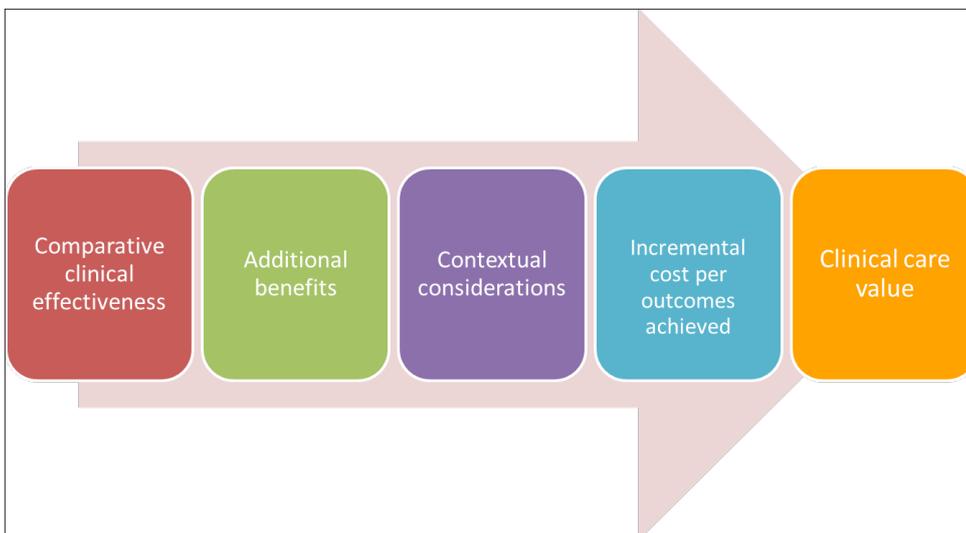


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2.1. Clinical care value

The ICER value framework proposes that clinical care value is a judgement based on five elements (Figure 2).

Figure 2. Elements in a payer assessment of value: clinical care value



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The first element of clinical care value is **comparative clinical effectiveness**. This can be assessed by measuring the magnitude of the *comparative* net health benefit, which includes judgements regarding the importance of the outcomes measured and how patient-centred they are. Comparative clinical effectiveness can be measured using disaggregated outcomes (specific clinical outcomes, e.g. disease-specific mortality) or aggregated outcomes such as quality-adjusted life years (QALYs).

The degree of certainty in the evidence on the net health benefit of the health technology is also important, but incorporating it explicitly and transparently remains a challenge. At ICER we have produced an online tool to assist formulary decisions that guides users to rate the magnitude of overall net health benefit and the level of certainty in the evidence. This “ICER EBM matrix” is now being used to train pharmacists in the US on how to review evidence and how to create an internal dossier.¹

We now move to the second element of clinical care value: **additional benefits**, where these are defined as potential benefits other than those considered part of clinical effectiveness. Valuing additional benefits is a common challenge among all HTA groups and is something that NICE has been struggling with in the UK.²

During discussions regarding additional benefits with American payers and other members of the Policy Development Group, the following key questions emerged:

- Are there benefits of the treatment that extend beyond patient-specific health improvement – for example, reduction in the care needed from friends and family, earlier ability to return to work?
- Will the treatment expand the population that will benefit from treatment – for example, allowing sicker patients or those with comorbidities to be treated?
- Does the treatment offer a new or different mechanism of action when many patients do not achieve adequate outcomes on other treatments?
- Are there other practical advantages related to preparation, storage or delivery of the treatment?

Contextual considerations, the third element of clinical care value, actually affect every other element. For instance, what if no other acceptable treatments exist for this condition? This can change the entire behaviour of the payer and significantly influence the coverage decision. Contextual considerations – for instance, high-severity diseases or priority conditions – also include ethical decisions and social values. There might also be cases where the treatment affects vulnerable populations, such as children, although defining these populations can often be challenging. In addition, it is necessary to include some practical contextual considerations such as consensus among professional statements on appropriate use (formal guidelines, for example).

Let us move on to **incremental cost for outcomes achieved**. The UK is a very empirical society. In the US, we have that as part of our tradition but this becomes problematic when it is defined too explicitly. Therefore, in trying to figure out how to bring cost-effectiveness into the US system, we have found that it helps if you talk more about the cost per *clinical outcome* (as opposed to QALY), at least to start with.

If you want to compare two drugs that were meant to reduce stroke risk, for example, you could ask, “How much does each cost to prevent an additional stroke?” and calculate the incremental cost

¹ See www.CERcollaborative.org.

² In 2014, NICE produced a consultation paper proposing changes to the Health Technology Assessment (HTA) process that would incorporate additional measures of value. See <http://www.nice.org.uk/Media/Default/About/what-we-do/NICE-guidance/NICE-technology-appraisals/VBA-TA-Methods-Guide-for-Consultation.pdf>.

per outcome. However, it is quite rare to be able to base the entire comparison on a single clinical outcome and ignore all the other potential side effects and things that would be considered in summing up to a QALY. Measuring the incremental cost per clinical outcome sounds good in theory but often breaks down in practice.

There is some understanding in the US that, if we are going to seriously consider cost-effectiveness analysis, we are going to need to use QALYs in some way. But this is still at an early stage: if you try to assign a threshold, as you have heard from Peter Newman, Milt Weinstein and others, I have found that you get a lot of pushback that this is “just not the way we do it in the US”.³ Nevertheless, I am trying to help payers see that they need to use cost-effectiveness and not just budget impact. Most payers really do not think about cost-effectiveness in a clear, transparent fashion.

2.2. Health system value

Health system value deals with the issue of affordability for the health system, which is driven by the implied risk of opportunity costs and impact on sustainable access to health insurance. One must consider the budget impact on the organisation as well as the impact on overall health care costs measured by potential impact on insurance premiums.

A key issue is capturing the budget impact of treatments that offer long-term benefits but come with high up-front costs. In my discussions I have not found one payer in the US that uses forecasting models that extend beyond three years, and most use only one-year models. There are practical reasons for this: for most private health plans in the United States, the average length of time that they will have a person on their health plan is between three and four years. Most individuals will move on to some other health plan in this time, meaning that many payers do not capture that long-term benefit.

2.3. Managing affordability

I put “managing affordability” as an action step in Figure 1 because I wanted to frame it so that it would be viewed as a joint challenge: if we have high clinical care value we need to figure out steps to bring it through into the health system with high health system value too.

That could be done by changing the payment mechanism. There is currently a lot of talk in the US about stretching out payment terms for expensive new drugs, so instead of paying for everything now, insurance companies would pay for it on an instalment basis over five or ten years. Another option for payers is to prioritise populations to reduce the immediate cost impact. They could – and others could help – find savings in other areas to overcome this investment problem, or they could share costs with the government or other funders or patients.

³ See <http://www.nejm.org/doi/full/10.1056/NEJMp1007168>.

3. A value flowchart for payers

The value flowchart (Figure 3) was created to demonstrate how the elements of clinical care value and health system value can be strung together in a way that is structured and transparent from the payer prospective. In using the value flowchart, a payer would move from left to right, putting the elements together to come up with a value rating of clinical care value, and from there to managing affordability and health system value.

Figure 3. Value flowchart for payers



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Figure 4 below shows three conceptual examples, which all have high clinical care value and high health system value. In the first example (row 1), the comparative clinical effectiveness of the new drug is judged to be superior to the old drug. In that case, additional benefits are somewhat less important, as are the contextual considerations. Let us assume this superior drug has a cost per outcome that is either below the comparator or below a threshold – I have used \$100,000 per QALY because it is a commonly cited number in the US context. Therefore, there is high clinical care value (see column shaded red).

Figure 4. Conceptual examples using the value flowchart

High clinical care value <i>and</i> high health system value:							
	Comparative clinical effectiveness	Additional benefits	Contextual considerations regarding the illness and therapy	Incremental cost per outcomes achieved	First value rating: "Clinical care value"	Managing affordability	Second value rating: "Health system value"
1	Superior	Less important	Less important	Below comparator or threshold (\$100K/QALY)	High	Can be brought below threshold (0.5-1% PMPM)	High
2	Incremental	Important	Important	Below comparator or threshold (\$100K/QALY)	High	Can be brought below threshold (0.5-1% PMPM)	High
3	Comparable	More important	Important	Below comparator	High	Can be brought below comparator (0.5-1% PMPM)	High

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The next stage of the process is to ask the question, do we need to use our policies to adjust either the price or either the payment mechanism in order to manage the budget impact? The term "PMPM", which stands for "per member per month", represents the insurance premium. In discussions with payers and others, we have found that new drugs whose budget impact raises overall costs by more than half a per cent (0.5% PMPM) cause alarms bells to go off. For payers, managing new costs beyond this level for an individual therapy raises the spectre of some sort of destructive opportunity cost. Certainly if it is below that threshold and it is of high clinical care value it can be judged of high health system value.

Alternatively, what if the new drug is only incrementally better in terms of comparative clinical effectiveness (row 2)? Here, additional benefits are more important to consider but I do not define this quantitatively. The value framework is designed to make judgements about the importance of potential additional benefits more transparent and explicit. In addition, contextual considerations become more important if the comparative clinical effectiveness is only incrementally better than other options. To get high clinical care value, you still need to be below the comparator or the threshold.

Finally, if the drug is equally effective but also less expensive, it can demonstrate high clinical care value (row 3), and if it can be brought through with a budget impact below a particular threshold, it also has a high health system value.

Let us move on to the real-world example of Sovaldi, used for the treatment of hepatitis C, compared to previous “triple” therapy (see Figure 5). I will simplify the actual numbers involved to make the conceptual use of the value framework easier to follow. Regarding comparative clinical effectiveness, the viral cure rate for Sovaldi is 90%, versus 70% using the previous therapy. One could argue that the drug also has additional benefits in the form of the much shorter duration of treatment. It does, in some sense, involve vulnerable populations, such as HIV co-infected patients. In the economic model that we developed, the cost per additional sustained virological response (SVR) was \$100,000, and the analysis from the National Institute for Health and Care Excellence (NICE) showed that the cost per QALY gained was less than \$50,000 for many populations. Thus, one would judge the clinical effect to be superior and, as a result, additional benefits and contextual considerations become less important. The incremental cost per clinical outcome was below the comparator and also below our stipulated cost/QALY threshold, so one would judge that the clinical care value of Sovaldi is high.

Figure 5. A test case: Sovaldi versus previous triple therapy

Comparative clinical effectiveness	Additional benefits	Contextual considerations regarding the illness and therapy	Incremental cost per outcomes achieved	First value rating: “Clinical care value”	Managing affordability	Second value rating: “Health system value”
SVR 90% vs. 70%	Shorter duration	1. Vulnerable populations 2. Professional guidelines encourage use	Cost per SVR = \$100K Cost per QALY < \$50,000		Rx for all known diagnosed would increase drug budgets by >10% PMPM by over 15% in first year	
Superior	Less important	Less important	Below comparator or threshold?	High	Can be brought below threshold?	Low if unable to modulate budget impact High if can reduce short-term budget impact

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This leaves the question of affordability. When I ran this by a public panel in California, where each member had a single vote for high or low value, they voted almost unanimously that Sovaldi was low-value despite being clinically superior. If I could put words in their mouths, I would say that it was because they were “internalising the implied opportunity cost of the budget hit that payers, especially Medicaid programs, were confronting”. I would say that if we were to remain unable to modulate the budget impact, they were not necessarily incorrect to call it of low health system value. On the other hand, if we could figure out a way to reduce the short-term budget impact I think Sovaldi could be considered of high health system value.

4. Conclusions

4.1. ICER framework versus NICE

If I had to stereotype the ICER value framework, I would call it a “categorical, part quantitative, part qualitative, approach”.

NICE, on the other hand, uses continuous relative effects with the potential for internal quantitative weighting and/or some discretion for the consideration of social values at the margins. I was always fond of watching NICE Appraisal Committees and I always felt they did a majestic job of not only looking at the numbers, but also thinking about the bigger picture and talking about social values. I understand the tension between qualitative wrestling with broader social questions and how it should factor them in, and the desire to achieve consistency and reliability by using a quantified, empirical process.

In some ways, these approaches are clearly not mutually exclusive. There is always a decision to be made about choosing to be quantitative or just showing the steps through which a decision group would have to go.

In trying to compare the relative merits of the ICER framework and the way NICE does it, I came up with six distinct attributes, discussed below.

First, are the frameworks **comprehensive in addressing multiple elements of value**? I think NICE’s is as good as ICER’s in trying to capture all the different elements, but having a different bucket called “additional benefits” has been popular with manufacturers and patient groups. The payers are always telling us that only clinical outcomes matter, when we all know that it is more than that – so somehow having a visually identifiable place to put additional benefits helps.

Second, are the frameworks **consistent across payers**? Mine is absolutely not and here I want to mention again part of the background. NICE and most agencies obviously are representative of a nation and consistency is one of the most integral principles that they are seeking to achieve. In the US, we accept that we have multiple payers and we know that these comprise non-profit companies, for-profit companies, regional companies and national companies. We do not even begin to assume that we are going to make the same decisions all the time. For example, it would not make the news if Aetna covers a treatment and WellPoint does not.

Third, are the frameworks **consistent across conditions**? Unless you have a cost-per-QALY approach, you are not going to be consistent. Therefore, this is really not something that the ICER framework can pretend to do. I think that in general, given NICE’s remit, it has to be consistent across conditions, and so that is an important difference.

Fourth, are they **transparent**? I do not think that this framework – certainly to the degree that I have developed it so far – is as transparent as NICE’s has always been and continues to be. This is something that I always tell people in the US: do not get mystified by the cost per QALY, go and

watch how they present their rules of the road and how they work with stakeholders and how they make everybody feel like you understand when it is happening, what is happening and what will happen next. NICE does it better than anybody.

Fifth, how well do the frameworks **address affordability**? My framework certainly does – some people would still say too explicitly. I am still getting a lot of pushback from pharmaceutical manufacturers who say that value does not include the concept of affordability. However, in the ICER framework it remains a distinct consideration as part of value.

Finally, to what extent are the frameworks **“one-size-fits-all”**? My framework certainly is not – it is meant to be malleable. A different payer could decide to give different weights to different additional benefits. I hope it meets some middle ground where it looks more structured and more transparent and will help payers justify both internally and externally some of the decisions they make. NICE, for both good and ill, has a very structured approach.

4.2. Thoughts for the US

I mentioned that, today, if you go to the US and you talk to payers, they will tell you that what they are worried about is next year’s budget impact. They also talk a lot about clinical effectiveness.

Manufacturers in the US talk a lot about comparative clinical effectiveness and they spend a lot of time trying to hone a value argument that is linked to additional benefits. That is where some of the friction with payers comes in because manufacturers will talk about mechanism of action or something else that is outside the box compared to clinical effectiveness, and communication does not always go well. Manufacturers will talk about cost-effectiveness as long as it is not used, especially by the government, and they just do not think that affordability should be part of their problem.

The hard part is that manufacturers really do understand the payers’ problems with affordability. The concern that manufacturers have repeatedly expressed to me is that the solution is too often considered to be price reductions. All of the other possible ways of managing affordability somehow never really get used and they think that is not fair.

My goal is to convince US payers to integrate cost-effectiveness thinking more into the way that they actually make their assessments and I do hope to be able to encourage manufacturers to think of affordability as a joint challenge and to figure out a more creative set of policy tools than they currently have. Public shaming is not going to work, so we need to think of other tools that we can use to achieve high health system value alongside high clinical care value.

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