What Do Value Assessments Miss?

Rethinking Value Using New Health Economics Evidence

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A PRECISION BRIEF

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Now more than ever, market priorities are shaping the core activities of pharmaceutical firms, device companies, health technology assessors, policymakers, and researchers. As drug prices continue to climb and novel therapies challenge traditional pricing models, we are seeing increased momentum behind value-based care and value-based reimbursement. In this white paper, I discuss the key drivers of the value conversation and explore how the definition of value goes beyond quality and cost of care to include other dimensions that should be factored into developing value frameworks.

Factors Contributing to the Value Conversation

Transformation in the Standard of Care

Over the past 50 years, the way that medical care is organized has changed dramatically. We are living longer, healthier lives, in large part due to technological advances available to hospitals, physicians, and other healthcare providers to help improve patient outcomes and quality of life. These technologies have transformed the current standard of care, even compared with 10 years ago.

Materialization of Curative Treatments

Another feature of the healthcare market today that distinguishes it from the one of the past is increased investment into and realization of cures for certain diseases. Historically, our approach to managing most diseases has been to try to prevent the complications associated with those diseases. In the past decade, however, we have seen the approvals of treatments that increase the cure rate of hepatitis C virus (HCV) while reducing the side effect profile. We are starting to see investments—and the fruits of those investments—that offer a chance of a cure for diseases such as thalassemia and sickle cell disease. We have also seen the approval of gene therapies for patients who previously had limited or no treatment options.

The Need for Justification of Price and Value

With the advent of these breakthrough therapies, the key question has become how to help patients access these treatments. Many of these new technologies are expensive. Consequently, sponsors are challenged with justifying the price of treatment and determining how to articulate value. Before sponsors can even begin thinking about pricing and value, they need to understand how the efficacy of the drug or technology compares with other treatments. Typically, the effectiveness of a treatment is evaluated by evidence developed from randomized, controlled clinical trials (RCTs). However, for many technologies, it is unknown whether the surrogate endpoint studies in an RCT will materialize in the real world. In fact, for many therapies, what is observed in an RCT may not generalize to the population. There may be subgroups for whom a treatment is particularly effective, and there may be other subgroups for whom the treatment is particularly ineffective. Thus, thinking about real-world treatment effectiveness and novel ways to assess that effectiveness has become a core activity in today's market.

The Debate on Value

Sponsors must also keep in mind the public policy debate that centers around how we should be valuing new medical technologies: What do patients value? What does society value? How should we be aligning the prices of new drugs, devices and other technologies with those values, especially if those values differ?

In economics, value represents what a consumer is willing to pay for a particular product. For example, assume a person is willing to pay $1,000 for a smartphone, but the purchase price is only $500 and the cost to manufacture it is $100. The total value of that smartphone per person in society is $900, the difference between what a person is willing to pay and what it costs to manufacture it. That $900 surplus is divided between the consumer, who gets a $500 surplus (the difference between what they are willing to pay and the purchase price), and the manufacturer, who gets a $400 surplus (the difference between the purchase price and the manufacturing cost).

Value can even extend beyond the individual who consumes the product. In the case of antibiotics, the person who is sick benefits directly from the treatment. People who are prevented from getting the infection also benefit, even though they are not consuming the product itself. This is just one example of how value to society may differ from how an individual values a technology.

To keep it simple, for the purposes of this white paper, assume that the value of a treatment or technology is based on what a consumer (ie, a patient) is willing to pay for it. The division of surplus matters. In the smartphone example, the value to society was $900, with $500 of the surplus going to the consumer and $400 going to the manufacturer. Is this the right split? And how does surplus division apply when looking at healthcare technologies? Should we be dividing more favorably towards patients versus manufacturers/innovators? How will the value division impact patients today and patients in the future?

In our analysis of treatments for cancer, HIV, and a host of other diseases, we have found that patients capture approximately 80% of the value of new healthcare treatments. Most of this is because health is very valuable to individuals. Although drugs, devices and new technologies can be expensive, the net value is still very positive to patients. This analysis excludes treatments that have become generic, as nearly all of the value of generic drugs goes to patients because manufacturing costs are driven down to marginal costs of production. In addition, over time, most technologies experience reductions in price, which makes things more valuable to consumers over time.

The Need for More Comprehensive Assessments of Value

Against this backdrop, there are a number of reasons why it is important to accurately assess the value that new healthcare treatments and technologies provide:

1. Healthcare competes for other societal resources, such as defense, education and infrastructure. To make informed decisions about resource allocation and return on investment, you have to know what you are spending money on and how you value it.
2. Value assessments make it possible to identify areas of low-value care (eg, unnecessary diagnostic tests, inappropriate antibiotics, unnecessary hospitalizations, etc), so resources can be reallocated from low value care to high-value care, improving population health at the same total cost.
3. Many high value services (eg, curative treatments for hepatitis C) are products of innovation. Reallocating of resources can increase not only returns to patients today, but also the likelihood of downstream innovation. Studies have shown that if the expected profits resulting from innovation are reduced, we will see less innovation. The question always is whether this foregoing of innovation matters to patients or not.

How should we be valuing new healthcare technologies?

A Modern Approach to Value

The traditional way of measuring value in healthcare is by measuring the quality-adjusted life years (QALYs) that one technology brings relative to another and applying a monetary value to each QALY to compute value. Traditional value assessments have included the health benefits of new technologies as well as their effects on cost and productivity. In essence, these assessments were designed to reflect value to individuals.

However, traditional value assessments miss some important dimensions that can have a significant impact on overall value (see Figure 1):

Insurance value

This value represents what a person would be willing to pay for an insurance policy that insures them against the health and financial risk that comes with developing an uncommon disease for which the treatments are very expensive. Put differently, individuals may value a treatment even if they never need to utilize it simply because they value that treatment being available to them should they, or a loved one, need treatment. The same is true for home fire insurance. Most people's homes fortunately do not burn down but insurance against this possibility is valuable to all homeowners.

Value of hope

Patients with a serious disease may be more willing to take risks than the general population. For example, when given a choice between Therapy A, which guaranteed 24 months of additional survival, and Therapy B, which offered a 50% chance of 10 months of additional survival, a 30% chance of 24 months of additional survival and a 20% chance of 50 months, 71% of patients with cancer preferred to gamble on Therapy B. Value frameworks that focus on mean or median improvements in survival may miss the fact that some patients, perhaps most, highly value the possibility of a long-term treatment response.

Option value

To illustrate, when AZT was approved in the late 1980s for the treatment of HIV, it was only modestly effective and was associated with considerable side effects. However, there was a subgroup of a patients who took AZT who were able to live long enough to be treated with more effective, better-tolerated treatments that emerged in the mid-1990s. In this scenario, AZT offered an "option value" to some patients. We are not yet at the point where option value can be explicitly factored into value frameworks and health technology assessments, as it could be argued that nearly every treatment has an option value to some patients. Nevertheless, it is important to understand that option value is a critical component of value. For example, Precision Health Economics recently performed an analysis on tyrosine kinase inhibitors (TKIs) for cancer and estimated that the option value is about 10% of the conventional value estimated from traditional cost-effectiveness models.

Spillover value

How families and society benefit from treating the individual. As mentioned before, antibiotics can have a spillover effect by preventing infections in individuals other than the one being treated. Treatments for mental illness can have a spillover effect on disability, crime and caregiver burden. Effective treatments for dementia, which currently do not exist despite large R&D efforts, will have potentially large spillover effects on family members and loved ones who care for patients with dementia.

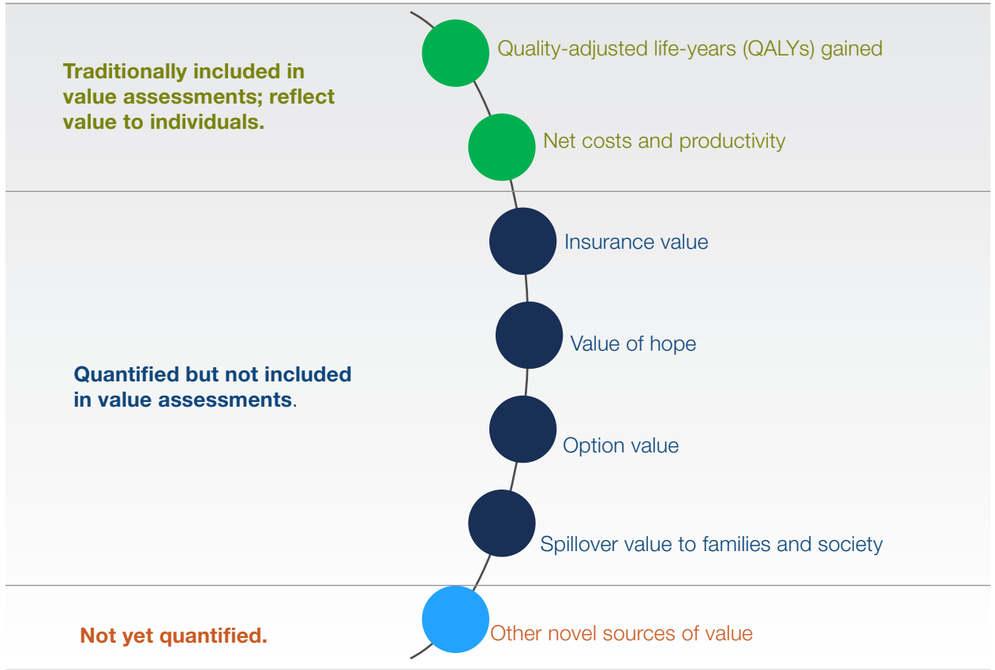

Figure 1. Where Is Value Created?

Calculating Unanticipated but Predictable Benefits

Some components of value are easy to understand and anticipate, while others—such as option value—may be difficult to anticipate. Another example can be illustrated by HCV and liver transplantation. HCV is the number one reason for liver transplantation. If we are able to cure the population of HCV, fewer people will develop end-stage liver disease or require liver transplant. Because end-stage liver disease and liver transplantation are both very costly, eliminating HCV in an individual is a cost savings to society.

However, the drugs are expensive and must be paid for as an upfront investment. This component of value is very well understood and the challenge lies in determining the dollar value for the benefit of eliminating the need for end-stage liver disease treatment and liver transplantation in patients with HCV.

However, there is another component of value associated with the reduced need for liver transplantation among patients with HCV: the increased availability of donor livers in the transplant pool has a spillover effect on patients who require liver transplantation for non-HCV-related diseases. In fact, in a Precision Health Economics analysis, we estimated that approximately 10,000 more livers would be available in the transplant pool if HCV treatments were more generously covered.

As should be clear, the paradigm shift toward potentially curative treatments requires a broader assessment of value. Gene therapies fall into this category, and they raise new questions about value because while the duration of treatment is short, the extremely high cost must be paid upfront and the return on investment is realized over a lifetime. By standard measures of value and standard health technology assessments (HTAs), these therapies are highly valuable even at their high proposed prices.

However, the questions that payers often have is, "What is the impact on our budgets?" Payers may also be reluctant to cover the costs of a lifetime cure when the benefits will accrue to different, future insurers.

So, how should we be approaching value in the case of cures? And, how should uncertainty be addressed (eg, what happens if the cure isn't sustained)? I believe the focus should be on long-term value, rather than short-term affordability, and the way to finance this focus is through alternative or innovative payment models that help address uncertainty. Currently, there are limited models where payers would be refunded if a disease recurs, but these models need to be implemented in broader scenarios. These payment models and the policies governing them should be rolled out and evaluated in a systematic way so that evidence can be gathered to assess their implementation.

Shifting the Focus From Cost to Value

In the current healthcare environment, scrutiny of evidence is driving priorities R&D and commercial priorities as well as HTA priorities. This increase in the use of HTA evidence is demonstrated by the fact that both CVS and the Veterans Health Administration are using evidence developed by the Institute for Clinical and Economic Review (ICER). The trend toward developing HTAs and value assessments is critical, and the time has come for active, vigorous dialogue and debate on the appropriate methods for developing value frameworks, assessing value, and determining reimbursement.

Sponsors often focus on demonstrating the value of their product in relation to another product in the space. But, as an industry, we should really be thinking more broadly about how biopharmaceutical products or medical devices compare to all of the other ways that consumers spend their healthcare dollars. As mentioned earlier, there are many examples of low-value care in the healthcare delivery system whose cost could be diverted into areas of higher value innovation. Rather than simply focusing on reducing the cost of high-value care, it makes sense to focus on reducing the cost or utilization of low-value areas of healthcare. However we approach it, the net result is a reduction in spending on services or technologies that are not generating significant benefit to patients and reallocation of that spending to incentivize new services or technologies that deliver more value to patients.

To that end, there are ample opportunities for innovative contracting. We are starting to see some of this now, with outcomes-based pricing contracts with PCSK9 inhibitors for lipid lowering and drugs for multiple myeloma. With outcomes-based pricing contracts, formerly known as risk-sharing agreements, payments to the manufacturer are tied to outcomes. Precision Health Economics and others have published articles on licensing or subscription-based payment models where pricing is upfront and all-inclusive. Potential benefits of a subscription model include increased utilization and adherence, as well as the ability to balance providing access to patients today with delivering sufficient innovation incentive to those who will manufacture the treatments of tomorrow.

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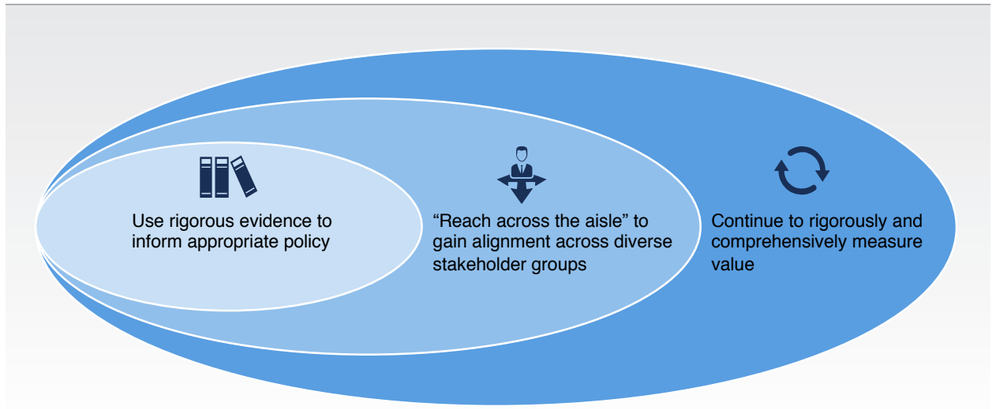

Figure 2. Supporting Policy Change Based on a Broader Definition of Value

Driving the Value Conversation Forward

As policymakers continue to debate on policies and roles for defining value, they need more than good comparative effectiveness research based on RCTs. They also need good comparative effectiveness research based on real-world evidence and sophisticated methods of understanding the true causal effect of one treatment versus another in the population at large. These methods may rely on the use of big data to analyze whether the average, observable effect is the same across all patients or whether the effect is heterogeneous and requires targeting of subgroups.

Using rigorous evidence to inform appropriate policy is just one way to drive the conversation forward (see Figure 2). Another way is to reach across the aisle to gain alignment across diverse stakeholder groups. Although discussions of value can be polarizing, the motivations behind the conversation for all concerned is figuring out how to provide access to innovative technologies that help people live healthier lives. By broadening our view and thinking rigorously and comprehensively about value—both to the patient and to society—we are poised to better balance the trade-off between innovation and access, bringing new treatments to the people who need them most.

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As an economist and physician, Dr. Jena's research involves several areas of health economics and policy, including the economics of physician behavior, the physician workforce, medical malpractice, the economics of healthcare productivity, and the economics of medical innovation. His work has been published in leading journals of economics, medicine, and health policy and has been featured several times in prominent news outlets such as *The New York Times* and *The Wall Street Journal*. He is a winner of the Garfield Award from Research America for the best paper in economics on the economic impact of medical research. In 2013, he received the NIH Director's Early Independence Award to fund research on the physician determinants of healthcare spending, quality, and patient outcomes. In 2015, he was awarded the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) New Investigator Award. Dr. Jena is a Research Fellow at the National Bureau of Economic Research. From 2014 to 2015, Dr. Jena served as a member of the Institute of Medicine Committee on Diagnostic Errors in Health Care.

About Precision Xtract

Precision Xtract, the health economics, market access, and payer analytics consulting arm of Precision Value & Health, is comprised of top-tier talent including scientists, former payer and pharma executives, and world-respected academicians ready to partner with clients throughout the United States, Europe, and beyond. With over 140 employees in 7 offices worldwide, Precision Xtract's breadth and depth of expertise complements an integrated, interdisciplinary approach to informing and guiding pharma, biotech, and device clients to commercial success.

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