

A NEW GOLD STANDARD FOR PRICING

Biopharmaceuticals in today's marketplace are under increasing economic scrutiny. From payors to providers, from patients to politicians, the calls to price innovative medicines fairly are louder than ever. Implicit to the concept of fair pricing is valuation. An evidence-based price represents a monetary valuation of a product from a buyer's perspective based on a foundation of proof. It is imperative for the economic efficiency of the healthcare industry, therefore, to develop a universally accepted pharmaceutical valuation system based on a standard set of principles. One obstacle to achieving this market-based fair price is imperfect information. In this column, I suggest the introduction of a health economic balance sheet to facilitate valuation using uniform information as a way to achieve pricing fairness and consistency.



DR. JOHN DOYLE, practice leader for market access at Quintiles Consulting, introduces the concept of a health economic balance sheet and evidence-based pricing for biopharmaceutical valuation.

CHANGE IS IN THE AIR

Historically, a biopharmaceutical's price was more likely to have been based on commercial considerations rather than on intrinsic value. Risks, benefits and costs associated with a biopharmaceutical are typically valued independently, and previous attempts to combine these value domains have been challenging and met with substantial manufacturer resistance.

For the last decade, payors – specifically managed care organizations – have shifted their focus to drug prices as a means to control health care costs. The changes that we're seeing now seem to start in the commoditized and heavily competitive therapeutic areas, such as respiratory illnesses, where there's a lot of pricing competition for asthma and allergy medications. Managed care organizations have started to put some tools into place,

either leaving a new drug off the formulary, which requires prior authorization for disbursement, or instituting some sort of internal treatment guideline to control and limit access to the drug.

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In addition to limiting access, managed care organizations and other payors are now starting to require some formal demonstration of value. This has been the case in Europe for a long time, but now we're seeing many U.S. entities employing similar evaluation techniques. Blue Cross and Blue Shield Association, for example, has a Technology Evaluation Center that helps plans evaluate a drug's value. And although plan efforts are often well-intended and based on economic evaluation, the current system for determining the value of medicines is disjointed and hardly suitable for today's global marketplace.

DETERMINING VALUE

The science of health economics has transitioned from its adolescent stage to a period of maturation in which scientists can focus on the standardization and application of its metrics. Most health economic studies are described in the literature as cost-minimization analysis (CMA), cost-benefit analysis (CBA), cost-effectiveness analysis (CEA) or cost-utility analysis (CUA). But where CMA and CBA offer a simple, standardized measure of end points in the form of currency, CEA and CUA use heterogeneous outcome measures in their denominator that present a more vexing problem to decision-makers attempting to compare therapeutics.

Scientists have attempted to address this problem by using a common denominator, such as quality-adjusted life years (QALYs). Cost-per-QALY is fast becoming a standard measure for health economic evaluations, but the random and non-random bias potentially associated with disparate measures of quality limits the objectivity of therapeutic comparisons. Additionally, the application of the QALY metric to therapeutics that lack prospective evidence demonstrating survival improvement leads to misguided interpretation.

One may argue that the focus should be on surrounding the core value proposition with a variety of standardized techniques that can be easily interpreted and compared.

QALY metrics generally work well in Europe and in other closed health systems that are predominantly driven by a single government payor. And one of the key reasons for its popularity is that the QALY metric, in many cases, requires a long-term outlook. But QALYs do not work as well in the United States, with its decentralized and managed-care driven market. Managed care organizations tend to look at a therapy's impact over a two- to three-year period, rendering the QALY metric less significant compared to a lifetime outlook.

Pharmacoepidemiologists also have attempted to capture the risks and benefits in a combined metric, but this approach lacks universal acceptance. Typically, risks and benefits are presented in a ratio format. For instance, one technique compares the number needed to treat (for benefit) to the number needed to harm (for risk). The selection and measurement of benefit and risk will, of course, vary within product research and across product comparisons. One may argue, then, that rather than searching for the ultimate metric that fully captures all value attributes for all stakeholders, the focus should be on surrounding the core value proposition with a variety of standardized techniques that can be easily interpreted and compared.

THE HEALTH ECONOMIC BALANCE SHEET

Financial analysts use a variety of measurement techniques to evaluate the value of products, services and companies. These financial valuation techniques may be referenced as a conceptual framework for biopharmaceutical valuation. Financial analysts measure company value under conditions of uncertainty to provide insight on investment opportunities. Similarly, pharmacoeconomists aim to measure product value under conditions of uncertainty in order to provide information on the net economic impact of commercial adoption of biopharmaceuticals.

Financial valuation already has served as a reference point for health economics. Indeed, health economists long ago adopted net present value techniques to account for time value of costs and benefits. The issue of time is of particular concern to pharmacoeconomists and pharmacoepidemiologists. Benefits, risks and costs often are accrued during different periods and at different rates. Discounting techniques can be used to account for the value of time, but there is little agreement on the selection and application of a discount rate. As the time value of money often is calculated to be industry and even company specific, greater specificity in the selection of the appropriate discount rate for a product's valuation is warranted.

Financial valuation begins with standardized financial reporting. Data are reported in similar formats by public companies throughout the world. For instance, the company balance sheet is a snapshot of financial health, with assets and liabilities presented in a standard format.

A health economic balance sheet may be conceptualized as a snapshot of positive and negative product attributes. Clinical development contributes most of the evidence supporting a health economic balance sheet prior to launch. Manufacturers will collect data on safety and efficacy, for starters, but they also might test things such as patient-reported outcomes (PROs). Additionally, you want to test economic metrics with regard to efficiency and effectiveness of the treatment process. Do you cause a patient to interact more or less with the health care system? Are they coming in for more administrations or fewer administrations? Are you decreasing length of stay? Are patients using fewer concomitant medications? Do adverse events require treatment?

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To make a standardized system for biopharmaceuticals work, we first need to weigh the overall constellation of product attributes. Before you actually value and try to assign a monetary amount to a product, you want to figure out what really matters from a payor standpoint. When they're making their decisions about medicines in a particular indication, what really matters to them? Is it morbidity or mortality concerns? Is it unmet medical need? Is it patient convenience? Each of those requires a relative weight, so you need to know what matters for that particular indication. The idea is to get a scorecard of what matters to payors at that particular time, and to use this as a framework for product valuation and construction of the health economic balance sheet.

The second step is to then factor in the product to determine where your product attributes measure up against what's important to payors. That should provide an idea of what elements are going to resonate with a payor in terms of a value negotiation. But the tricky part – and the one in which some of the art form comes in – is assigning some type of value to your product's attributes to differentiate it from the current standard of care. The selection of current standard of care, or reference product, anchors the evidence-based price. Ideally this comparison between reference products could be done in a standard side-by-side format.

The key to a health economic balance sheet is presenting all of this information in a standard format listing short-, mid- and long-term benefits. These benefits could be further categorized as direct, indirect and intangible. The former two lend themselves to monetary valuation and the latter is reported in physical benefits (e.g., PROs). Then you'd want to do the same thing on the liability side of the equation to demonstrate the short-, mid- and long-term liability of the product, such as adverse events. The point of the exercise is to enable the decision-maker to draw wise comparisons based on clear, concise and accurate information.

CHALLENGES AND LIMITATIONS

Part of the challenge is just getting all the data in a standardized format and reporting it in a way that all stakeholders will accept. Cost figures for oncology drugs, for example, are reported by companies quite differently, including cost per cycle, cost per treatment regimen, cost per year, cost per patient, cost per survived patient and so on. The lack of standardization makes it very difficult for members of a pharmacy and therapeutic committee to accurately compare one product to another.

But in order for this tool to meet the needs of all stakeholders, there are two primary things to consider. First, payors in a decentralized health care system – such as in the United States – need to step up their health technology assessments and do them in a standard way. The present system of proprietary assessments is extremely inefficient and places biopharmaceutical products at risk of not always being valued fairly. Second, if centralized health technology assessment comes to pass, such as NICE in the U.K., then de facto price controls may stifle innovation. A system such as that would develop its own set of standards for determining a drug's price, which will likely not be commensurate with a holistic view of a product's value.

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Lastly, any biopharmaceutical pricing mechanism must ultimately provide patient access to life-saving and life-extending medicines while ensuring economic returns to the innovator companies who develop them. It is incumbent upon the biopharmaceutical manufacturers, therefore, that any health economic balance sheet also must be available to patients. Perhaps it is only an abridged version, but there's no reason in today's climate of consumer-driven health care why there couldn't be a patient version of the balance sheet that could be accessed online, just like a drug label. Placing this information in the public domain would give patients a top-line economic assessment of risks and benefits in a transparent manner and would ensure that patients are at least included in the process of determining a medicine's value.

The endorsement of a health economic balance sheet by groups such as Pharmaceutical Research and Manufacturers of America (PhRMA) or the International Federation of Pharmaceuticals Manufacturers & Associations (IFPMA) could play an enormous role in the realization of this concept. But the onus of implementing a tool that introduces evidence-based rationale for the pricing of medicines ultimately falls to the pharmaceutical manufacturers. It is up to the industry – and in the industry's own best interest – to embrace a new, transparent manner in which to price its products.