NEXT STEPS IN INNOVATIVE PRICING MODELS: SHARED RISKS, SHARED BENEFITS

BY MICHELE CLEARY

WITH THE INFLUX OF NEW THERAPIES AIMED AT GENETIC TARGETS with curative intent, innovative pricing models are increasingly used in hopes of broadening access to affordable, high-value care. The old, rigid pricing model where you have a fixed price (ie, a single price for a vial or a pill used with fixed chronicity) is being eclipsed by the growing interest in pricing models that pay for healthcare outcomes as opposed to paying for a particular volume of medications. Michael Schroeter, PhD, Sachin Jain, MD, MBA, and Bethanie Stein, PharmD, spoke about the growing use, inherent challenges, and opportunities for improvement to innovative pricing models.

Growing need for innovative pricing models

Over the past decade, innovative pricing models have been used with increasing frequency. These models include volume-based pricing, indication-specific pricing, financial risk-based contracts, mortgage models, and subscription models.

Michael Schroeter, founding partner at Viopas Partners, in Basel, Switzerland, sees 2 primary factors driving this increased use. First, more drugs are being developed for multiple indications. Second, changes within regulatory practices are accelerating drug approvals, many with surrogate endpoints and smaller clinical trial data packages.

More flexible, innovative pricing models can address the misalignment between clinical benefits delivered by different indications or drug combinations. In these cases, Schroeter views the move towards innovative pricing as more clinical and less economic, stating "I think it's the science that is pushing towards more use of innovative pricing models."

But Schroeter also cited changes within regulatory practices as further accelerating the use of these new pricing models. "You've seen that the FDA [US Food and Drug Administration] has accelerated approvals…and approved drugs with less stringent kinds of data associated with it." Accelerated approvals often rely on the use of treatment endpoints that the FDA accepts, but which are a poor fit for pricing models. Uncertainty surrounding long-term outcomes may also drive the use of these models. "This uncertainty," Schroeter noted, "must increasingly be managed through outcomes-based pricing. Innovative pricing models can help you mitigate uncertainty around the data."

Mitigating uncertainty

Schroeter outlined how conditional approvals mean drugs lack "the perfect kind of dataset" that would allow a payer to determine the value and then set the price. Instead, these data limitations lead to uncertainty. "Getting hold of these data in a consistent and quality fashion is still a challenge."

He continued, "We need to be able to track drug utilization and outcomes…to track how much of the drug was used, by how many patients, over which period of time, in which quantity, for which indication, with which outcome. But this is often a long and arduous goal."

Challenge to find ideal endpoints

Sachin Jain, former CEO at CareMore and adjunct professor at Stanford University, Stanford, California, USA, agreed with Schroeter that pricing models are challenged by the choice of appropriate endpoints in pricing models. In other words, what makes clinical sense may not be meaningful or acceptable to all stakeholders.

Jain noted how ideal endpoints vary by disease, stating, "I think there's going to be some diseases where this type of pricing is easier and others where it's going to be harder." He continued, "If you look at an area like cystic fibrosis [with] medicines that people need to take in perpetuity, you could think about models that are focused on certain types of outcomes. These are diseases with a clear cause and clear effect that can be measured easily."

Endpoints may be more complicated with chronic conditions. The choice of endpoints is further complicated as new drugs are introduced with new modalities, providing longer-term outcomes. In oncology, for example, models traditionally used overall survival as the primary endpoint. Jain said that pricing models for oncology drugs are now using more surrogate endpoints, such as progression-free survival. These surrogate endpoints often lead to conflict. "The FDA is more open to the use of surrogate endpoints for drug approvals," Jain said. "But payers don't want to pay for progression-free survival. They want to pay for overall survival."

This lack of survival data and the inherent uncertainty that comes with that, Jain stated, creates demand for innovative pricing models. "Not having that data at hand, researchers are left with more surrogate endpoints, which from the payer perspective, puts the uncertainty back into the manufacturers' court and off the payers'."

Payer perspectives

Faced with uncertainty surrounding treatment outcomes, many payers are turning to innovative pricing models. Bethanie Stein, Vice President of Strategic Contracting, Purchasing, and Analytics at Humana in Pittsburgh, Pennsylvania, USA, shared her insights into how Humana has been using these models. One of the first payers in the United States to create a value-based contracting strategy, Humana has since completed over 50 of these agreements since 2012.

Stein noted that Humana typically utilizes value-based contracting in disease states where there is a lot of specialty drug use, such as oncology. "We feel that those drugs are typically fast-tracked by the FDA or offered some sort of breakthrough status, and typically approved on phase II clinical trials [oftentimes] without the rigor of standards that we see with other drug classes, like diabetes, for example." She

continued, "Whenever we focus on those specific classes, we construct a value-based contract to answer the uncertainties that exist around those first-in-class agents or accelerated drugs, and those contracts are typically around a safety, efficacy, or total cost-of-care element."

From her perspective, surrogate endpoints can be problematic. "I think that a lot of manufacturers tailor the value-based contracts to their FDA label, which is unfeasible and really hard to manage," Stein reflected. She added that Humana wants to move away from surrogate markers, such as A1C or adherence, and really focus on answering value-based questions.

Humana uses its own claims data to capture some of these unknowns surrounding safety, efficacy, and total cost of care. For instance, when uncertainty surrounds product tolerability, Humana may examine discontinuation patterns. Stein provided an example of a manufacturer that argues that patients should be able to tolerate the drug for 3 months. She noted that if patients discontinue before that 3-month marker, clinical benefits are impeded by tolerability or safety issues. In this case, Humana views this event as a failed outcome and the drug manufacturer would assume the risk for that treatment.

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Pricing models for cancer drugs often include progression-free survival, a common efficacy marker. She recounted how a manufacturer may tout a product's superior ability to achieve progression-free survival at 8 months. However, if Humana's data found that a member died or had added or changed their drug therapy, this would be viewed as a failing. In this case, she stated, the manufacturer would "go at risk."

And finally, the manufacturer could "go at risk" for the total cost of care, where total costs of care with a new drug would be compared to the cost associated with standards of care. Stein stated, "If the total cost of care is less than the standard of care, [the manufacturers] would not assume any risk. If it was more, then they would assume more risk."

Stein recommended that manufacturers keep it simple around safety, efficacy, and total-cost-of-care endpoints. But primarily, she encourages dialog between the payers and the manufacturers. She said, "The message that I have been sharing publicly is to say, 'Come to us with your gene therapies, high-cost drugs, or specialty orphan oncology [products] and let's have a conversation around what a meaningful value-based contract looks like.'"

Further data limitations

Jain emphasized that effective utilization of these models requires better outcomes data. "I think we need new ways of thinking about data and the role of health services research and outcomes research data in the development of medicines." He cited firms, such as Vertex Pharmaceuticals, that use new methods like artificial intelligence and machine learning to extract information about diseases and treatments. These digital tools and technologies provide a new look at real-world outcomes data and real-world functional outcomes. "We're talking about creating a new ecosystem that is going to drive and create a lot of value for the industry and for patients."

But Schroeter cautions, "For real-world data to answer a scientific question, the data set needs to be representative of the disease so that you can make statistically sound decisions for commercial agreements (eg, geography)." In addition, he emphasized the need to incorporate stakeholder perspectives, stressing that moving to an innovative pricing model only makes sense if you can address the different stakeholder needs through that model. "If it is just a model to address one stakeholder need and for one stakeholder to benefit from it, then it will be a failure. You might succeed with one drug, but you won't be able to repeat it with your next drug in the pipeline. I think that's a huge miss."

The need for regulatory changes

Both Stein and Jain felt these innovative pricing models could be improved through regulatory changes. Stein stated, "I would love for more plans and payers to come up with similar strategies and push manufacturers the way that we are pushing them versus allowing manufacturers to dictate a value-based contracting strategy." But she notes that regulatory barriers would need to be removed. "It would allow both sides to take on more risk. It would improve access to those really high-cost gene therapies if we were able to share in that risk."

Jain echoed this call for regulatory reform, arguing the need to simplify both how we measure value and how we pay for value. He stated, "I think the challenge is that this is really a regulatory environment where a lot of pricing is tied to average wholesale price across the marketplace." Jain continued, "If you have an outcomes-based pricing model [where], for whatever reason, the outcomes are poor and there's zero payment, the model actually takes the average wholesale price of the drug. That influences how government payers and others actually pay for those medicines."

Challenges along the value chain

While Jain believes that introducing value-based pricing is very straightforward, he argues that paying for value is complicated by the large number of participants within a value chain. "I would say the implementation is stymied by the great

complexity of what it takes to actually get drugs into the hands of patients."

With so many participants along the value chain, administering value-based programs where the simplest level would involve rewarding a drug manufacturer developer for a particular outcome becomes untenable. "It's oftentimes hard to attribute where the true outcomes improvement comes from," said Jain. "As a result, attempts to capture value sometimes overreach because while there are few clear cases where the value is produced entirely by the medicine, the value could have been produced by other parts of everything that goes into delivering care for the patient, [and] the clinical model in which the care is delivered."

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Schroeter voiced his concerns surrounding who should pay for products that generate benefits over time, especially if they fail to generate cost offsets. In these cases, he asks how we can spread the costs over the period of benefits, especially if the product is only administered 1 or 2 times to the patient. This is especially problematic in the United States where patients can readily switch payers. "Why would I as a payer pay for something upfront when the next payer then benefits from a healthy patient and I carry all the burden?"

Pricing demonstration projects

Jain proposes an entirely new framework for measuring the effectiveness of medicines and for paying for the value created, but notes the problem is identifying which party should own all of the risk. "The question is whether there is going to be some kind of company that owns all the risk. That's technically what health plans should be doing, but they're not really organized to do that because they don't often own all the elements in the care delivery and all these other pieces."

He argues that to make these models work, we need further evolution in the structure and design and organization of healthcare delivery in the United States. "I think we need to develop demonstration projects for pricing models—some bold demonstrations of value-based/outcomes-based pricing in practice," Jain said. "You could imagine a whole new category of companies that could take risks for specific diseases and build a set of solutions that include medicines, and lifestyle interventions, and ultimately try to optimize outcomes for particular types of patients. And you see pieces of these types of companies all across the marketplace."

Jain proposed that his former organization, the Center for Medicare and Medicaid Innovation (CMMI), be a participant in such a project. "I think the federal government, being the largest payer in healthcare, has a role to play. There's an increasing level of engagement between CMMI and pharmaceutical manufacturers. I think there's some interesting work potentially going on in insulin and diabetes outcomes. I think once the federal government and Medicare/Medicaid start playing in the space, I think it becomes easier for everyone else to play in this space."

Affordability remains the challenge

While these models may help mitigate uncertainty and help payers manage their budgets, affordability remains a primary concern, especially under current budget constraints. In this regard, Schroeter argued, "It's not a clinical problem. It is really a problem of how to deal with it economically."

"'Affordability' doesn't necessarily mean 'cheap,' but it needs to generate significant cost offsets to help reduce overall healthcare spend," said Schroeter. "I think you get into increasing the conundrum by trying to justify from a health economics perspective that it's something [that] makes sense." Jain understood how payers might rationalize the high cost of a drug or therapy this way: "Yes, it's expensive, but it helps me save costs overall by reducing hospitalization and by moving a chronic disease into a curable state." He continued, "Thinking through these kinds of paradigms and generating significant cost offsets, even in a budget-constrained environment, can make drugs affordable despite the fact that they are high priced."

Turning crisis into opportunity

As health systems globally face even further budgetary constraints under the current COVID-19 crisis, Jain remains optimistic. He sees opportunity for change that will improve pricing processes. "Crisis moments like COVID-19 give us an opportunity to really look at how things are organized now... evolving to a clearer view of what the country needs."

"I think as we formulate a view of the future, we have to be flexible in our thinking, cognizant of the current crisis, but not overly reactive to it either. The challenge is that sometimes the right thing involves short-term pain to create long-term gain. What I believe we need more of is courage."

About the Author

Michele Cleary is a HEOR researcher and scientific writer with more than 15 years of experience in the healthcare field.