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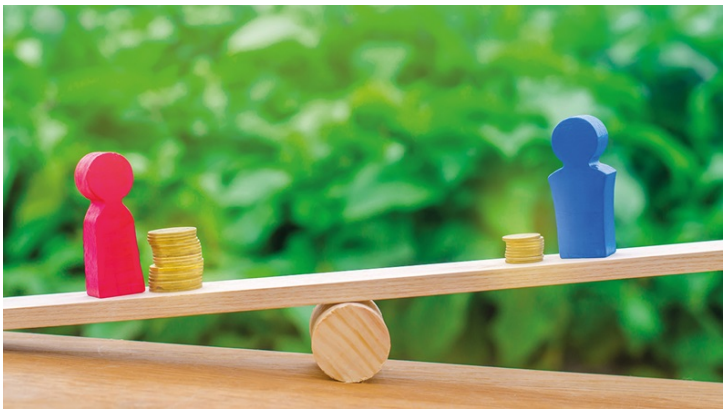
High-Priced Drugs For Rare Disease: Leading US Payers Discuss Cost Concerns, Value-Based Contracts And ICER

14 Jun 2020 | **ANALYSIS**

by Cathy Kelly | Catherine.Kelly@informa.com

Executive Summary

There are as many rare disease patients as there are diabetics, Cigna's Steve Miller notes, arguing that exorbitant prices for each treatment could drive counterproductive categorical exclusions by payers.



OptumRx Inc. chief formulary and procurement officer Kent Rogers, Cigna Corp. chief clinical officer Steve Miller and Harvard-Pilgrim Health Care chief medical officer Michael Sherman offered their perspectives on reimbursement for high-cost therapies, both chronic and curative, at the Biotechnology Innovation Organization virtual international convention. They were joined by Jason Tardio, chief commercial officer for Ovid Therapeutics Inc., which is developing treatments for rare disease.

The panel discussion, moderated by Real Endpoints CEO Jeff Berkowitz, focused on pharmacy benefit manager and payer concerns with affordability, the role of patient advocacy, value-based purchasing and value assessments conducted by the Institute for Clinical and Economic Review. Below is a summary of the various points made.

Rare Is No Longer Rare

Cigna chief clinical officer Steve Miller: "When you take all the patients who have rare and orphan diseases [you see] they're not rare. There are 30 million people in the US and when you take the 6,000 orphan diseases and put them together, that's as many [patients] as there are diabetics. … As we have more rare and orphan diseases with successful therapies, which is what we all want … the question is how do we afford these things?"

"We have clients who are actually thinking about, 'How do I carve these things out. How do I not cover rare and orphan disease or how do I not cover gene therapies?' These are unprecedented conversations. They are looking for bright lines that they can use to define subsets of the population [and say], 'We're just not going to pay for cell and gene therapy.' … That's the wrong thing. We need to be as innovative on paying for drugs as the pharmaceutical manufacturers are being in discovering these drugs."

A 'Fair' Price For Rare Disease Cures: Payers, Physicians, Patients Weigh In

By Cathy Kelly

02 Jun 2020

Industry-funded survey by Charles River Associates explores diverse perceptions about pricing for high-cost cell treatments – including curative cell and gene therapy – among various stakeholder groups.

Read the full article here ›

Harvard Pilgrim Health Care chief medial officer and senior VP Michael Sherman: "Eight percent of the population, it's estimated, has a rare disease. So collectively when you are looking at drugs that are frequently priced in the mid-six figures – and we're seeing some cell and gene therapies higher for one time use – and you look at that collectively, you wind up with the issues of affordability and value. It's also true in many cases that these may be shown to work in some of the population and if there is variation it's hard to argue that it's worth anything close to that price tag when it's less effective."

OptumRx senior VP, chief formulary and procurement officer Kent Rogers: "At the end of the day, what is interesting and fascinating about the innovations that are coming to market is that we've got a lot of clients that are asking: 'How do we avoid paying for this? How can we create a secondary insurance market that will help pay for this?' So whether we're talking about chronic rare disease or 'one and done' curative treatments, I think the price is still probably where I'm going to go – and say we've still got to be able to afford it."

Patient Advocates Should Push For Treatment Affordability

Harvard-Pilgrim's Sherman: Patient advocates "absolutely have an important role to play. … Having them express their concerns and help us understand what's important to them in outcomes matters. … I also think there's a lot of noise out there and I understand advocates exist to help drive research and want coverage, but it's worth remembering that they presumably want what I would want and anyone would want, they want a treatment that works" and many of them "understand that affordability is an issue."

Cigna's **Miller**: "There's always a tension here because there's no advocate like a parent with a child with an illness" but "we still have to be science driven. The advocates are really crucial and knowing what's important for a family is really important. … But "the one thing I would ask the advocacy groups is they actually have to be more involved in affordability.

"As a parent, we always make the statement that we would pay anything for a product. But you've got to remember it's not the individual family that pays for those products when they're on group insurance. They're asking everyone in the group to pay for it. So they have to be very cognizant of costs and they have to do everything in their power to help influence affordability."

Outcomes-Based Contracts May Rely More On Patient-Reported Results

By Cathy Kelly

27 Jan 2020

As interest in immune/inflammatory disease increases and savings achieved dip, payer focus on patient-reported outcomes for contracts reflects shift in focus, Avalere survey suggests.

Read the full article here ›

An Evolving Role For Value-Based Contracts

Harvard-Pilgrim's **Sherman**: "There is a really terrific opportunity" for value-based arrangement "where we need them. We don't need value-based agreements for press releases, just for the sake of saying that we're doing these. … It needs to be worth it. In many cases for common chronic disease you have competitive markets and other forces at play and in many cases there's not a whole lot of question about whether a drug works when it's taken as a directed. So in those cases, we actually we don't need these. It just creates work for everyone.

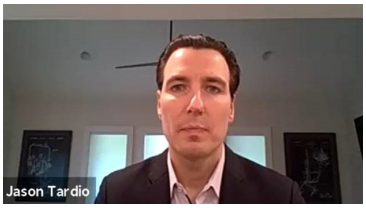
"However, when you look at high cost rare disease treatments, particularly those that are new to market … we need these kind of agreements to facilitate adoption so that payers are willing to spend the money and employers are willing to support that knowing when [the treatments] are not effective, they'll be paying a lot less.

"This gets into the outcomes measures and whether they are reasonably robust and how much is at risk. In many cases, we've said 'no' to potential agreements because [the amount at risk is] miniscule relative to the cost. That would let a stakeholder say, 'Hey we're addressing the affordability issue.' But not if it's only a couple of percentage points at risk. It needs to be far more robust when you're talking about these price points."

OptumRx's **Rogers**: "My personal view on this is while I think there are some interesting pilot project ideas that are out there, I'm more interested in something that is scalable.

"I prefer to think of the provider, meaning the specialty pharmacy or specialty infusion house, as the best possible way to create an outcomes-based agreement, where you're actually getting regular data being collected because of just the normal interaction with the provider and patient. [Data can include] the concomitant use of medications, medication switches, the stage of disease they're within, side effect management and what works and what doesn't."

"…Even at an aggregated level, this is very, very valuable real-world information that I feel is a better broad-based application of value-based agreements. It's not related to how a drug is covered from a formulary perspective. But I feel as we move into these higher-cost, rare, orphan disease therapies, it's incumbent upon us to have this kind of information being part of the routine nature of working with the providers."



Ovid Therapeutics chief commercial officer Jason Tardio: "Ultimately I think value-based agreements really hover around whether you believe in your product. … Do you believe your product is going to provide similar clinical results as seen in … clinical trials as it will in the real world? And ultimately, if a manufacturer believes that, then they shouldn't be afraid of these value-based contracts."

"Clearly this is where reimbursement is going. … At the end of the day, if our patients can't access our therapies then we don't have a drug. All that we've done in our development is foregone. So we have to balance a bit around how we think about pricing and our pricing principles."

The Inevitability Of Value Assessments, QALYs And ICER

OptumRx's Rogers: "I think [value assessment] is an effort that we have to employ." The quality-adjusted life year (QALY) "is just a basic health economics metric that's been applied throughout Europe. What's interesting is that metric hasn't really changed in about 40 years, so I think there's probably arguably some adjustments that need to be made and maybe COVID has something to do with that as well.

"But you haven't seen that broadly adopted here. It's a much different system. We don't have a one-payer system even though the [US] government in many respects [covers] about the mid-50% range of the insured population of the country and COVID is probably going to increase that through Medicaid expansion.

"We need to find some measure that creates a defined value, or at least some consistency in how medicines can be viewed with a defined value. The challenge is that there are some disease states, [multiple sclerosis] is a really good example, where there is not a good QALY for that."

COVID-19 Economic Fallout May Delay Cell And Gene Therapy Reimbursement Solutions

By Cathy Kelly

07 Apr 2020

The economic stress on payers caused by the pandemic will cause a setback in the development of payment approaches for regenerative therapy, Blue Cross Blue Shield executive warns.

Read the full article here ›

Nevertheless, "this very sterile, somewhat offensive health economics metric is going to start to be applied. I know Dr. Miller and his group and well as our group and Dr. Sherman and his group have been worried about this for some time now and we've all got our individual programs in place to try to seed this notion within the marketplace."

"I'd say in the last year or two, there's been a more concerted effort to applying that to how we decide than I think the rhetoric has been in the past. Steve [Pearson]'s group at ICER is bringing it to the fore. So companies like Harvard-Pilgrim and Express Scripts and OptumRx and United, we have to come up with strategies that either are our own or in one way or another, we use [ICER] as one of the data points that we access as well."