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14 Sep 2020 | **Analysis**

# Value-Based Contracts Are Evolving

*US Payers Turn To High Cost, Curative Therapies*

by Cathy Kelly

Value-based contracting in the US is moving toward high-cost drugs for rare disease and away from lower cost chronic treatments as payers plan for the coming pipeline of cell and gene therapies. Marketed gene therapies all have some kind of risk sharing deal in place but further progress on the most innovative payment models is hampered by regulatory and operational challenges.

After experimenting with value-based contracts over the past three to four years, US payers are shifting their deal-making focus from chronic use drugs to high-cost agents like cell and gene therapy. The change reflects payers' growing sense that value-based or outcomes-based contracts for chronic use therapies can be more trouble to hammer out than they are worth. Another driving factor is that highly expensive gene therapies are beginning to reach the market, more are coming, and collectively they are expected to pose a serious reimbursement challenge.

The value-based purchasing environment has matured over the last four years, where before there was more talk than actual contracting. (Also see "*US Outcomes-Based Contracts: Big Uptick In Interest, But Not Execution*" - In Vivo, 6 Nov, 2016.) More companies and some Medicaid agencies have gotten involved in value-based contracts since then. Many agreements in place are outcomes-based, risk sharing arrangements with clinical or economic endpoints. Some are subscription-based arrangements. But a few things have not changed over the years: details about specific arrangements are hard to come by and the parties involved generally do not disclose how the contracts play out.

The past few years "have been something of an experience curve," Harvard Pilgrim Health Care chief medical officer Michael Sherman explained in an interview. As one of the most visibly proactive payers engaged in innovative contracting, Harvard Pilgrim has been able to "demonstrate that we can execute these kinds of arrangements. We've built some strong

relationships" and managed "some adjustment in payment," he recounted.

*Payers are at a point "where people are realizing value-based contracts are not in themselves a panacea and probably not necessary in many cases," says Harvard Pilgrim Health Care's Michael Sherman. "But for high-cost, new-to-market drugs with limited data and high variability in terms of response, they will be extremely valuable in promoting access to those that need them."*

But "the fact is that many of these [agreements] have come in chronic disease, where you have drugs that are fairly well characterized [and] where in most cases you have more rational pricing and a competitive landscape" that can help restrain prices, Sherman added. In other words, many contracts have focused on categories where a risk sharing contract may not be as helpful.

## Is The Juice Worth The Squeeze?

"As we see companies coming forward, wanting to offer a couple of percentage points" off the price if a drug does not perform as it should, "the question is, in many cases, is it worth it?" he pointed out. "Is the juice worth the squeeze? Is it worth the work involved for all of us to put these together, to change our policies and to do the work of monitoring, measuring and truing up?"

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Manufacturers appear to be getting the message. "After my having provided that feedback to pharma companies, I'm seeing less discussion and fewer pharma companies coming to us offering what I'll call less interesting, lower value agreements," Sherman reported. He acknowledged some of the slowdown in overtures from pharma could be attributable to the COVID-19 pandemic but maintained that "this change in focus preceded the crisis" and "I suspect it's something we're seeing in the industry" more broadly.

Avalere Health senior advisor Kathleen Hughes agreed that cell and gene therapy is "absolutely where payers' emphasis has been redirected." Payers are now "used to chronic compounds and

they're finding different ways to deal with those" besides value-based contracts. But now with the new "cadre" of very high-priced agents, "they're having to consider very innovative things."

That is not to say that some new drugs that fall along the spectrum between traditional chronic use agents and gene therapies would not benefit from outcomes-based arrangements, depending on what they cost, where they fit with other agents and the size of their patient population.

Disease-modifying agents for Alzheimer's disease are an important example. *Biogen, Inc.*'s aducanumab may get FDA approval next year. And "given the likely high cost and massive potential demand for such a product, I think you will see a great surge of value-based contracting associated with this and other compounds for Alzheimer's disease treatment" when they reach the market, Hughes predicted.

In the meantime, pharmacy benefit managers (PBMs) are predicting the cumulative cost of gene therapies will reach $15bn or more in the US by 2024, which is driving them to develop solutions *(see Exhibit 1)*.

Exhibit 1.

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One example of a solution, launched earlier in 2020 by *Cigna Corp.* and its *Express Scripts Holding Company* unit, involves payers enrolling and making monthly per member payments into a newly dedicated program that would cover the cost of gene therapy in the future should the need arise. (Also see "*Cigna/Express Scripts Gene Therapy Solution Involves Plans 'Pre-Paying' For Coverage* " - Scrip, 10 Sep, 2019.)

The program is available to Cigna and Express Scripts' commercial customers as well as health plans, employers, insurers and health maintenance organizations that do not work with Cigna or its PBM. It currently covers two gene therapies – *Novartis AG*'s Zolgensma (onasemnogene abparvovec-xioi) for spinal muscular atrophy and *Spark Therapeutics, Inc.*'s Luxturna (vortigene neparvovec-rzyl) for congenital blindness.

## Gene Therapies Covered By Value-Based Contracts

Marketers of gene therapies have been highly proactive in engaging with payers on value-based contracts. "Virtually all of them have got one type or another of a value-based contract associated with them," Hughes observed. (Also see "*Progress Being Made With Cell And Gene Therapy Market Access, But Challenges Remain*" - Scrip, 8 Oct, 2019.) Zolgensma is covered by value-based arrangements in at least two Medicaid programs, in Massachusetts and Oklahoma.

And companies like *bluebird bio* are actively laying the groundwork for future launches in the US.

Bluebird is developing a gene therapy for sickle cell disease called LentiGlobin and is planning to seek US approval in the second half of 2021. LentiGLobin is marketed in Europe as Zynteglo for the ultra-rare indication of transfusion-dependent beta-thalassemia.

A one-time treatment, Zynteglo is priced at around $1.8m, which the company has been proposing to split into annual increments over five years for payers in those markets, a strategy it is also promoting in the US. (Also see "*Bluebird Pushes For Zynteglo Pricing Of Five €315K Annual Installments* " - Scrip, 14 Jun, 2019.)

US government advisory organizations are also urging progress on innovative payment models for gene therapy. The National Academies of Sciences, Engineering and Medicine released a report 10 September calling for the US Centers for Medicare and Medicaid Services and state Medicaid programs to accelerate efforts to develop new payment models, such as installment approaches, for managing the high upfront costs of gene therapies for sickle cell disease. (Also see "*Sickle Cell Gene Therapy: Medicaid Needs Solutions For High Upfront Costs Soon*" - Pink Sheet, 10 Sep, 2020.)

Installment payments for gene therapies have attracted a lot of interest and discussion but so far have yet to find traction among payers because of operational challenges. In the fragmented payer market that exists in the US, one of the most formidable challenges is how to handle payments over time when patients move between insurers. Manufacturers are also concerned that such arrangements, and other types of value-based contracts, face substantial regulatory obstacles in the US.

## CMS Finally Moves To Revise 'Best Price' Rules

Many point to Medicaid rules requiring that the program has access to the "best price" offered anywhere for a product as an serious obstacle, because a price concession reflecting treatment failure in a single patient could set a new best price obligation to every Medicaid program in the country. Companies also worry that a single installment in a five-year payment plan might be considered a new best price.

The Centers for Medicare and Medicaid Services took a step toward addressing those concerns in a proposed rule issued in June. Stakeholder reaction to the proposal has been mixed but most agree that the fact that CMS has opened the door to change is encouraging and an important step forward. (Also see "*CMS Changes The Dictionary: Medicaid 'Best Price' Could Soon Mean Many Things*" - Pink Sheet, 18 Jun, 2020.)

The proposal presents two pathways to resolving the best price problem. First, it would amend CMS' definition of a bundled sale so it can accommodate value-based purchasing arrangements and help ensure that a price concession associated with one failed outcome is not reflected as the lowest price for best price reporting. CMS defines a bundled sale as an arrangement under which

the rebate, discount, or other price concession is conditioned upon the purchase of the same drug, drugs of different types or some other performance requirement.

Second, the proposal outlines an innovative approach that would allow companies to report multiple best prices to account for varied health outcomes over time for individual patients in a value-based purchasing arrangement. Medicaid programs that track the same outcomes would take advantage of the relevant best price for the associated patient outcome.

Manufacturers support the revision to the bundled sales definition, which they say essentially codifies a principle that the biopharma industry "has always understood to be the case – that contingent value-based concessions may be addressed in price reporting through the bundled sales methodology," according to comments submitted to the agency by the Biotechnology Innovation Organization.

> *"We're in the second inning of a nine-inning game" on removing regulatory obstacles to value-based contracting, says attorney Jeffrey Handwerker.*

However, the bundled sales pathway would not apply to many gene therapies on the market or in development because those treatments target rare conditions and would not have enough volume with individual payers to use the pathway, BIO and others point out. Treatments for conditions such as spinal muscular atrophy, sickle cell disease, muscular dystrophies, severe hemophilia A and B and lysosomal storage disorders "would only be able to avail themselves of the proposed multiple best price pathway to prevent refunds or reimbursements triggered by non-responding patients from potentially skewing their quarterly reported best price," BIO maintains.

On the other hand, CMS needs to flesh out its thinking on the multiple best prices significantly before the policy can be implemented. Comments from the Pharmaceutical Research and Manufacturers of America suggest the agency withdraw the multiple best price section from the proposed rule and issue it as a separate proposal with much more detail, "as this section lacks sufficient information for us to understand the proposed approach and formulate informed comments."

The Alliance for Regenerative Medicine also points out that data systems will need a major overhaul to implement multiple best price reporting. "Despite current capabilities to report

multiple best prices manually, all systems and reporting mechanisms used in Medicaid best price reporting will likely need to be upgraded, if not overhauled, to be able to track and report sets of best prices based on particular pricing structures," the group said.

Furthermore, "we are concerned that the operational changes necessary to accommodate multiple best prices are so significant that we could see delays of at least two to three years before implementation, which only serve to further postpone greater adoption of" value-based purchasing (VBP) arrangements, ARM added. "Clarifications in the final rule would help mitigate that potential delay, as would iterative, subsequent guidance issued by CMS to tackle details that may be too technical for rule-making."

And the National Association of Medicaid Directors is worried the multiple best price proposal could drive up costs for states. "Contrary to the positive impacts for manufacturers and commercial payers, multiple best prices conditioned on specific VBP parameters create significant risks for states," the group argued in its comments.

"It is not clear from the rule as drafted if states would default to having the option to participate in a VBP arrangement offered to a commercial payer," NAMD pointed out. "It is not even certain that manufacturers would be able to share the details of a VBP arrangement, as such information is generally proprietary. Nor is it clear if states would need to be in identical VBP arrangements with the same targets, metrics and outcomes to avail themselves of best prices realized between manufacturers and private entities."

CMS will need time to work through the issues flagged by stakeholders, which probably means there will be no near-term regulatory solution to the best price problem.

"We're in the second inning of a nine-inning game on this," Arnold & Porter pharmaceutical pricing and government contracts attorney Jeffrey Handwerker said in an interview. "We're at the beginning of the thought process here." Nevertheless, "it's terrific that CMS is thinking about the issue and making proposals to address what is a very real policy concern."

Handwerker predicted there will be further proposals over the next several years to remove regulatory obstacles to value-based pricing "no matter who wins the presidency, because it's really not a political issue." There will be "more and more cell and gene therapies on the market" going forward, he noted, so using value-based purchasing arrangements to manage their cost "is an issue that we're going to have to tackle as a country."