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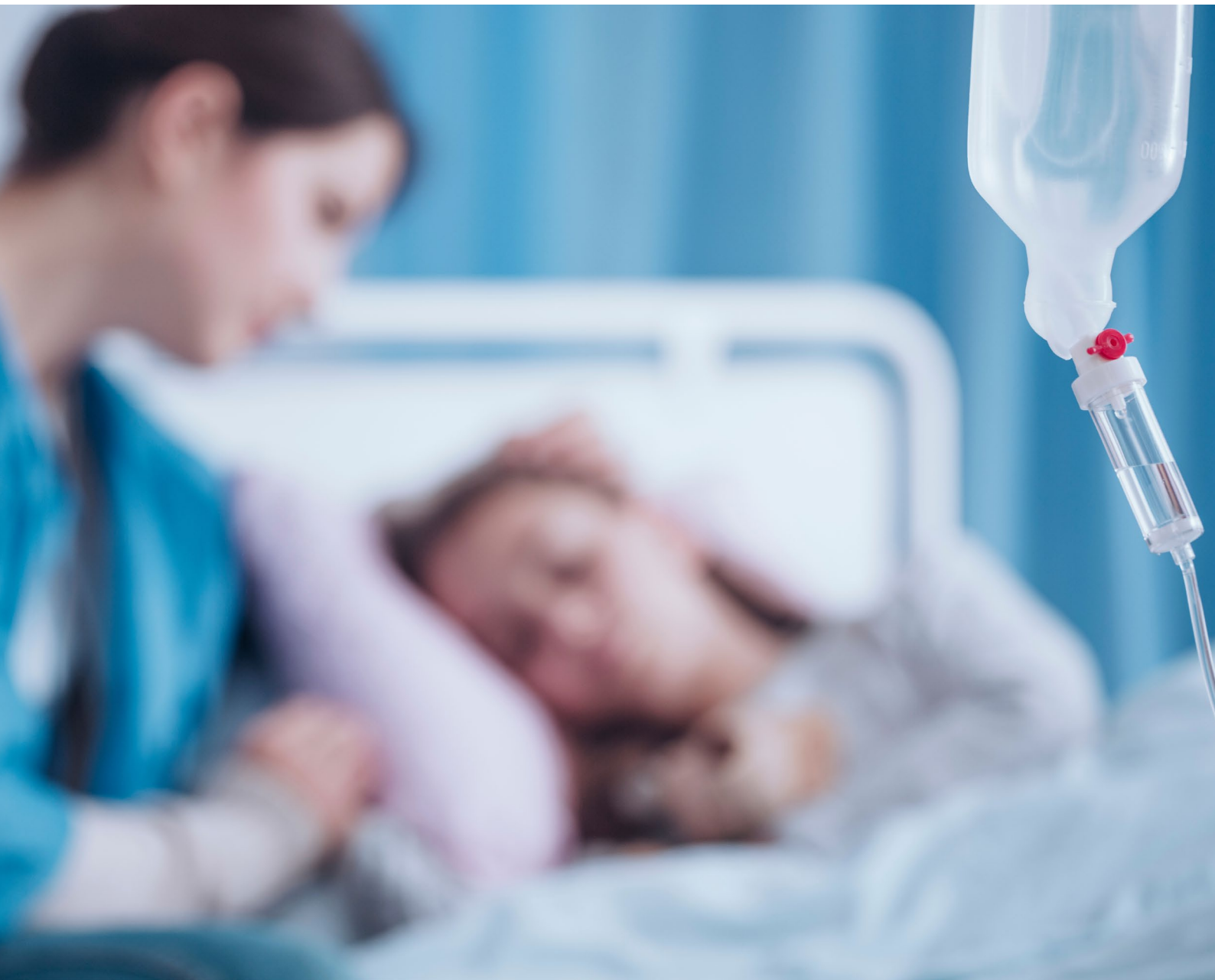
Toward outcomes-based payments:

The push to generate evidence and demonstrate value



Introduction

Pharma's R&D efforts have shifted from blockbuster drugs that help millions of patients with common chronic conditions to highly targeted therapies for rare diseases or specific cancer biomarkers. That is largely because these treatments offer a chance to address diseases that have a staggering economic burden.

Approximately 7,000 rare diseases impact 30 million Americans, or 9% of the population[1]. This amounts to nearly $420 billion in direct care costs annually, plus nearly $550 billion in indirect costs (things like productivity losses and reduced income for patients and their caregivers, along with the associated spend on transportation, home modifications, etc.). Cancer, which impacts[2] nearly 10% of Americans, contributed to $183 billion in direct care costs in 2015 and is projected to increase 34% to $246 billion by 2030[3].

The paradox is that treatments for these high-cost conditions often come at a high cost themselves – owing in no small part to the years of extensive R&D that goes into producing a highly targeted therapy for a small segment of the patient population.

Pharma's R&D efforts have shifted from blockbuster drugs that help millions of patients with common chronic conditions to highly targeted therapies for rare diseases or specific cancer biomarkers

The one-time cost for the spinal muscular atrophy gene therapy, Zolgensma, is $2.1 million, while the sickle cell anemia gene therapy Zynteglo has a set price of $1.8 million[4]. For small health plans or self-insured employers, a single therapy for a single member could represent a sizable portion of total annual medical spend. That could lead health plans to limit coverage of these treatments, which in turn could limit patients' access to potentially life-changing therapies.

Outcomes- or value-based contracts for individual therapies, which often stipulate that the amount a health plan pays for a therapy depends on how effective it is, offer one way to address this challenge. But their number remains small: Fewer than 80 had been publicly announced as of the end of 2021[5] (although the actual number, including those that have not been publicly announced, is likely higher).

The complexity of contracts and the time required to draft overall terms and outcomes metrics amenable to both sides – especially when it comes to the evidence needed to demonstrate overall efficacy and individual patient outcomes – has hindered their more rapid adoption.

However, many stakeholders within the life sciences industry suggest that these contracts are increasingly becoming the default standard for negotiation with payers. Despite the challenges, medical and finance teams say value-based agreements represent the best opportunity to provide the right therapies to the right patient at the right time, improving quality of life while reducing the long-term cost of care.

[1] https://everylifefoundation.org/burden-study/
[2] https://www.cdc.gov/nchs/fastats/cancer.htm
[3] https://aacrjournals.org/cebp/article/29/7/1304/72361/Medical-Care-Costs-Associated-with-Cancer
[4] https://www.biopharmadive.com/news/bluebird-sets-18m-price-tag-for-blood-disease-gene-therapy/556923/
[5] https://www.pharmexec.com/view/value-risk-and-reward-taking-stock-of-value-based-contracts-in-pharma

How today's drug pipelines will influence tomorrow's outcomes-based contracts

PhRMA's list of value-based contracts publicly announced in the United States indicates that the first contract was signed in 2009, between Merck and Cigna, for the type 2 diabetes therapies Janumet and Januvia. Only 10 contracts were signed from 2009 to 2014. The pace began to pick up the following year, with eight contracts penned in 2015 and 10 or more in each year that followed.

Initially, value-based contracts tended to focus on a range of common chronic conditions, from type 2 diabetes and high cholesterol to multiple sclerosis and hepatitis C. There was no hard and fast rule for whether a given therapy or condition was a good candidate for a value-based contract; it depended largely on the shared interest between the payer and manufacturer.

Both parties set outcomes at a population level, looking at general metrics such as number of hospitalizations, and relied largely on claims data. "Outcomes-based contracting has been a small part of the toolbox. It's rarely been as big as the rebate," says Lisa Carman, PharmD, Vice President, HEOR and Value-Based Contracting, Optum Life Sciences.

"However, the emergence of high-cost but potentially curative therapies in oncology and rare diseases is changing the market dynamics. It's putting an emphasis on how the therapies are working, and what is their value."

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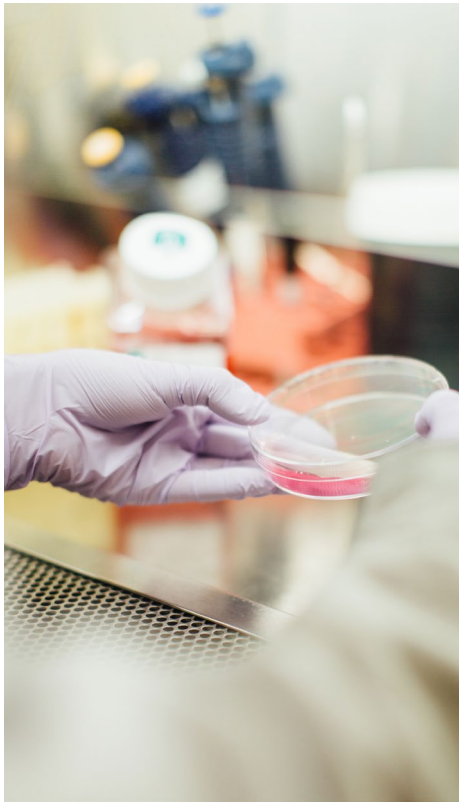
The business case for outcome-based contracts in oncology and rare diseases

Today's life science organizations are increasingly focusing their R&D pipelines on highly targeted treatments for individual genetic mutations or biomarkers that cause cancer and a range of rare diseases. Across the industry, there are nearly 1,800 gene and modified cell therapy targets in manufacturers' pipelines.[6]

Increasingly, these therapies call for outcomes-based contracts in which life science organizations and payers share financial risk based on clinical outcomes on which all parties agree. If the patients taking the therapies don't achieve these outcomes, or if patients need more doses of a medication than the cap established in the contract, then the payer isn't required to pay the drug's full price. This is a critical difference from outcomes-based contracts for common conditions: Outcomes are determined at an individual level, not a population level.

"More and more, the high-cost therapeutics are where payers are initiating the discussion, and narrowing the targeted population has become a more targeted approach," says Marit Hansen, Corporate Account Director, EMD Serono. "Payers are saying that they're still interested in traditional access-based rebates, but within their specialty spend, they're looking to ensure outcomes. That's where they're looking for value-based agreements."



> "The treatments work so well. Even though they're expensive, there's certainty. Everyone buys into the value. Why complicate things?"
>
> Kasem Akhras, PharmD, Senior Director of Translational Access, Geneconomics & Outcomes Research for New Products, Novartis Gene Therapies.

[6] https://www.optum.com/business/resources/library/forum-life-sciences.html

Where outcomes-based contracts make the most sense

Admittedly, not all targeted therapies are candidates for these types of outcomes-based contracts. This is typically the case if value has been established, due to either an abundance of treatment options already on the market (as is the case with high cholesterol) or a high success rate despite a high cost. Here, hepatitis C is a commonly cited example: Though treatment costs range from $54,000 to $95,000, the range of approved therapies have a 95% cure rate. "The treatments work so well. Even though they're expensive, there's certainty. Everyone buys into the value. Why complicate things?" says Kasem Akhras, PharmD, Senior Director of Translational Access, Geneconomics & Outcomes Research for New Products, Novartis Gene Therapies.

Not all payers may be ready, either. "It depends on where the organization is on their journey. If a payer already has value-based agreements in place with physicians and providers, then collaborating on these types of agreements with pharma is a natural progression," says John Struck, Vice President and Head of US Market Access and Value, EMD Serono. "If they are early in their journey, then they may be in a place to measure adherence as opposed to outcomes. It's important for us to understand our customers' needs and where they align with that value proposition."

Still, there are several circumstances when it makes sense for payers and manufacturers to explore an outcomes-based contract (see Sidebar). Many of these scenarios have two things in common: There's uncertainty about the future, whether it's about costs or outcomes, and there's a concern that patients will miss out on the treatments they need.

"Stakeholders are reluctant to take on financial risk in a VBA without a commitment from the manufacturer to deliver the desired outcome," says Joanne Sellner, Associate Vice President of National Accounts, Lilly USA. "Value-based arrangements give us the opportunity to stand behind the value of our medicines – and ensure that patients will get access. That's important, because these aren't therapies that a physician is going to pick from a formulary."

Other industry stakeholders agree. "When we use outcomes-based contracts where it's appropriate, we're able to expedite patients' access to life-saving therapy," Akhras says.

Added Allison Shimooka, Senior Vice President of Strategy and Product Innovation, Optum Life Sciences, Optum: "We all come to the table with the same goal, which is increasing access for patients. We have to start there. The potential is incredible – but if we're not willing to engage, we'll be limiting access."

Ensuring that the right patients have access to the right therapies, and that those therapies deliver on their desired outcome, requires a significant shift in the way payers and manufacturers gather evidence. Increasingly, both groups of stakeholders see value in collecting data as soon as possible in the product development process.

Common scenarios for an outcomes-based contract

A therapy may be a good candidate for an outcomes-based contract if it meets one or more of the following conditions:

- Therapy requires post-market, real-world assessment due to limited clinical trial size
- Disease or patient(s) cannot be tracked using traditional claims data or ICD-10 codes
- Manufacturer is first to market with a therapy for a given disease
- Access to potential life-saving therapies for a given disease is otherwise limited
- Uncertain and unpredictable treatment costs on behalf of payer and its customers
- Uncertain clinical outcomes suggest risk in paying full price for a therapy
- Therapy is administered once and results may not be realized for several years

The benefits of getting a head start on evidence generation

Traditionally, manufacturers start to think about generating real-world data and evidence of efficacy during Phase III of a clinical trial. This approach is understandable, as manufacturers prefer to ensure that a therapy has a high likelihood of reaching the market before investing in the resources necessary to gather additional data.

For targeted therapies that are likely to be covered in an outcomes-based contract, though, evidence generation needs to start sooner. For starters, by Phase III manufacturers tend to be focused largely on regulatory approval; they may de-prioritize additional evidence generation at that stage, only to wonder in hindsight if they may have missed something. The sooner the discussion about collecting data occurs, the sooner that manufacturers can communicate with regulators and understand what data they'll need to expedite the approval process.

Beginning with the end in mind

The other reason to act early is because gathering data in the early stages of product development and clinical trials can better inform the design of future trials – and future contracts.

"We're much more keen to know, early on in the program, what payers are going to want to see. If you launch, but you don't have the right metrics, you have to go back and collect them, and that takes years," says Shirley Bachman, Vice President of Market Access, Alnylam. "You have to gauge how the payer and their customers are going to think. Anything you can do to align with that way of thinking will help build your organization's brand and gain respect."

For stakeholders to agree on the terms of an outcomes-based contract, there needs to be a shared understanding of how a clinical trial's endpoints will translate to a therapy's value proposition in the real world.

This is especially important for rare disease therapies or orphan drugs. Clinical and financial outcomes tend not to be well understood, as therapies often address conditions that have never been treated before. In addition, it may take several years – far longer than the duration of the average clinical trial – to measure the true long-term impact and benefit of a therapy. As a result, manufacturers should be prepared to make clinical trial endpoints available early to their internal medical affairs, market access, and financial teams. This way, medical affairs and finance can create risk models in the early stages of negotiating payer contracts and update those models as additional trial data comes in – effectively connecting the clinical trial and the payer contract.

"Starting early is critical, because that allows you to generate evidence to better understand the natural history of the disease and the unmet medical need. Real-world evidence is a critical piece of outcomes-based agreements," Novartis' Akhras says. "With the evidence from the clinical trial, you can discuss outcomes – and design the subsequent trials to support not just regulatory approval but also pricing and reimbursement models."

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Shirley Bachman, Vice President of Market Access, Alnylam

Treating data aggregation as a team sport

Real-world evidence plays a key role in measuring outcomes as patients take and respond to a therapy, as this determines whether a patient has achieved the desired outcomes and whether a payer is eligible to recoup some of the total cost of a therapy.

Unlike common chronic conditions such as diabetes and high cholesterol, few rare diseases have a distinctive ICD-10 code that can be easily tracked in medical claims. In addition, it may take several years to determine if a gene therapy or other targeted drug has 'worked'. That requires ongoing monitoring of a patient's condition – and under most outcomes-based contracts, this must continue even if a patient has switched insurers. Finally, patients who qualify for these therapies tend to see many providers at many healthcare facilities; payers cannot expect that obtaining usable data from all of these providers will be straightforward.

These challenges make it critical to collect and analyze data on a range of well-defined metrics, from medication persistence and adherence to switching treatments, as well as those that are less defined, which often include a range of patient-reported outcomes (PROs) linked to side effects and overall quality of life.

Having the infrastructure in place to gather, harmonize, and store data is critical to the success of outcomes-based contracts, regardless of whether organizations assemble this infrastructure in-house or acquire it from a third-party technology vendor. "It's one thing to design and sell the agreement, but if there's no way to aggregate the data, you won't be in the value-based care business very long," Alnylam's Bachman says.

Likewise, analyzing data – especially data coming from disparate sources that payers and manufacturers alike traditionally don't look at – depends on the availability of state-of-the-art technology. "This isn't a quick query. You need to be able to build algorithms, and they need to be validated because, with very rare diseases, you're often working off other biomarkers," says Optum's Carman.

Aggregating and analyzing data is a collaborative effort both within the life science organization – as the onus is in efficacy and safety teams, not just the regulatory team – and among payers, and patients. (Notably absent from that list: Providers. Stakeholders agreed that requiring clinical staff to provide outcomes data above and beyond what they're already required to report would be an unnecessary burden.)

"It's not a one-sided thing. Many decision-makers need to come together to ensure that data is captured and made available the right way," EMD Serono's Hansen says. Collaboration also helps both the payer and the manufacturer create transparency around each other's data, she adds. "The more impactful real-world data that we can get, the more believable it is for the payer. Typically, they'll take the lead on collecting data and generating reports, and then we come together to analyze performance. When we look at the results as a team, that's more impactful."

Increasingly, stakeholders engage directly with patients to obtain outcomes data – particularly for quality-of-life metrics, such as quality of sleep, that are nearly impossible to track through retrospective clinical or claims data. Novartis, for example, has set up a patient hub through which it conducts patient/family outreach and gathers PROs[1]. This has proven valuable for patients being treated with Zolgensma, as the progression of spinal muscular atrophy must be closely monitored, especially in young children.

Takeda's Medical team, meanwhile, is working with payers to develop and offer smartphone applications that let patients share their PROs with payers. "Data can often be a limiting factor, so it's important for us to align on the data sources that are readily available," says Mark Gimbert, Head of Managed Markets Accounts and Strategic Partnerships, Takeda. "Since patients with rare diseases tend to want to be more involved in their care, this will provide another source of data that can be aggregated by payers and analyzed with manufacturers to improve patient care."

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[1]https://app.box.com/s/8va3l5of6ji0z5mz5w3kvavy9f9dguii

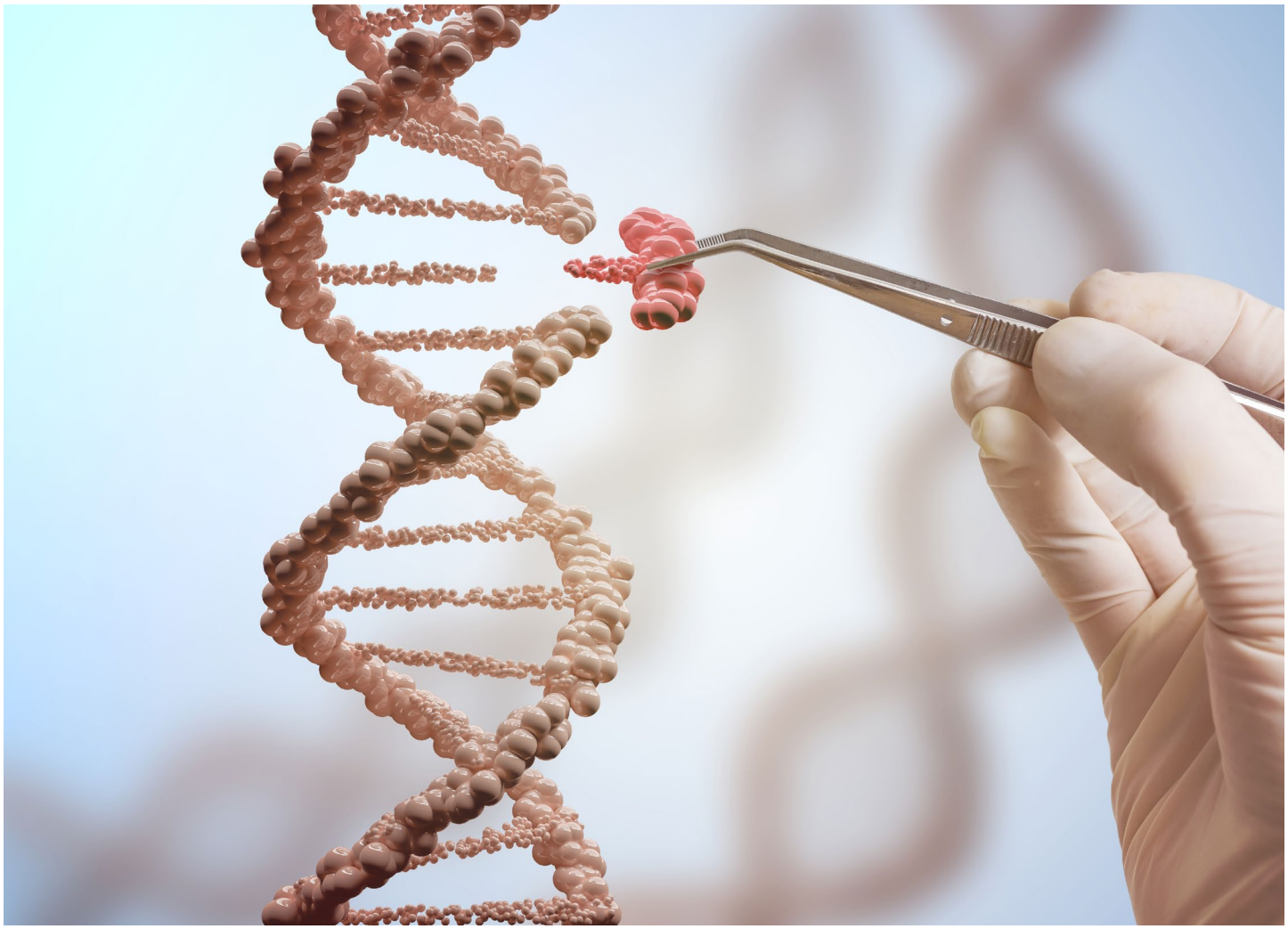
The role of evidence in articulating the value proposition

Gathering data and generating evidence offer value beyond the existing contract for a given therapy. When payers understand how a drug is performing within their current patient population, that provides useful insight for making future coverage decisions. This could be applied to a new outcomes-based contract for a similar type of therapy, or to an updated contract that introduces a new risk model based on an analysis of a therapy's performance so far.

"We need the ability to track how a therapy is working," says Optum's Shimooka. "The data helps you figure out who it's working for, and who it's failing for. It helps you determine if the failure was a medical factor or some other factor, or if it was something the therapy had actually addressed. That starts to get everyone aligned on therapeutic success."

Defining success is one of the biggest challenges associated with outcomes-based contracting. It varies from one stakeholder to another, from one therapy to another, and from one patient population to another.

"The challenge is all the extra variables: The data collection, the regulatory hurdles, the reporting, and the contracting terms," says Dan Van Horn, Senior Vice President of Market Access & Patient Solutions, EMD Serono. "It's not always easy, but if you can align on objectives and metrics that provide value for both sides, that's where you find a match."



Moving from clinical to business value

Manufacturers start to define the value of a therapy based on its cost effectiveness, safety, and quality of life impact in a real-world setting. That's the clinical component. From there, manufacturers and payers can get down to business.

"What we deliver to the marketplace rests on the ability to deliver the efficacy that patients and providers want – but that's the minimum," says Eric Dozier, Vice President and Chief Commercial Officer, Lilly Oncology. "It's also important to communicate the economic story. The healthcare marketplace is so large. How do we stand out? We make sure we address quality metrics and the total cost of care, and we use real-world evidence early on to articulate this. That's how we show that it's worth it."

As with other conversations about outcomes-based contracts, the sooner the value discussion happens, and the more transparency there is about the data and evidence being used to measure, value and track outcomes, the less likely it is that there will be a dispute down the line.

The discussion is around areas like timeframes and milestones to track for a certain disease. Then we go into more practical considerations, such as contract implementation, measurement and tracking, verification of outcomes, and how the contract fits within the frameworks in each country," says Burcu Kazazoglu Taylor, Senior Director of Global Patient Access, Novartis Gene Therapies. "From formulation to contract design to implementation, it takes significant time to get off the ground."

Predictability is a critical component of defining a therapy's value to payers, according to Alnylam's Bachman. This is especially true for categories of therapies with which they are unfamiliar.

"The more you can do up front, the better. Tell them what the primary endpoint was, and how you measured it. If the payer understands the clinical attributes of the product, the appropriate patient, and they feel good about the product profile, that's your entry point," she says. "And to execute the contract, make sure whatever you put in is measurable. Make sure you can submit your data to capture the outcomes measures and tie them to clinical endpoints that a provider can submit claims on."

Manufacturers should also take the time to understand that different types of payers will have different interests and needs regarding outcomes-based contracts.

A commercial payer covering small and medium-sized businesses, for example, is likely to have stop-loss insurance in place to protect against large, unanticipated claims. High-cost therapies for previously undiagnosed cancers or rare diseases certainly count, but not all stop-loss plans will cover them.

Meanwhile, Medicaid managed care plans must contend with high rates of member churn as individuals' eligibility for Medicaid changes. Small and medium-sized health plans are likely to have limited experience with outcomes-based contracts, along with limited resources to draft them. This further emphasizes that the value proposition can differ dramatically from one payer to another.

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Understanding the total cost of care

Another important consideration in the value conversation is the total cost of care, not just the total cost of the therapy. To increase the likelihood of hitting outcomes targets, manufacturers may pay for things that payers traditionally cover.

One manufacturer agreed to cover wraparound services for patients taking its hepatitis C medication. Some manufacturers are also paying for BRCA genetic testing to better identify patients who would benefit from targeted gene therapies for cancer.

Takeda's outcomes-based contract with Prime Therapeutics for the hemophilia treatment Advate® takes a similar approach. The agreement takes into account the total cost of care, including emergency department visits for care episodes such as unexpected bleeding.

"It's not a reimbursement for medicine. It's not just signing and submitting quarterly reports. It's a more continuous relationship that looks at patients more holistically," Gimbert says. "The agreement is built to allow us to access aggregate clinical outcomes and data on cost implications – and to share financial risk with our customers if the total cost of care for a patient on treatment exceeds expectations.

"Because we have rich data, we can also take a hard look at why costs for some patients may have exceeded expectations. Are they not taking the therapy? Do they have comorbidities? Could their physician be playing a more prominent role?"

Tracking the total cost of care has an additional benefit: It keeps stakeholders accountable. "Ultimately, we want to know whether these therapies work. That's why figuring out how to track patients longitudinally is so meaningful," Optum's Shimooka says. "Otherwise, we're just driving up collective costs – and we're deliberately working against each other."

The future of reimbursement in the age of precision medicine

Many manufacturers indicate that they increasingly regard outcomes-based contracts as their default for negotiating with payers. Lilly USA's Sellner says the company began initiating value-based arrangements in 2014 and now has a multitude of VBAs across therapeutic areas. She notes that Lilly contemplates VBAs for every new asset as part of launch readiness. Representatives from EMD Serono, Novartis, and Takeda says their companies try to leverage outcomes-based contracts whenever possible, recognizing that these contracts are not appropriate for all therapies or payers. Alnylam's Bachman says the company (founded in 2002) placed an emphasis on outcomes-based contracts before its first rare disease therapy came to market. "Value-based care is here to stay, and we intend to be true to where we started," she says.

Three trends help to explain stakeholders' increased interest in these types of reimbursements:

1. New best-price rule offers flexibility in pricing
2. Research and development pipelines focused largely on targeted therapies for rare diseases and orphan drugs
3. The potential to apply to mass-market therapies the advances in data gathering and analysis being used to measure outcomes for targeted therapies

New best price rule offers flexibility in pricing

On July 1, 2022, an evolution to so-called "Medicaid best price rule" went into effect[7]. The Trump administration originally proposed the modification, with an effective date of January 1, but the Biden administration delayed the rule by six months to allow manufacturers and government agencies more time to prepare.

The new rule builds on the longstanding Medicaid Drug Rebate Program (MDRP), through which manufacturers provide rebates to state Medicaid programs. Under the initial MDRP, signed into law in 1990, manufacturers had to report a single "best price" to the government for a given brand-name drug. Generally, this is the price that a wholesaler, retailer, or provider pays for the drug. This has been viewed as detrimental to outcomes-based contracting, as a deep discount for a therapy written into a contract would become the new, low best price.

The new rule provides flexibility to manufacturers, who are now able to set multiple best prices for a therapy. This allows them to better negotiate outcomes-based contracts with Medicaid and other payers. In announcing the rule in December 2020, the Centers for Medicare & Medicaid Services (CMS) estimated the new best price approach would encourage outcomes-based contracts that would yield $228 million in savings over a four-year period.

"Manufacturers had been hesitant to provide what amounted an across-the-board discount. Now, CMS is saying there are some exceptions when it comes to value-based contracts," Optum's Carman says. "There are still some nuances that need to be figured out, but the thought is that this will remove the inhibition to take on risk."

[7]https://www.medicaid.gov/prescription-drugs/downloads/mfr-rel-116-vbp.pdf

A new standard for doing business

As discussed, the life science industry is shifting its pipeline from blockbuster drugs to highly targeted treatments. According to data from Optum, there are roughly 1,800 precision therapies in the drug development pipeline at the end of 2020. Though the U.S. Food and Drug Administration has approved more than 1,000 orphan drugs, that leaves several thousand rare diseases without an approved therapy.

This momentum is only increasing interest in outcomes-based contracts. In fact, as manufacturers gain regulatory approval and bring therapies to market after clinical trials that may only enroll as few as 20 patients, outcomes-based contracts may be a matter of necessity for obtaining coverage from payers who are uncertain that these often very-high-priced therapies will deliver on their promise to each individual patient.

"We expect that this will be the industry standard for therapies targeting smaller patient populations," Lilly USA's Sellner says. "For example, in oncology, it's less about prevention and more about whether the therapy that a payer is spending money on is going to work, and whether the patient is going to benefit. We want to stand behind the value proposition of our products and prove that clinical trial results translate to real-world settings."

Because each outcomes-based contract is unique, stakeholders may feel as though they are in a "perpetual pilot" phase, a phenomenon similar to the implementation of technology innovations. While this can pose a host of operational obstacles, it also offers the opportunity to reapply best practices, avoid the failings of past contracts, and increase the organization's willingness to take on risk in an incremental fashion. "Existing agreements are a good place to collect data and support value demonstration. You can learn from what's already in place," says EMD Serono's Van Horn.

A return to population-level outcomes tracking

Finally, manufacturers and payers see potential to extend the data-sharing scale that's necessary to track utilization, costs, and outcomes for targeted therapies into mass-market therapies that have typically been assessed based on population-level outcomes. For example, the ability to broadly assess how patients with conditions such as type 2 diabetes or hypertension have responded to previous treatments could enable more targeted approaches to prescribing certain therapies to certain sub-segments of the patient population.

The challenge is determining the metrics necessary to establish value. Since even a sub-segment of the type 2 diabetes population is likely to be larger than an entire rare disease population, outcomes may need to be less specific and more ubiquitous. However, Alnylam's Bachman says the process for gathering population-level outcomes is similar to the process for gathering patient-level outcomes needed for rare diseases.

"I don't think it matters whether the population is large or small, especially as data collection becomes more of a mainstay in the process of managing outcomes-based contracts," she says. "If anything, it's potentially less complicated for mass market therapies, since there are more identifiable endpoints and metrics. Plus, manufacturers are routinely looking at and publishing health outcomes – and sometimes in tandem with payers."

This level of collaboration will be critical as stakeholders continue to enter outcomes-based contracts and monitor patients for months (if not years) to come. "Manufacturers and payers are realizing that we have to solve things together," Takeda's Gimbert says. "Organizations are going to be more open about sharing data and more transparent about how the dollars are flowing, and how that's impacting patients."

A rallying cry

Although the adoption of outcomes-based contracts has expanded somewhat slowly, the potential for these contracts to become the default expectation in certain drug classes or therapy areas is very real.[8] R&D pipelines are full of targeted therapies with life-changing potential, but even the ones that achieve regulatory approval will likely come to market with limited clinical trial data available at launch. For high-cost drugs that help combat complex, high-cost conditions, interest in these innovative contracts will almost certainly continue to grow.

By tying reimbursement to objective measures of drug effectiveness, each stakeholder in the care ecosystem is taking steps to enable access to treatments that can improve the quality of life for both patients and their caregivers. "At the end of the day, we're looking to raise the standard of care beyond the medicine itself," says Lilly Oncology's Dozier.

Industry experts recognize that these contracts are complex and, like any new systemic standard, come with their own set of challenges. These complications range from the physical (like geographic dispersion) to the behavioral (like medical documentation) to the psychological (like a willingness to take on risk). "You have to put the value in the context of the complexity," says Novartis's Kazazoglu Taylor.

But as more payers and manufacturers collaborate on innovative outcomes-based contract models, they'll learn valuable lessons about identifying and tracking endpoints, allocating risk, adjudicating contracts and working together. Over time, these learnings will inform future contracts and collaborations, ideally lowering the burden of complexity and giving way to a wider range of risk-sharing arrangements.

While each individual rare disease impacts a small population, the 9% of Americans who are impacted by rare conditions need new ways to get treatments. Contracting models anchored in shared definitions of value and measures of impact can not only expand access to life-changing therapies, but they can also help improve the efficiency of our health care system writ large: a true win-win-win scenario for the life sciences, payers, and—most of all—patients.

[8] https://icer.org/news-insights/press-releases/icer-publishes-evidence-report-on-gene-therapy-for-beta-thalassemia/

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