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Orphan Medicines: Averting Price Debacles And Winning Payer Support

Thought Leadership In Association With Syneos Health

by

As coverage and reimbursement landscapes change, drug developers must assess how healthcare systems will handle the incoming wave of treatments for rare diseases that often carry a high list price, and they should prepare for restrictive practices that pass more of the costs along to patients. Developers can speed orphan medicines to patients through better communication of value to payers and other stakeholders.

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After months of withering scrutiny over pricing, orphan drug developers must have breathed sighs of relief when they read President Trump's "Blueprint to Lower Drug Prices." Released in May, the document makes no mention of rare diseases, and in 39 pages the phrase "orphan drugs" appears just once. Many of the proposals in the Blueprint tackle discounts and rebates that arise mainly when there are multiple therapies to treat non-rare conditions.

Dodged bullets aside, however, the market access environment for orphan drugs is changing quickly in ways that will not reward complacency. With orphan-designated therapies projected to make up one-fifth of global prescription drug sales by 2024 [i], payers are steeling themselves for a wave of treatments with high list prices.

The US Food & Drug Administration (FDA) is making moves to approve orphan drugs using smaller, efficient development programs—action that is feeding the surge of new rare disease drugs. But "approvable" to regulators doesn't mean payers will provide unfettered access. In fact, patients with rare diseases are already facing a number of coverage and reimbursement hurdles that seem poised to intensify in coming years. The good news: developers can mitigate some of these obstacles by wisely investing time and money during clinical development to substantiate the value of the new drugs and accurately communicate this information on the runway to commercialization.

Among restrictive tools and practices, co-pay accumulator programs have drawn criticism from patient organizations because the value of co-pay cards and other assistance cannot be applied to patient deductibles or out-of-pocket maximums—leaving patients exposed to more of the cost. [ii] Already, close to one-fifth of self-funded employer plans may be using such programs, and the number could rise to 50 percent by the end of the year, analysts predict. [iii]

Other practices that may be on the rise include prior authorizations applied in expansive ways to restrict coverage well beyond subsets of patients who were excluded from clinical trials, and delays in coverage decisions by health insurers that have the impact of rationing care. Admittedly, reviewing new drugs takes time. This is especially true when accelerated regulatory reviews leave payers with less time to prepare and limited evidence to help them manage uncertainty. Still, coverage delays that can stretch to half a year put an undue burden on patients who rely on "fast start" or bridge programs the developers craft to make drugs available during the gap.

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Misaligned Perspectives

The restrictive practices highlighted are not intended to be combative, even though some drug developers see them as blows across the bow. The practices often arise from misalignments in perception of a medicine's value—which is always in the eyes of the beholder. Insurers, pharmacy benefit managers and employers apply restrictions to products when they do not have enough information on the benefits and durability of response to justify the cost.

Small developers, in particular, often miss precious opportunities to plan for and provide the education and evidence payers require at key junctures in the development process. With limited resources, these companies sometimes focus on communicating potential value to investors and regulators. But, in today's increasingly price-sensitive and restrictive environment, it is important to learn how payers, prescribers and patients define value too. Developers must be able to substantiate and communicate in metrics and language each group understands—and not to wait until the drug is approaching launch.

Outreach to payers requires particular forethought. Early in the development process, orphan drug developers should be gathering evidence payers will find compelling. This ranges from data on disease prevalence, the number of patients who might be in the payer's plan and the costs for hospitalizations and co-morbidities in the absence of a new medication, to the impact on payers' budgets and how the drug will reduce cost burdens to yield savings. By 18 months to a year before approval, the drug developer should be finalizing this information and figuring out how to document and tell a persuasive story.

The Payer's View

Evidence of this sort takes time to develop. Health economics outcomes research (HEOR) can help, and can be especially persuasive when validated by institutions the payers trust. By publishing and presenting this evidence throughout development and commercialization, developers can build a peer-reviewed story of the disease burden and its economic consequences.

James T. Kenney, RPh, MBA, Manager of Specialty and Pharmacy Contracts at Harvard Pilgrim Health Care, says seeing this information before a drug clears FDA review can make a big difference on coverage decisions and timelines.

"If we're budgeting 12-18 months in advance, it's nice to know what's coming so we can put it into our budget models," Kenney told us in a recent interview. With orphan drugs on an accelerated pathway, the best time may be when the manufacturer emerges from Phase II, while there's still a chance to build specific outcome measures into the testing. "That would help us evaluate the drug and figure out how to use it, which is the real spirit of cooperation and collaboration," he says. "Then, instead of coverage decisions coming six months after launch, maybe we could shorten the timeframe and identify appropriate patients earlier and allow faster access to these agents."

Some of the communications Kenney describes are constrained by the FDA's guidance on Section 114 of the 1997 FDA Modernization Act (FDAMA 114). As President-Elect of the Academy of Managed Care Pharmacy, Kenney is one of many industry executives—payers and pharma companies alike—who wish to see Congress ease the rules and free up necessary communications.

The Patient View

A true multidimensional approach must also take stock of how patients and prescribing physicians get information and respond to news. Consider, for example, a small biotech company whose treatment for a genetic disorder has reduced hospitalizations to zero in a trial lasting 18 months. Enthusiasm gets the best of the company's chief executive. In an earnings call with analysts and media, he discloses an internal proposal to price the drug at $500,000 per treatment.

A decade ago, an earnings call like this might have had few consequences other than raising spirits on Wall Street. Today, messages from a CEO to investors are instantly propagated across a network of highly engaged patients.

Furthermore, suppose that in this call the CEO makes no mention of patient assistance programs or any positive feedback from health plans—a serious mistake. Instead of winning the support of patients, the pricing message aimed at investors triggers despair in people who now fear they can never afford the drug. Patient groups are activated as critics when they might have been allies of the developer. These groups may now be less likely to assist the developer in charting the natural history of the condition, building registries, recruiting patients in trials, and making a case for the value of the drug.

The most serious consequence of missteps is delaying and diminishing the prospects of access to a new drug. "People forget that being a patient is full-time job," explains Jen Horonjeff, PhD, CEO and founder of Savvy Cooperative, an organization that seeks to strengthen partnerships between patients and drug developers. Poor communications among stakeholders "is wildly detrimental to patients gaining access," Horonjeff said in an interview. "It's the patient that gets hurt in the end."

Lack of access is especially cruel at a time when science is delivering mind-spinning advances in atomic-level molecular analysis, DNA engineering and related progress in gene- and cell-therapies. That is why developers and payers alike must find solutions to ensure access while preserving the viability of multiple, co-existing business models in a free-market healthcare environment. When conversations about value are grounded in evidence that is gathered, validated, and communicated with high levels of confidence, solutions will be within reach.

To read more about key factors shaping the U.S. market landscape for rare diseases, download the free full report at *www.syneoshealthcommunications.com/orphandrugvalue*

About the Authors

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[i] EvaluatePharma. (2018, May 25). *Worldwide Orphan Drug Sales are Forecast to Grow at a CAGR of 11.3% from 2018 to 2024, Double the Rate Forecast for the Non-Orphan Drug Market* [Press release]. Retrieved from *www.evaluategroup.com*

[ii] Franklin, E. (2018, April 16). *Copay Accumulator Programs: What's at Stake for Patients?* Retrieved from *www.cancersupportcommunity.org*

[iii] Silverman, E. (2018, April 18). *Which drug makers are most vulnerable to a new cost-shifting maneuver?* Retrieved from *www.statnews.com*