Volume 11, Number 11 November 2014

SPECIALTY PHARMACY NEWS

News and Strategies for Managing High-Cost Specialty Products

With Orphan Drugs' Launches Increasing, Payers Need Effective Management

Orphan drugs likely do not figure prominently on health plans' radars. After all, these are therapies used in conditions impacting fewer than 200,000 people in the U.S., so an average plan may not have many members taking such products. But as more of these drugs launch in the U.S., payers should make sure they have policies in place to ensure appropriate use of the costly therapies.

The term "orphan drug" was established with the passage of the Orphan Drug Act in 1983. Because these medications treat such small patient populations and thus may generate less in sales than drugs for larger populations, that law provides incentives to manufacturers to develop these treatments. Benefits include federal funding of and tax credits for clinical trials, as well as a seven-year period of exclusivity on the U.S. market, during which these drugs will not face competition. Since the law was passed, oncology-related conditions have been the most common focus of orphan-designated drug approvals.

The new EvaluatePharma Orphan Drug Report 2014 by Evaluate Ltd. found the following:

- Orphan drugs could account for 19% of total prescription drug sales excluding generics by 2020, with $176 billion in annual sales worldwide.
- Sales of orphan drugs in the U.S. are expected to grow at an annual rate of almost 11% through 2020, compared with approximately 4% for drugs to treat larger patient
- populations.
- The FDA approved 16 new orphan drugs in 2013, down from the 22 it approved in 2012, but those 16 represented 46% of total new drug approvals in 2013.
- The FDA approved 32 orphan designations in 2013, up from 25 in 2012.
- u The average per-patient per-year price for orphan drugs in 2014 is $137,782, compared with an average of $20,875 for a non-orphan drug.
- Orphan drugs' median cost per patient differential is 19 times higher than that of non-orphan drugs.

"With the growth of new drugs to treat orphan diseases, the overall specialty pharmacy market is increasingly targeting smaller patient populations," points out Stephen Cichy, founder of and managing director for Monarch Specialty Group, LLC. "In 2014, the median patient population size being served by a top 100 drug is 146,000, down from 690,000 in 2010."

"The sheer number of new orphan approvals point to the success of orphan drug legislation," says Cichy. "However, this puts big pressure on payers regarding budgeting and reimbursement policy. Ten years ago I'd say the general viewpoint among most payers was that because orphan drugs target small populations, their impact on the pharmacy budget was fairly limited. "But today, he tells SPN, "I'm seeing payers look at orphan drugs differently in light of more approvals, expansion of indications and higher prices. What's more, due to orphan drugs targeting rare diseases and a smaller universe of potential patients, it's harder for manufacturers, distributors and payers to accurately predict volume and actual claims to be paid each year. If there is a [management] trend, I'd say it's more toward higher patient cost sharing and more imposition of conditions of reimbursement."

According to Bill Sullivan, principal with Specialty Pharmacy Solutions LLC, health plans approach management policies the same way for all drugs: They have their pharmacy and therapeutics (P&T) committees review them. “Orphan drugs tend to go through the process a little faster, as they usually are in categories with no current competing therapy or old therapies that merely manage symptoms as opposed to modifying the disease process,” he says. “Once P&T approves a drug, then the benefits people decide how to treat it as a covered benefit. That is when cost and patient liability is determined. The medical department also determines any prior-auth requirements. Sometimes these activities are comingled.”

“While drug-specific characteristics such as safety and efficacy or market factors may be evaluated by plans through its P&T committee, it’s more than likely that an orphan drug obtains formulary status if FDA approved”. Because many of the conditions don’t have alternative treatments, it’s highly unlikely that a plan would exclude an orphan drug from its formulary. “Most payers will require prior authorization as a condition of reimbursement. In addition, payers may employ more stringent conditions, such as on-label indication restrictions, step edits and/or quality limits to control
for utilization.”

Sullivan says he is not aware of a case where a plan didn’t cover an FDA-approved orphan therapy. But in situations where numerous treatments are available, such as Gaucher’s disease, plans may choose to have a preferred therapy, which could be motivated by manufacturer rebates, he says. “Lower prior-auth barriers and lower patient out-of-pocket are the common methods to push selection of a preferred product on a plan formulary.”

When evaluating an orphan drug, “plans would first want to know how many members are diagnosed with that condition,” Sullivan tells SPN. “Many members with orphan conditions are already high-cost members (for acute care — office visits, ER, hospital admissions, home care, etc.) so a new — even costly — therapy may be a ‘net lower cost’ alternative, especially if the therapy reduces acute care costs. In cases like that, the plan may be very proactive in having network physicians prescribe the new medication.”

One potential challenge with orphan drugs is that many “have REMS [i.e., Risk Evaluation and Mitigation Strategy] requirements, black-box warnings or potential severe side effects,” notes Sullivan, so plans would want patients on such therapies to be closely monitored. However, “a specialty pharmacy would likely be handling this already, as most orphan drugs are available only through limited distribution, and the manufacturers are requiring the specialty pharmacies to be on top of these patients regardless,” he points out.

In addition, “A major challenge for a payer in dealing with orphan drugs is finding a right balance between product access and patient out-of-pocket cost when the treatment costs hundreds of thousands of dollars,” explains Cichy. “This is especially relevant for orphan drugs that are subject to coinsurance.” For example, if plan has coinsurance of 40% for its top tier, which is likely to be the tier in which orphan drugs are placed, members are looking at tremendous out-of-pocket costs for drugs that may cost hundreds of thousands of dollars
per year.

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