

COVERAGE AT THE CROSSROADS

The prescribing of orphan and specialty drugs is at a crossroads, where access issues are acutely felt—and things may get worse. As these drugs account for a larger part of total spend, insurers say a barrage of tactics are needed to manage costs. **Sarah Morgan** on why this is a defining moment



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Orphan and specialty drugs are, by definition, drugs with which the majority of patients will never concern themselves. Nor will the majority of physicians. But when orphan and specialty drugs are necessary, patients already facing a problematic condition find themselves handling a slew of additional problems.

These drugs tend to be lesser known, difficult to get approved and covered, challenging to lay hands on and even complicated to take. Patients typically say that they “have to jump through hoops to be able to even get” their medication.

The prescribing of orphan and specialty drugs has come to a crossroads, where access issues are being acutely felt. Earlier this year, *MM&M* commissioned three polls—of patients, physicians and payers—for insight into what each believes are the key issues, and how these might be solved. Here, we give some background, and discuss some takeaways.

“Rare” becomes common

An “orphan drug” is one that has been developed for a rare disease, defined by the FDA as one affecting fewer than 200,000 people. The

Orphan Drug Act of 1983 works to defray some of the disincentives for developing these drugs, and similar legislation has been enacted globally.

“Specialty drug” is a broader term. While an orphan drug could be a specialty drug, the term focuses on delivery rather than target. Specialty medications often have complex usage requirements: they may be injectable, require refrigeration or need frequent dosage adjustments. A specialty drug often treats a chronic condition such as cancer, hemophilia, hepatitis, HIV, multiple sclerosis or rheumatoid arthritis.

The National Institutes of Health has classified 7,000 rare or “orphan” diseases, which, in total, affect nearly one in 10 Americans (25-30 million people). And there are quite a few drugs for them: 442 have been given orphan-drug designation by the FDA. Moreover, about a third of drugs approved in 2013 carried orphan-drug status. Nearly 200 orphan drugs enter development annually, according to stats cited by Thomson Reuters.

It’s hard to build a list definitive list of specialty drugs, because the classification is a managed-care term of art, not a clinical defi-

nition. However, statistics on spending align with this assessment.

In fact, specialty drugs are the fastest-growing segment of healthcare expenses, at an annual rate of 15%-20%, according to pharmacy benefit consultant PSG, and are expected to account for 40% of total drug costs by 2020. US specialty-drug spend is projected to increase 67% by the end of 2015, the PBM Express Scripts estimates, while spending on traditional medications is forecast to decline 4% in that time, thanks largely to generics. With healthcare costs ballooning, it becomes clear why payers see a need to shift their focus.

It's been easy to mentally relegate orphan and specialty drugs as unimportant players in the healthcare space. But small numbers add up, and the monikers make these diseases sound far more infrequent than they are.



62% of patients said they sometimes deal with prior authorization or step edits when obtaining these drugs

Currently, the world's second-most-profitable drug is an orphan/specialty drug: Genentech's Rituxan (rituximab), used for chronic lymphocytic leukemia and non-Hodgkin's lymphoma, as well as the non-orphan disease rheumatoid arthritis.

When statistics come to bear, it becomes clear that while words like "orphan" and "specialty" suggest an insignificant niche, the truth is very different. The titles are becoming ironic: these medications are becoming more prevalent than ever before.

Patients list barriers to access

When asked to describe issues that patients felt were barring access to specialty pharmaceuticals, apart from cost, respondents—24 health activists belonging to the WEGO Health social network (screened to include only those from rare or specialty disease communities)—most commonly cited distribution. In the days of Amazon Prime and Seamless, it can be hard, especially for those in urban areas, to remember that geographic delivery limitations are still common.

Trivio's voice-response patient research platform captured their responses. One patient said, "Sometimes...location can be a barrier. [I]n more rural areas, it may be harder...to get into a bigger city for treatment, or to find someone...properly trained in administering the medication."

Another barrier was that of information: doctors and patients need data about the right drugs and how to get them; but also, insurance-company personnel require a knowledge base relevant to a case.

"There are some guidelines being put together by...specialty physicians," another patient said, "but this information does not always make its way out to local doctors, beyond the centers of expertise."

Apart from high cost and inadequate distribution of both product and information, patient barriers centered on coordination. Respondents expressed a need for a simpler and more streamlined system with less red tape. Sixty-two percent said they occasionally or frequently deal with prior authorization or are asked by insurers to try cheaper alternatives when obtaining these drugs.

Asked to rate the effectiveness of specialty drugmakers in working with MCOs to ensure patients have access to medications, the average rating was a 4.95 (out of 9).

Documentation required for pre-authorization requires a sig-

nificant volume of paperwork be completed by both physician and patient. And communication challenges mean that patients are unclear about what they need to do, who they need to contact, what the status is or who's responsible each step along the way.

"I'm often lost as far as who's supposed to call me back and where I am in the process of whether I'll be able to obtain it and when I'll be able to start it," yet another patient said.

Time—the passage of it and the lack of it—can do much more than simply frustrate. One patient told of their process needing to be repeated entirely, because time ran out. Another told of needing to wait weeks, first for pre-authorization, then for delivery.

WEGO Health's director of market research and insights, Louise Revers, points out the "anxiety and anticipation" which accompanies these patients' frustrations. In addition to administrative challenges, patients are, of course, facing their disease, and the physical and psychological challenges that accompany it.

What the doctors say

Physicians cited many of the same barriers to securing coverage as did patients, and they also express nervousness about the collision of these two opposing forces, access and cost. Although, as one doctor put it, "Cost is everything," some see a way out.

Many are looking to empowered patients to address the issue: Almost two-thirds of doctors, surveyed via the MedLIVE global physician panel from WorldOne Interactive, said they think that patients can and should take a role in improving their access to drugs.

Roughly 50% of the 76 physicians who said they currently prescribe orphan or specialty drugs to their patients mentioned wanting patients to lobby legislators or agitate with payers, and 13% want more patient involvement in the administrative process.

"Because of the nature of the impersonal online format," says WorldOne data analyst and methodologist Kat Lynch, "I saw physicians putting more responsibility on patients getting involved than in



66% of doctors said they think patients can and should take a role in improving their access to these drugs

individual interviews. This might be an area where physicians would like to see patients doing that, but they're not comfortable voicing it, or it's something they don't usually think of off the top of their head. But in this format it did come out as something they want."

Lynch points out in particular clinicians' desire for patients to "advocate with power holders"—legislators and payers (though not with manufacturers).

Payers aim defense against physicians, patients

Payers' bulwark against the influx of expensive requests is to require pre-authorization paperwork and reviews, results show, and these techniques were not looked upon favorably by patients.

One patient summed up the sentiment thusly: "The insurance company having doctors of their own that review the claims and determine whether the patients should have the medication or not—I think that whole process is nonsense. If you can't trust the prescribing doctor, who can you trust?"

However, payers don't seem to be stemming the influx of claims.

Of 200 polled, some 83% of respondents—medical directors and those belonging to P&T committees, as polled by the firm MediMedia Managed Markets (MMMM)—found orphan-drug claims stable, with 17% experiencing an increase and none seeing a decrease.

To try to ensure proper treatment, payers require prior authorization to verify diagnosis. They may also require that inexpensive treatments be tried before more costly ones, a tactic known as “step-edits.”

Half of respondents said they expect to maintain the levels of these current strategies over the next two years, and 17% said they each expect to increase either the use of step-edits, or the cost to patients.

However, Earlene Biggs, VP of market research at MMMM, says those numbers may be conservative. “There’s a lot going on in healthcare right now,” she says. “For example, a new hepatitis C drug costs \$1,000 a day and \$84,000 for a course of treatment—and it’s recommended to be taken in combination. Those are happening more and more. Every time [payers’] bottom line gets hit, they’re going to have to react.”

Another change may be afoot. Half of payers who responded to the survey manage specialty drugs under the pharmacy benefits, and half under medical benefits. Biggs opined, “I suspect that you’ll see [specialty drugs] moving under pharmacy more and more, and the transfer of management tactics from pharmacy to medical.”



67% of payers said they expect to either maintain or increase formulary management of these drugs

This is in part because, as specialty drugs become increasingly common and more refined, administration can be done at home rather than in-office or in an infusion center. The side effect is that, in getting the drugs to patients via pharmacies more often, payers will be able to manage prescribing more strongly.

Solving everyone’s problems

Payers have a real, practical business concern: they must find a way to pay for members’ treatments. Physicians and patients seek the best possible treatment for each individual. These are not actually diametrically opposed goals—or, at least, they don’t have to be. All three want the right patients to get the right medicines. All three agree that the current system is not optimal. The question is, how can the three work together to improve it?

A variety of practical difficulties in developing these complex medications prompted the legislation to ease that burden. The FDA Orphan Drug Act gives entities with this classification an extra two years of brand exclusivity. But data shows that an equal percentage of orphan drugs achieve blockbuster status as non-orphan drugs (29%), suggesting that they are a worthwhile business bet.

As Thomson Reuters put it, “Economic drivers such as tax credits,

Survey methodology

The opinions and quotes in this article came from three surveys conducted on behalf of MM&M in January 2014. Each focused on one of the relevant populations: patients who have taken a specialty or orphan drug, physicians who prescribe them and payers.

The patient survey was conducted by WEGO Health using the Truivio mobile research platform and database of 65,000 health activists. It was fielded January 30. Of 24 total responses, 18 consumer-health influencers had direct experience with specialty pharmaceuticals. Six did not or were not sure, instead speaking on behalf of the experience of their community.

The physician survey data was derived via the MedLIVE global physician panel from WorldOne Interactive. It was fielded online February 3. Of 102 respondents, 76 physicians did currently prescribe orphan or specialty drugs to their patients.

The payer survey was conducted by MediMedia Managed Markets through their proprietary, secure MedicalDirectorsForum.com social network and the community of the monthly *P&T Journal*. The survey was fielded January 29-31 and was cut off at 200 respondents, per company policy.

grants, waived FDA fees, reduced timelines for clinical development and higher probability of regulatory approval, coupled with commercial drivers such as premium pricing, faster uptake, lower marketing costs and longer market exclusivity, further fuel the economic power of orphan drug development.”

This is good news in terms of bringing innovative treatments to market. It also validates the need to improve the process of getting them. Both physicians and patients frequently mentioned the need for some type of liaison to help with paperwork. There is such a professional. It’s not news that “specialty patients need care and handling as much as the drugs they take,” as was noted in 2007 in the journal of the National Home Infusion Association. “Patient navigators” are increasingly answering that need, helping patients surmount barriers to healthcare, which can include education, appointment coordination, paperwork and other practical or emotional support.

Another suggested solution was the improvement of specialty-pharmacy logistics. It may be possible to bring some of the expertise of global online and just-in-time fulfillment processes to bear.

Improving educational content for patients, physicians and payer staff may also help everyone better understand the diseases, their treatments and the procedures to obtain them.

Finally, more online solutions, also suggested by both physicians and payers, could bring transparency—providing status updates; automating parts of the process of getting, filling and refilling a prescription; and directing participants to the appropriate help (such as a patient navigator).

The consequences of inaccessible specialty and orphan medicines have human faces. They can change people’s lives, if the players involved can figure out how best to make it happen. ■



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