Hot Space

Our second annual survey of patients, physicians and payers reveals a significant degree of frustration—on every side—in and around the ever-booming specialty drug segment. Sarah Morgan interprets the results

A year ago *MM&M* sought to outline and investigate the key issues affecting the specialty and orphan-drug spaces. Three surveys were commissioned—of patients, physicians and payers—and the challenges were examined from all three viewpoints. A year later *MM&M* sought out those populations again to update its insights and discover whether the reported issues have improved or intensified.

Today, payers report that the number of orphan-drug claims has nearly doubled and that they expect the increase in claims to continue (based on 38 medical directors and members of P&T committees—see sidebar for survey methodology). Similarly, the average cost per patient of an orphan drug has gone up 24% from 2010 to 2014. While the average cost of a non-orphan drug increased 51% in that same time, the difference is that the average non-orphan drug costs roughly one-seventh as much as an orphan drug.

It's easy to see that, with prices rising (even if more slowly than elsewhere) and claim numbers shooting up, the demand on the market of these unusual drugs will increase any already-felt pain points in the system. But what are these drugs and why are they so important?

The terms *orphan drug* and *specialty drug* connote niche products, and to an extent this is true. Orphan drugs have, by the FDA's definition, been developed to treat diseases that affect fewer than 200,000 people. The designation, dating back 31 years in the US and mirrored similarly around the world, is an official one, meant to encourage drug development for rare conditions.

"Specialty drugs," as a phrase, is less defined because the term is not a legislative or clinical definition; rather it's a managed-care term of art. It refers to products that have complex delivery or

usage. They may need to be refrigerated or injected, as opposed to a standard bottle of pills on a retail pharmacy shelf.

Orphan and specialty drugs are indeed rare classifications, but that truth can camouflage their impact. As noted in *MM&M*'s piece a year ago, "orphan" diseases in total affect nearly one in ten Americans and have over 400 drugs currently approved to treat them.

Specialty drugs are the fastest-growing and the costliest segment of healthcare. From 2012 to 2018, the category is expected to grow 155% (for perspective, it already has more than $50 billion in worldwide sales). Some studies show that, by 2018, specialty drugs will account for half of US healthcare expenses. So while these drugs and the conditions they treat can be complex, chronic and uncommon, the numbers demonstrate that, far from having a negligible impact on the market, orphan and specialty drugs have an increasingly center stage role in conversations about drug treatment and health management.

Payers struggle against the onslaught

Payers, not unexpectedly, continue to implement and ratchet up management strategies to deal with the influx of specialty claims. Nearly 80% of those surveyed require prior authorization to verify their diagnosis, and about a third require step therapy, the implementation of less expensive treatments before a costlier one.

Nearly all (92%) plan to make those strategies more stringent in the next two years. Two-thirds expect to increase the use of step therapy, for instance, and 56% expect to use data analysis to improve the cost-effectiveness of formulary decisions.

But doctors and patients are not impressed. "We shouldn't have to beg," one survey respondent, a physician, noted. And as one patient put it, plaintively, "There's really no reason anybody would want to take these type of medications unless they need them."

In any case, the tactics don't seem to be effective. The impact of orphan drugs on the average per member per month pharmacy gross cost is significant. While the overall average PMPM dropped slightly from 2012 to 2013, as reported in the PBMInstitute 2014-2015 Report, the trend for specialty products was +12.2%.

What, then, might help?

Physicians fatigued by the process

The physicians surveyed were exactly split (50.5% vs. 49.5%) on whether they believed drug companies were doing a good job of working with payers to ensure patient access to orphan and specialty drugs. Those that believed companies were working well with managed care often cited tenacious behavior and mid-range expectations as useful approaches for all parties: "With persistence, companies are responsive to our inquiries"; "[payers are] at least working with drug companies to try and cover some orphan drugs"; "something is better than nothing."

Those that did not believe that companies were doing a good job in this regard, however, expressed their vexation with the process. "From my end, the process is burdensome and tedious," said one. Another agreed: "I've just never had any experience in which the managed-care company appears to be helpful." A third described "far too much hassle for something that was obviously necessary."

However, even the physicians who said they dealt with orphan and specialty drug issues the most often (more than 25 times a month), were evenly split on whether they believed drug companies are doing a good job at working with payers. So despite frustrations, opinions remain split. More than 80%, however, did mention cost as a concern.

SURVEY METHODOLOGY

The opinions and quotes in this article came from three surveys conducted on behalf of *MM&M* in January 2015. Each focused on one relevant population: patients who have taken a specialty or orphan drug, physicians who have prescribed them and payers.

The patient survey was conducted by WEGO Health using the Truvio research platform. It was fielded January 14–16 from a panel of 22 patient-health activists who self-selected as having rare disease experience.

The physician survey data was derived via MedLIVE PULSE, a product offering from SERMO. It was fielded online on January 8 and included 103 respondents. Of that total, 83 physicians have dealt with prior authorization or step therapy for patients with specialty or orphan diseases.

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 7–11 and included 38 respondents.

Patients have an idea

But patients, perhaps surprisingly, as a cohort offered the most concrete suggestions in our surveys for improving the state of the orphan and specialty treatment space. While many did indeed discuss general issues of approval, access and cost, they gave an overall approval rating of 80% to the job that drugmakers do working with payers to ensure access. And nearly a third volunteered a specific issue that they felt was hindering the process: the need to increase the clinical education of those responsible for specialty cases.

Among the most telling responses: "I think the biggest barrier is the lack of a proper understanding of the disease and the need for proper education"; "the most difficult part of working with insurance providers is that they're uninformed about what a medication actually is"; "companies could work with payers to let them know that this medication, if taken proactively, can actually lower costs in the long run and that this medicine is a necessity … versus a luxury"; "when insurance is calling the shots on whether you receive coverage or not, and they know nothing about the drug or the condition, this becomes a travesty"; "health insurance companies are understaffed in terms of clinicians and researchers who understand new drugs and how they make a life-or-death difference in the treatment of many diseases."

Today we live in a healthcare world in which patient engagement and empowerment are common buzzwords. Data have time and time again demonstrated that knowledgeable, involved patients have better outcomes and cost the system less. Developments in technology provide increasing fuel to the engine of patient empowerment.

It appears, however, that patient proactivity may not always be met by availability on the side of payers or collaborative efforts on the side of pharma. Certainly, cost and access will continue to be strikingly important for specialty pharma to address—and therefore important to the healthcare industry overall. Working to increase the ability for productive medical conversations to take place may be an interesting new tack for specialty pharmas to smooth the rough waters their patients experience. ■