MANAGED CARE March 2013

COVER STORY

Tiger in the Fiscal Room: Beware the Increasing Cost And Number of Orphan Drugs

While Congress worries about physicians' salaries and employers bemoan the cost of imaging, it's orphan drugs that might deserve most attention

Ed Silverman

Earlier this year, NPS Pharmaceuticals surprised nearly everyone who was eagerly waiting for the drug maker to start marketing its recently approved Gattex treatment for short-bowel syndrome, a rare disease in which nutrients are not properly absorbed because a large section of the small intestine is missing or has been removed.

In remarks to Wall Street analysts, NPS executives unexpectedly announced that Gattex would cost $295,000 per patient per year, roughly three times what the company had signaled only weeks earlier. And instead of a potential patient population of as many as 15,000, the pool was now estimated to be between 3,000 and 5,000.

The price was increased after NPS compared the results of several prevalence studies with estimated patient headcounts at several large home-infusion companies. That left the drug maker with a much smaller universe of potential patients than forecast previously, although the lower number also placed Gattex in the so-called ultra category, which can command a higher premium.

"Yes, it was very late in the game when we came up with this surprising number, but Gattex is not unique," says François Nader, NPS chief executive officer and president.

"But it's not unusual to have a dichotomy between what those [prevalence] studies give you and the actual addressable population" that was obtained after extrapolating patient data and updating forecasts.

"But we don't have the luxury of running comprehensive and multiple epidemiological studies. This is an art and a science. We talked to payers to estimate the burden of illness based on their data and tried to answer several questions: What does the drug offer to mitigate the condition and meet an unmet medical need? Does it help lower overall health care costs and improve quality of life?"

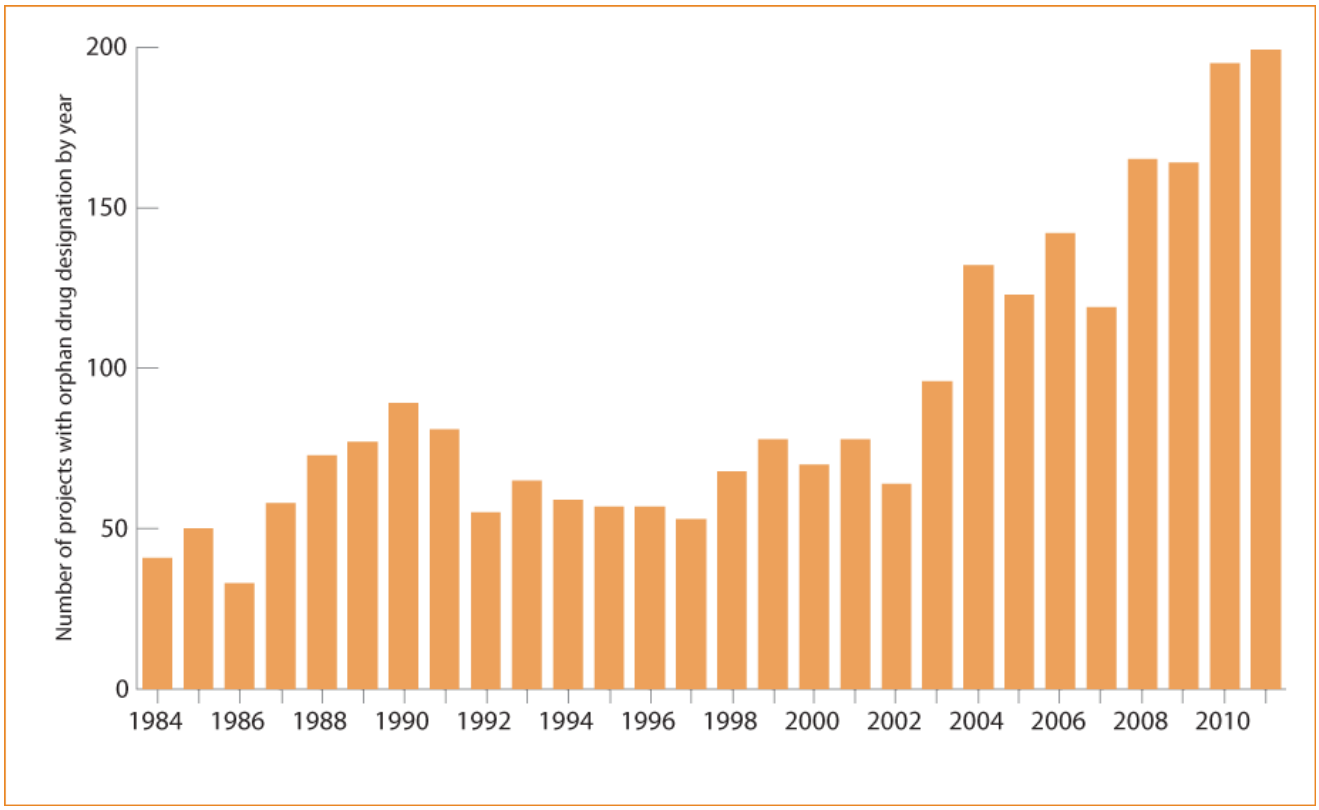
Sweet spot

These questions speak to the so-called value proposition, that hard-to-define sweet spot between cold calculation and actual benefit. And this proposition is increasingly being scrutinized and refined as more orphan drugs win approval from the Food and Drug Administration and are launched at ever-higher prices that are worrying managed care plans and employers.

Last year, the FDA approved 13 new molecular entities as orphan drugs — one third of the 39 drugs approved that year. This was the largest number of drugs to win this designation in the past seven years. Already this year, two newly approved treatments were granted orphan status by the FDA.

FIGURE 1 Orphan disease designation by the FDA, 1984-2010

As of October 2011, a total of 1,795 projects with FDA orphan designations were in development

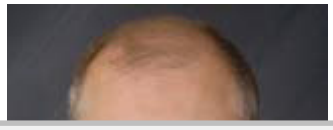


Source: PhRMA

Significantly, five of the most recently approved orphan drugs will cost at least $150,000 per patient per year and three of these medications cost $300,000 or more annually. And the trend is raising hard questions about the ability of the health care system to absorb the costs and, therefore, about how third-party payers are going to make coverage decisions.

This is a familiar concern, of course, but one that is expected to play out at an accelerating rate in coming years as big drug makers and small biotechnology companies recognize that pursuing orphan indications can fatten their bottom lines. In fact, PhRMA — the Pharmaceutical Research & Manufacturers of America — recently counted 1,795 orphan designations in the development pipeline.

Right now, pharmaceuticals account for about 15 percent of health care spending, but the costs of orphan drugs and other biologics are rising 20 percent to 25 percent a year, says Ed Pezalla, national medical director for pharmaceutical policy at Aetna. And he adds that state insurance commissioners are not often willing to allow managed care companies to increase premiums by a commensurate amount.



For insurers, "what we need are numbers to understand how many patients are really helped," says Ed Pezalla, national medical director for pharmaceutical policy at Aetna.

"We do cover a number of orphan drugs, but we anticipate the pipeline increasing. And the costs are relatively high compared to other medications, so this is getting our attention," he says. "The prices are growing faster than premiums can grow and there's going to be a point where the costs will be an extremely serious concern, and this is a concern that the pharmaceutical industry will have to consider. They can't hide by saying orphan drugs are only a small portion of our budget when we know that these are the fastest-growing part."

This means added strain on budgets as it becomes harder to accurately predict actual claims to be paid each year, which will cause increases in premiums to the extent allowable by state agencies and higher hurdles for patients to qualify for coverage.

More than 1 of 4 orphan drugs had sales of over $1 billion. There are very many orphan diseases with no drugs — yet.

But any payer that considers denying coverage will be in a bind because to do so is, simply put, to appear heartless. In the scheme of things, orphan drugs are used by relatively few people, so to refuse coverage — even when costs are rising — runs the risk of creating an enormous public relations problem, since alternative treatments are generally lacking.

"The public, state agencies, and federal government are all in alignment for assuring full coverage and without any pre-existing condition rules," says Randy Vogenberg, a principal at Bentelligence, a benefit consulting firm, and a member of Managed Care's editorial advisory board.

Until recently, though, the issue was largely overlooked, because there were fewer orphan drugs. Now, the recent spate of expensive medications is quickly appearing on more radar screens, raising fresh debate about the extent to which the trend can continue and whether it will engulf the health care system.

New molecular entity approvals for rare diseases Calendar years 2006–2012			
	NMEs and new biologics	Rare (% of total approvals)	
2012*	31	7	(23%)

2011	30	11	(37%)
2010	21	7	(33%)
2009	26	9	(35%)
2008	24	8	(33%)
2007	18	6	(33%)
2006	22	6	(29%)
*Data as of Nov. 30, 2012 Several NME applications for rare diseases have PDUFA goal dates before Dec 31, 2012.			

“The question is whether the pricing will threaten the whole system. I’m not convinced it does, but the problem with saying we’ll charge $300,000 is that the marketplace still requires you to conduct the right studies first,” says Steven Grossman, a former congressional aide who worked on the Orphan Drug Act before its passage in 1983 and now heads the HPS Group, a policy and regulatory consulting organization.

“If you assume the pricing decision is made in the boardroom without being subject to scrutiny, then we’ll see pushback. So if there’s a drug that’s going to cost $300,000 or $500,000, we damned well better have a justifiable price. If the numbers work correctly, then it should be paid for. But that doesn’t mean that any CEO who gets FDA approval can or should arbitrarily charge that price,” says Grossman.

Says Vogenberg: “Ultimately, this kind of pricing is going to force more and more disclosure about what goes into pricing and methodology. At some point, there’ll be a review similar to what England, France, Germany, and Australia have done. They will look at the same questions: Are these prices justifiable and should there be coverage? There has to be more balance between the clinical significance and economic impact when determining reimbursement for new biologic or specialty products.”

Certainly, there is a lot of money to be made selling an orphan drug. An estimated 25 million people in the United States alone live with some sort of orphan disease, and spending on orphan drugs makes up 6 percent of total pharmaceutical sales, assuming a total market value of $880 billion, according to Thomson Reuters.

“I would not be surprised to see more premium prices for orphan indications,” says Alan Carr, an analyst at Needham & Co.

DOCKET ALARM

Explore Litigation Insights

Docket Alarm provides insights to develop a more informed litigation strategy and the peace of mind of knowing you're on top of things.

Real-Time Litigation Alerts

Keep your litigation team up-to-date with **real-time alerts** and advanced team management tools built for the enterprise, all while greatly reducing PACER spend.

Our comprehensive service means we can handle Federal, State, and Administrative courts across the country.

Advanced Docket Research

With over 230 million records, Docket Alarm's cloud-native docket research platform finds what other services can't. Coverage includes Federal, State, plus PTAB, TTAB, ITC and NLRB decisions, all in one place.

Identify arguments that have been successful in the past with full text, pinpoint searching. Link to case law cited within any court document via Fastcase.

Analytics At Your Fingertips

Learn what happened the last time a particular judge, opposing counsel or company faced cases similar to yours.

Advanced out-of-the-box PTAB and TTAB analytics are always at your fingertips.

API

Docket Alarm offers a powerful API (application programming interface) to developers that want to integrate case filings into their apps.

LAW FIRMS
Build custom dashboards for your attorneys and clients with live data direct from the court.

Automate many repetitive legal tasks like conflict checks, document management, and marketing.

FINANCIAL INSTITUTIONS
Litigation and bankruptcy checks for companies and debtors.

E-DISCOVERY AND LEGAL VENDORS
Sync your system to PACER to automate legal marketing.