Manipulating the Orphan Drug Act

What an Orphan Designation for an Old Drug Means for Payors
Old Drug, New Price

Early in February, the U.S. Food and Drug Administration (FDA) approved Emflaza, from Marathon Pharmaceuticals, to treat Duchenne muscular dystrophy (DMD) among patients age five and older. The post-approval price tag for Emflaza is $89,000. In addition, Marathon received a seven-year exclusivity as part of the “orphan” designation it received to treat this deadly genetic muscle deterioration disorder that affects about 15,000 Americans, mostly boys.

While this is the first time the drug has been approved for sale in the U.S., it is a decades-old steroid, previously known as deflazacort. Deflazacort has been available – for roughly $1,000 a year – in the European Union and Canada, and has been imported into the U.S. What’s more, Marathon did not develop deflazacort. The company acquired the rights to the drug’s original clinical trial data and – along with some additional analysis – used it to gain FDA approval. Marathon received a priority review, shortening the regulatory process by four months.

The company’s actions drew scrutiny and at least one lawmaker announced an investigation into potential abuse of the Orphan Drug Act by Marathon. Following the outcry, Marathon halted the launch of Emflaza, although there is no word on when the company may now launch it or whether the price tag will change.

Unintended Use of a Well-Intentioned Provision

Emflaza’s high price tag isn’t unusual for orphan drugs. As many as 30 million Americans have a “rare disease,” a condition such as DMD or amyotrophic lateral sclerosis (ALS), that affects 200,000 people or less. The 1983 Orphan Drug Act provides tax incentives and patent protection, and eliminates FDA application fees for companies developing therapies to treat rare diseases. The law was passed after advocacy by a coalition – which has since become the National Organization for Rare Disorders (NORD) – representing patients who had virtually no approved pharmaceutical treatment.

In the decade before the law passed, just 10 drugs for rare diseases came to market. Since 1983, more than 450 drugs for rare diseases have been approved. Last year, the FDA received a record number of applications for orphan drugs. Orphan drugs offer treatment options for patients who didn’t have any before. However, in some instances like Emflaza, provisions of the law can be used to set higher prices for drugs leading to increased costs for patients and payors.

FDA Approval of Orphan Drugs from 1973-2016

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Manufacturing a Monopoly

Because orphan drugs typically treat very small populations, one provision of the Orphan Drug Act gives manufacturers who receive the first orphan designation exclusive market rights. An orphan designation for the newly named but decades-old steroid, Emflaza, means Marathon also received market exclusivity for seven years allowing the manufacturer to create a “monopoly” and set any price without competition.

The strategy has been used before. Taro Pharmaceutical Industries purchased dichlorphenamide, once marketed under the brand name Daranide, to treat glaucoma. Taro applied for orphan approval for the drug to treat periodic paralysis. The company was bought by Sun Pharmaceuticals Industries. The drug received orphan status. Its average wholesale price (AWP) went from $50 a bottle in 2002 to $13,650 a bottle in 2015, at re-launch.

A recent analysis by Kaiser Health News showed that more than 70 orphan drugs currently on the market were first approved for mass market use by the FDA and later received orphan status earning manufacturers hundreds of millions of dollars in government incentives and market exclusivity.

The trend in orphan drug development is likely to continue. In 2015, 21 of the 45 new drugs approved by the FDA were for orphan diseases. And in 2016, the FDA received a record number of requests for orphan drug approvals. This is a significant and positive development for under-served patient populations with these rare diseases. However, better regulatory oversight and legislative action to eliminate loopholes that allow the system to be abused are needed.

What the Orphan Drug Designation Means to Manufacturers

- Exclusivity
- Priority review
- Tax incentives
- FDA fee waiver
- Protocol assistance
- Research grants

For instance, rituximab, a drug originally approved to treat follicular B-cell non-Hodgkin’s lymphoma, a disease that affects about 14,000 patients a year, is now also used to treat rheumatoid arthritis, which affects 1.3 million Americans. While this increases options for patients with rheumatoid arthritis, it has significant cost considerations for payors. A recent report by America’s Health Insurance Plans, the main trade group for private payors, stated that of a sample of 45 drugs studied, more than half of the usage of orphan drugs was for non-orphan conditions.

The average per-patient cost of an orphan drug is about 13.8 times higher than that for a non-orphan drug.
How to Manage Spend and Access

A comprehensive formulary strategy is the foundation to effective cost management to help rein in the trend impact of these types of medications. This includes strategic and targeted tools such as formulary exclusions and new-to-market evaluations, which can help blunt the impact of a new drug or supplemental indication and protect payors from high trend impact while maintaining appropriate patient access.

We are also developing other approaches such as indication-based pricing, which ensures that reimbursement for a drug is determined based on factors such as its efficacy in treating a disease and competition within the therapy class. Other clinically appropriate utilization management tools may include prior authorization with required documentation of tests and screening to confirm the appropriate diagnosis. Each drug is unique, demanding a differentiated approach based on the market landscape as well as the product’s clinical properties and potential.

The primary goal in integrating these cost management strategies is to help ensure appropriate prescribing and delivering more sustainable pharmacy costs while preserving patient access to the most-effective treatment.

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* $1,000 price for generic available in Canada and EU and imported into the U.S. prior to FDA approval; $89,000 price for the newly-approved branded drug Emflaza in the U.S.

This document contains references to brand-name prescription drugs that are trademarks or registered trademarks of pharmaceutical manufacturers not affiliated with CVS Health.