Sky-high drug prices for rare diseases show why Orphan Drug Act needs reform

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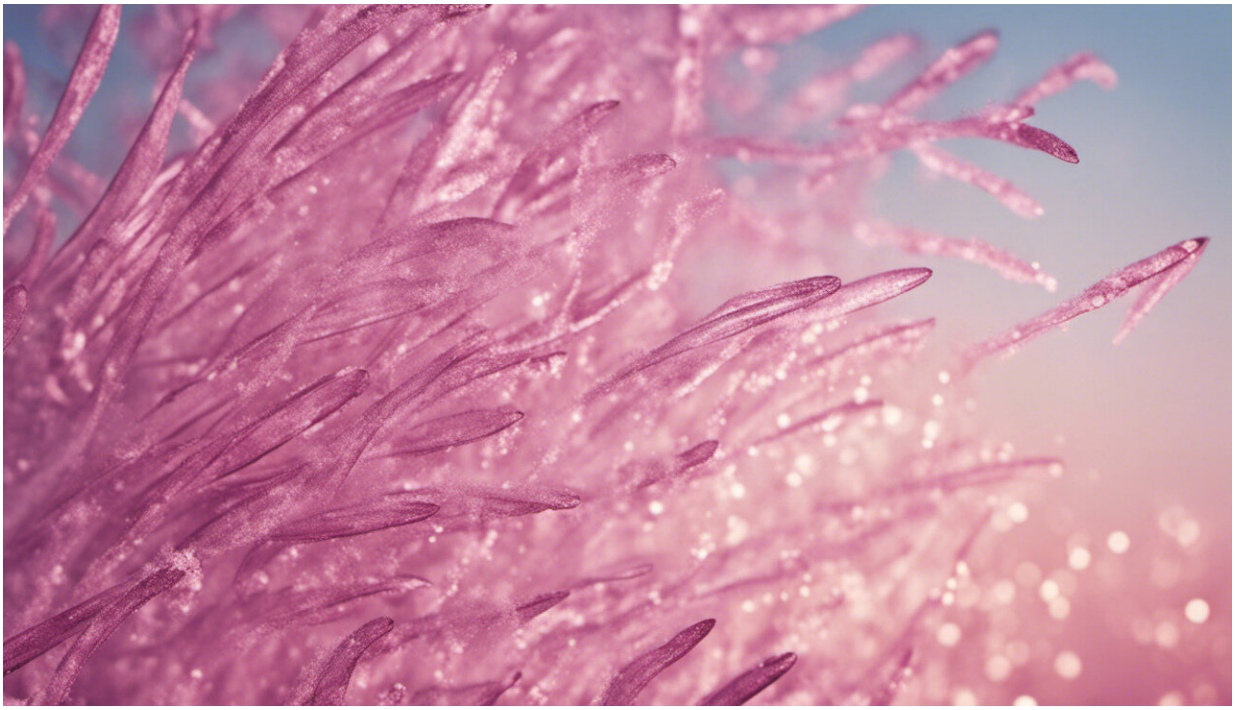

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When Marathon Pharmaceuticals announced in February it would market a drug for treating Duchenne muscular dystrophy for US$89,000 a year, the negative reaction was so intense that the company immediately suspended the rollout. (On Thursday, March 16, Marathon announced it was selling the drug to PTC Therapuetics for US$140

million in cash and stock, plus a one-time payment of $50 million if sales reach a certain milestone). Even the industry's trade group cried foul.

A good part of the outrage stemmed from the fact that FDA approval of the drug simultaneously closed the door on much cheaper imports. Deflazacort, a steroid which Marathon wanted to market in the U.S. under the brand name Emflaza, has been sold abroad for decades. The cost to U.S. families who imported it was only $1,000 to $1,600 a year.

The outrage was intensified by the nature of the patients. Duchenne muscular dystrophy predominantly affects boys, depriving them of the ability to walk by ages seven to 15 and killing them in their late teens and 20's. It strikes about one in 7,500 males between the ages of five and 24.

The Marathon case is the latest example of what has gone wrong with the Orphan Drug Act (ODA), which was passed 34 years ago to promote development of drugs aimed at diseases that afflict small groups, typically under 200,000 people. Marathon sought and won approval for Emflaza under ODA provisions.

As health economists who study drug pricing, we see an opportunity for change.

Even great ideas sometimes go awry

The ODA contains a powerful inducement for pharmaceutical firms: New treatments for rare diseases earn seven years of market exclusivity, including protection from imports. That means the companies can price without fear of competition and sell to dependent populations. It also means patients with rare diseases have some hope that their conditions are not neglected.

However, the nature of drug development has changed. The number of requests for orphan designation has quadrupled since 2000. The result has been a boom in drug sales and profits. The average price of an orphan drug exceeds $100,000 a year. Orphan drugs now make up one in three drugs approved by the FDA. Sales of orphan drugs rose 12 percent in 2016 to $114 billion, compared to a rise of 2.4 percent for all other branded drugs to $578 billion.

Some of the drugs developed under the ODA are lifesaving, and many are cost-effective. But over the years many other drugs, such as Emflaza, have won ODA status even if they are not new or represent a scientific breakthrough.

For example, a cheap off-patent drug approved by the FDA for one condition, but widely prescribed as an "off-label" treatment for an orphan disease, can be transformed into a big moneymaker. By putting the drug through clinical trials to document its safety and effectiveness, a pharmaceutical company can gain FDA approval for use on the disease. The accompanying seven years of monopoly status can mean large price hikes for a drug that was already in wide use.

Insurers are usually stuck with the bill, but – as should be clear to everyone by now – these costs get passed to consumers via higher premiums.

Even though Emflaza's price tag is relatively small compared to many orphan drugs (Biogen introduced a $750,000 drug to treat spinal muscular atrophy in December), it seemed to hit a nerve. In February, Sen. Chuck Grassley (R-Iowa), the chairman of the Senate Judiciary Committee, announced that he will convene a formal inquiry into potential abuses of the Orphan Drug Act.

What to do?

The first reaction is to call for government price setting. But this is not just a public payer issue, and the government doesn't set prices in the private insurance plans.

In addition, in some ways the ODA is doing what it was designed to do: Offer temporary monopolies as an incentive for drug discovery. The FDA could factor pricing into its decision-making, but this is a slippery slope for an agency that has never explicitly factored prices into review.

Something could be done, though.

On the access side, patients with these diseases are costly to society, but their care is valued. This means patients with rare diseases should have guaranteed access to high-risk insurance pools – an issue of some importance in the current Obamacare replacement debate – and these high-risk pools cannot skimp on orphan coverage.

On the pricing side, private insurers haven't scrutinized these orphan products enough. Traditionally, it wasn't worth the insurers' time. While prices are high, the total costs are small when spread over the entire population, and the patient communities are vocal. (In the Duchenne example, only about 800 patients receive the drug currently.)

But the world is changing. Advances in personalized medicine and genomics also demand change. We now face the prospect that more common treatments – like cancer vaccines – could be custom designed for each patient, and that these will be reflected in even more orphan applications.

Insurers need to hold the line with manufacturers, and make them accountable to offer value-based prices for all products, including orphan drugs. The FDA can help by insisting manufacturers collect rigorous and complete post-market surveillance data about how patients

fare after taking orphans – across all payers, private and public.

We have the tools to deal with this problem. We just need to start using them.

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