



U.S. Drug Costs and Patient Access Issues in the 21st Century

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High drug costs and concerns regarding patient access to expensive medications are not new. In fact, for decades conversations have centered on the limitations that drug costs have on patients' ability to access such treatments. However, in the past several years, the focus has shifted to specialty pharmaceuticals, which generally are significantly more expensive than other prescription drugs, and the impact that this category of drugs is having on health care spending and patient access, particularly for life-saving medications. This article explores the drug category of specialty pharmaceuticals and addresses several of the cost and access issues associated with these products.

What Are Specialty Pharmaceuticals?

The term "specialty pharmaceutical" or "specialty drug" has become commonplace in the health care industry. But, what exactly are specialty drugs? There is no clear definition of the term at this point. Many payers and pharmacy benefit managers (PBMs)¹ have adopted their own definitions of specialty pharmaceuticals in their individual service agreements with one another. Below are two examples of specialty pharmaceutical definitions found in some agreements between payers and PBMs:

1. "Specialty Drug" means a prescription drug that is typically a high-cost biotech, injectable, infused, oral, or inhaled



prescription drug, and/or a prescription drug that requires special storage, handling, and/or requires close monitoring of the patient's drug therapy to ensure appropriate use and clinical outcome. Specialty drugs are typically dispensed at a specialty pharmacy, but may also be available from other participating pharmacies.

2. "Specialty Drug" shall mean a high-cost, complex pharmaceutical that has unique clinical, administration, distribution, or handling requirements and is not commonly available through traditional retail or mail pharmacies.

In other words, drugs that are costly, often in the tens to hundreds of thousands of dollars annually, and tend to treat rare and/or complex diseases like hepatitis C, certain cancers, HIV, rheumatoid arthritis, and hemophilia often fall into the category of "specialty drugs." Interestingly, as shown in these definitions, the drug categories often have little connection, and may be lumped together simply based on price points. In addition to price, these also are drug types where: (1) there is no therapeutic equivalent; (2) a treatment exists, but it is less effective and/or has severe side effects; or (3) if there is a similar treatment, it is high cost as well.

Cost Issues Associated with Specialty Pharmaceuticals

One common theme among the various definitions of specialty drugs is that they are expensive. The hepatitis C drug Sovaldi is often considered a "specialty drug" and costs roughly \$84,000 in the United States for a 12-week treatment.² Harvoni, a similar hepatitis C treatment, can cost up to \$95,000 for a 12-week treatment.³

But, why are these drugs so expensive? Typically, the drugs themselves are considered breakthrough therapies and often are the only effective treatment for a specific disease. Although Sovaldi and Harvoni are high cost, they have revolutionized hepatitis C treatment; until recently there was no effective cure for hepatitis C in the majority of patients with the disease.

Additionally, specialty drugs often enjoy patents and/or Food and Drug Administration (FDA) marketing exclusivity.⁴ This leaves no option for a generic manufacturer to produce a lower-cost version of the drug. FDA marketing exclusivity can run from three to seven years depending on the approval type. For example, Sovaldi enjoys FDA marketing exclusivity until 2018 and Harvoni until 2019. In addition, patents on both products run as late as 2030.⁵

Because of the patent and FDA marketing exclusivity, specialty drugs often face little competition. Although Harvoni and Sovaldi are both treatments for hepatitis C, they were brought to market by the same company, leaving little incentive for the manufacturer to price competitively since, at the time of their introduction, one company held the majority of the market. Interestingly, once a competitor enters the market—for example late last year a new hepatitis C treatment, Viekira Pak, was approved—prices often drop. Although not significantly reduced, Viekira Pak is priced at just above \$83,000 for a 12-week treatment.⁶

In addition to limited competition in the market, drug companies have cited the cost of research and development to justify pricing drugs high. Companies that spend millions of dollars on research and development need a way to recoup those costs, often by building them into a drug's price. While these new specialty medications have caused sticker shock, they also may lower overall drug spending in the long run by minimizing and, in some cases, eliminating certain costly diseases. That being said, the immediate impact of specialty medications on drug spending has been significant.

Financial Impact

A recent study published by the IMS Institute for Healthcare Informatics indicates that in 2014 drug spending reached its highest level since 2001, up to \$373.9 billion, which is a 13.1% increase.⁷ Spending was "driven by innovative new medicines, lower patent expiry and higher list prices," and not, as many may assume, by demand for health care services due to the increase in covered lives as a result of the Affordable Care Act (ACA).⁸ In other words, specialty pharmaceuticals contributed

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to most of the drug spending growth in 2014.⁹ Spending on specialty drugs alone grew 26.5% in 2014, making spending on specialty drugs one-third of overall drug spending. Of that one-third, new hepatitis C treatments contributed \$11.3 billion in growth. This spending growth was the result of more than 161,000 patients starting hepatitis C treatment in 2014, nearly ten times the number of patients who did so in 2013.¹⁰ Not only have more people entered treatment, but more breakthrough treatments are on the market than in the past.

The Proliferation of Specialty Drugs

In recent years, there has been a strong push for medications to treat common diseases that currently have no cure or limited treatment options, as well as certain rare diseases. In 2012, there were 350 pharmaceutical products approved to treat 7,000 rare disease that, at the time, affected 30 million Americans.¹¹ In 2012, the President's Council of Advisors on Science and Technology identified "opportunities to accelerate the approval of a broader range of truly innovative drugs for patients who need them," and recommended that FDA fully utilize the accelerated approval pathway and create new pathways to speed approval.¹²

In 2014, FDA issued procedural guidance calling attention to its expedited programs for serious conditions.¹³ These pathways are available to "facilitate and expedite development and review of new drugs to address unmet need in the treatment of a serious or life-threatening condition" and include the fast track designation, breakthrough therapy designation, accelerated approval, and priority review designation.¹⁴ The fast track designation allows for frequent interactions with FDA and rolling review. Accelerated approval speeds the development and approval of eligible drugs based on endpoints that are measurable earlier in the development process, although the process often requires post-market trials.¹⁵ The priority review designation "means [that] FDA's goal is to take action on the marketing application within 6 months of receipt"¹⁶ as opposed to the typical longer time period, and the breakthrough therapy designation allows rolling and priority review, as well as frequent interactions with FDA.¹⁷

In 2014, FDA approved 41 new molecular entities and new therapeutic products, a number of which enjoyed accelerated pathways to approval.¹⁸ The number of drug approvals with breakthrough designations increased nearly fivefold from 2013 to 2014. In 2013, there were three breakthrough therapy designation approvals, including Sovaldi.¹⁹ In 2014, 14 products with a breakthrough therapy designation were approved, including Harvoni and Viekira Pak.²⁰ To date in 2015, there have been eight breakthrough therapy designation approvals.²¹ Note that since 2013, a large number of drugs have been given the breakthrough therapy designation, but have yet to be approved, or in some cases, the designation has been rescinded.²² In furtherance of FDA's focus on accelerated approvals, the agency released July 7, 2015 a report outlining the need for targeted drug development for a number of diseases that are still awaiting treatments and/or cures.²³

Also in July 2015, the U.S. House of Representatives passed the 21st Century Cures Act (Act) with bipartisan support. The Act aims to address the development and approval of new and innovative drug products and medical devices.²⁴ The Act includes provisions to compel FDA to address "precision medicine," also known as personalized medicine, for orphan-drug and expedited-approval pathways.²⁵ In addition, the Act encourages FDA to approve drugs with a breakthrough therapy designation in an even more expedited manner, provided that the current requirements for approval are met, i.e., safety and effectiveness.²⁶ The Act also would allow for extended market exclusivity for drugs with an orphan drug designation.²⁷

Although the dialogue surrounding the legislation has mostly focused on its potential benefits, the Act has not gone without criticism. Around the same time the House passed the Act, the White House issued a Statement of Administrative Policy indicating "concerns" with certain provisions, including the additional funding of the National Institutes of Health (NIH) and FDA without addressing sequestration generally and how extending "drug exclusivity beyond current law will affect drug costs."²⁸ As of this writing, the Senate was working on its version of the legislation, which is expected to be unveiled this fall.

With more specialty drugs entering the market and the continued push for innovation and accelerated approval pathways, treatments now exist to cure diseases that have never been available before. The reality, however, is that not all patients will have an opportunity to receive these new treatments.²⁹

Access Issues Go Far Beyond Cost

The costs of specialty drugs are a barrier to many patients, including patients without insurance coverage for certain drugs, patients who are responsible for the full cost of their drugs, and insured patients who face high out-of-pocket costs (even with out-of-pocket maximums set by the ACA). Less obvious, however, are the barriers to access that payers and PBMs have imposed as they attempt to contain costs associ-

ated with the recent proliferation of specialty pharmaceuticals in the market. Although blockbuster drugs are nothing new to payers and PBMs, the recent uptick in specialty drugs that came to market in 2014 may have been unexpected.

Many payers and PBMs were not prepared for the costs of the new treatments that became available in 2014, including the nearly 161,000 patients who entered treatment for hepatitis C last year. To manage the unforeseen costs associated with specialty pharmaceuticals a number of PBMs have taken steps including limiting network pharmacy activities, formulary exclusions, and requiring exclusive use of certain pharmacies.

For example, in April 2015, a large PBM informed its network pharmacies that it was updating its pharmacy manual to incorporate a list of prohibited activities by pharmacies that dispense compounded drugs, which were categorized as specialty drugs.³⁰ The list limits pharmacies' ability to act as an outsourcing facility, dispense compounded medications, act as a mail-order pharmacy without permission from the PBM, and submit claims for a compounded drug product when there is a similar, manufactured drug product on the market.³¹

In December 2014, another prominent PBM notified its network pharmacies of formulary changes for hepatitis C drugs.³² Specifically, the notice stated that starting January 1, 2015, certain medications within the hepatitis C therapy class would no longer be covered on most clients' specific national formulary for patients new to therapy. The excluded drugs for hepatitis C included Harvoni, Incivek, Olysio, and Sovaldi.³³ The available option, or preferred alternative, was limited to Viekira Pak.³⁴ An explanation for the exclusions was not given. In theory, these types of decisions could be driven by discounts or rebates made available to the PBM by the drug manufacturer.³⁵

Formulary exclusion is a prime example of the struggle between pharmaceutical companies and organizations like PBMs and payers that cover the costs of drugs. PBMs have an incentive to use exclusions because they can assist in treatment decisions by encouraging the use of a "lower-cost" yet effective alternative. They also send a clear message to other drug companies that pricing should be competitive. However, formulary exclusions also limit patients' options, as the PBM's members do not have access, or have limited access, to excluded products. In some cases, formulary exclusions also require patients to obtain a new prescription from their physicians naming the preferred alternative drug.

Another means to reduce costs is allowing access to specialty medications only through a certain pharmacy, which is often owned by the PBM managing the member's drug benefits. The 2014 PBM notice, mentioned above, indicated that most sponsors' prescription drug benefits would require members to fill Viekira Pak, the preferred alternative to Sovaldi, at a specific pharmacy with no initial fills allowed at retail. The specific pharmacy is a wholly owned subsidiary of the PBM and acts as its specialty pharmacy. A PBM's use of a

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single pharmacy means that instead of going to their preferred retail pharmacy, patients must fill the specialty drug through a pharmacy that may not have a convenient location or is a mail-order only pharmacy.

Access Programs and Advocacy

Programs and organizations have been created to promote access to medications, including specialty drugs. The 340B Drug Discount Program, which has operated for over two decades, is one example of a congressionally mandated program to increase access. The program requires drug manufacturers to provide significant discounts on outpatient drugs, including specialty medications, to certain eligible entities, including certain hospitals, health centers, and specialized clinics. This program is not without controversy, however, including concerns related to program operations, transparency, and enforcement. This and other programs likely will continue to be vehicles to promote access to specialty drugs in the event that costs do not decline.

In addition to federal access programs, certain advocacy and activist groups also are taking legal and business actions to reduce costs and increase access for specialty drugs. For example, activist groups are starting to put pressure on drug companies to release patents to certain drugs so generic drug manufacturers can produce a less costly version for sale in the United States.³⁶ In late 2014, one such group filed a class action lawsuit against a drug manufacturer in federal court relating to the high price of one of its specialty drugs.³⁷ The complaint accuses the manufacture of "price gouging," and claims unjust enrichment, discrimination in violation of the ACA's non-

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discrimination provision (Section 1557(a)), and breach of specific duties.³⁸ The complaint seeks a determination that the manufacturer was unjustly enriched.³⁹

States also have taken an interest in this issue. A number of state legislatures are considering cost transparency bills,⁴⁰ some of which require disclosure to the state of drug costs over a certain dollar amount, as well as the disclosure of costs associated with drug manufacturing, marketing, and advertising that go beyond what is already required reporting for pharmaceutical companies. One bill would allow insurance companies to take action if a company did not file required cost reporting, such as refusing to pay for a drug.⁴¹

Conclusion

How do we reconcile the business of the health care industry and the needs of patients? In other words, new and innovative treatments now exist that not every patient who needs them has the opportunity to access.

The push to expand the availability of specialty drugs by rewarding innovators with market exclusivity that allows them to price products high is in tension with a health care payment system that focuses on reducing costs, often resulting in limited patient access to these new medications. While the issue is not new, the recent proliferation of “life-saving” specialty drugs in the last several years has brought this tension into sharper focus without producing a clear answer. As the push for more breakthrough therapies continues, the need for action in this area will only accelerate. More transparency, in the form of expanded disclosure requirements, as well as the growing scrutiny of patient advocates, may help temper any unnecessary increases in the costs of specialty drugs. Going forward, the focus on innovation and its associated rewards must be balanced enough to spur further research and development to bring new drugs to market while still allowing for increased competition and lower costs. The 21st Century Cures Act and the potential for new FDA exclusivity are steps in the right direction to encourage the discovery of new cures, but we also need to ensure that these benefits are balanced with ensuring access to patients that need these treatments the most. 

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Endnotes

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