

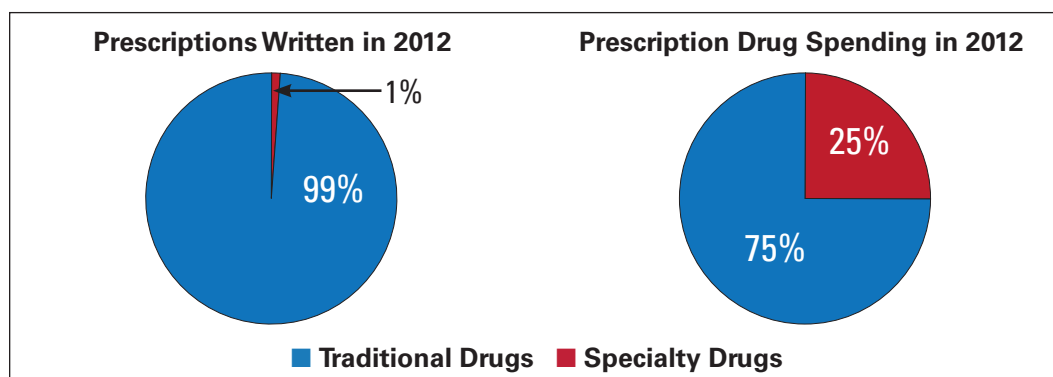
Specialty Drugs—Issues and Challenges

Advancing Effective Strategies for Addressing Soaring Drug Costs While Assuring Access to Effective Treatments and Promoting Continued Medical Innovation

Introduction

Spending on specialty drugs represents an increasing share of U.S. prescription drug spending and is growing at a rapid and unsustainable rate. Addressing these cost-trends is critically important to assuring a sustainable health care system and achieving affordability for businesses and consumers. Last year alone, U.S. spending on prescription drugs totaled \$263.3 billion—25% of which was spent on specialty drugs.¹

Figure 1: U.S. Spending on Prescription Drugs, 2012



Source: Specialty Therapy Class Forecast, 2012. Express Scripts Drug Trend Report.

Specialty drugs—which are generally understood to be drugs that are structurally complex and often require special handling or delivery mechanisms—are priced much higher than traditional drugs. While these drugs have been ground breaking in the treatment of cancer, rheumatoid arthritis, multiple sclerosis, and other chronic conditions, the cost of treating a patient with specialty drugs can exceed tens of thousands of dollars a year. The treatment regimen for some of the most expensive specialty drugs can cost \$750,000 per year.²

While these drugs offer tremendous promise when medically necessary, their high costs and extended use has put a strain on our health care system. Health plans, employers, and other stakeholders are searching for new ways to restrain cost growth while simultaneously maintaining access to safe and effective drugs for patients.

This issue brief explores recent trends in the specialty drug market, highlights some of the innovative strategies health plans are adopting to control costs, and recommends additional policy solutions to further promote high-value, high-quality care.

Prescription Drug Cost Trends

National spending on all pharmaceuticals has moderated in recent years – following similar trends in overall health care spending. The Centers for Medicare & Medicaid Services (CMS) estimates that prescription drug spending was approximately 9.4 percent (\$263.3 billion) of all health care spending in 2012, growing only 0.4 percent from 2011.³ While CMS attributes this decline to greater adoption of generic drugs and several “blockbuster” drugs losing patent exclusivity, it projects sustained increases in drug spending from 2015-2022 of six percent or more annually as both drug prices and utilization increase.⁴

Specialty drugs account for a disproportionate share of overall drug spending because of their extremely high cost. The average annual retail cost for a specialty medication to treat a chronic condition was almost \$29,000 in 2009⁵, with some drugs costing as much as \$750,000 (Figure 2).

Figure 2: Selected Conditions, Top Medical Conditions and Specialty Drugs, 2010

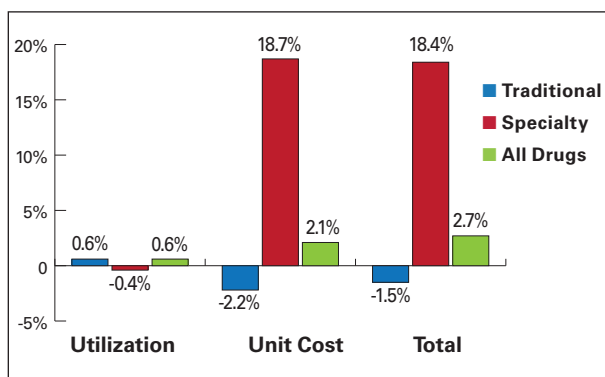
Health Condition	Average Cost Per Treated Member Per Year
Inflammation Conditions (Embrel, Humira, Remicade)	\$14,455
Multiple Sclerosis (Copaxone, Avonex, Rebif, Tysabri, Avastin, Provenge)	\$24,118
Cancer (Revlimid, Gleevec, Tarceva, Avastin, Provenge)	\$11,089
Pulmonary Hypertension (Tracleer, Revatio, Letairis)	\$32,570
Respiratory Conditions (Xolair, Prolastin)	\$18,550

Source: Limited Options to Manage Specialty Drug Spending. Center for Studying Health System Change Research Brief. April 2012.

Because of the comparatively high cost of these drugs, the commercial trend for pharmaceutical spending in 2012 was driven almost entirely by increases in the unit cost of specialty drugs (Figure 3). Were it not for the nearly 19 percent

annual increase in the unit cost of specialty drugs, the overall pharmaceutical market would have experienced negative growth in 2012. Unlike their traditional counterparts, spending on specialty drugs has shown no signs of abating; similar double digit increases are forecast for 2013-2015.

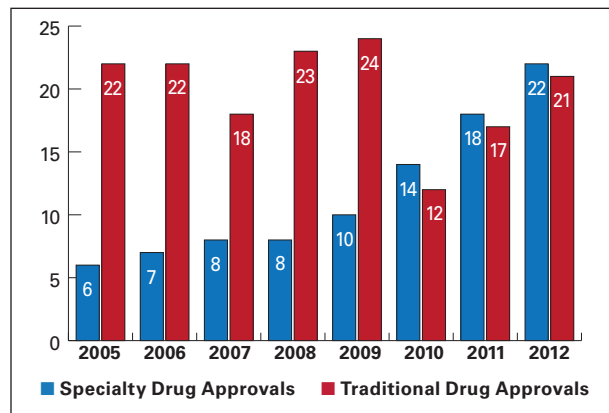
Figure 3: Components of Commercial Trend, 2012 (January-December 2012 compared to same period in 2011)



Source: Express Scripts Drug Trend Report. Available at: <http://www.drugtrendreport.com/commercial/total-trend>

The increasing presence of specialty drugs in the pharmaceutical market further explains their position as the driver of drug spending. In 2010, specialty drug approvals by the Food and Drug Administration (FDA) exceeded traditional drug approvals for the first time (Figure 4), a trend that has continued each year since. And in 2013, 60 percent of the drugs approved by the FDA are expected to be specialty drugs.⁶ A recent report by health care accrediting agency URAC noted that the marked increase of chronic illnesses in Americans (such as cancer, obesity, diabetes) coupled with the increasing complexity of the pharmaceutical industry has positioned the specialty drug market for continued growth.⁷

Figure 4: FDA Traditional and Specialty Drug Approvals, 2005–2012



Source: Medical Cost Trend: Behind the Numbers 2014, PricewaterhouseCoopers Health Research Institute, June 2013, Figure 6.

The Prescription Drug Market

Unsustainable growth of specialty drug spending is due to many complex factors but can be explained, in part, by the legal and regulatory treatment of these therapies in the prescription drug market. Under current law, brand name biologic drugs are given a 12 year exclusivity period upon approval from the FDA – in effect a government-approved monopoly. This period of exclusivity is longer than exclusivity protections extended to traditional drugs—which last 5 years. Although these exclusivity periods give pharmaceutical manufacturers the incentive to take on the risk of developing groundbreaking drugs, they also precipitate a number of negative policy consequences.

Granting exclusivity to specialty drugs removes the economic benefits of price competition, resulting in higher prices relative to what they would be in a perfectly competitive market. This trend can be seen in Medicare spending for Part B drugs, which are most often specialty drugs since they are covered through the medical, rather than pharmacy benefit. The Government Accountability Office released a report examining trends in Part B spending and found that in 2010, not only did 10 drugs account for 44 percent of all Part B spending, but none of these drugs had a generic version also approved by

the FDA.⁸ The lack of adequate substitutes for these drugs constrains payers' (health plans, the government, or employers) efforts to implement effective cost-containment policies. Health plans have developed expertise in using value-based purchasing or cost-sharing designs that steer individuals toward high-quality/high-value treatments and care. But when generic or therapeutic alternatives do not exist, the options available for encouraging high-value and cost-effective care are limited.

As a result of this confluence of factors—increased FDA approvals for specialty drugs, near-monopoly pricing, and the growing need for effective treatments—experts project that “spending on specialty drugs is expected to skyrocket over the next decade and beyond” and “intensifying the cost and access trade-offs that payers and purchasers already face.”⁹

Health Plan Efforts to Manage Specialty Drug Spending While Assuring Access to High Quality Care

Health plans have developed a number of strategies in response to sustained cost increases that ensure access to critically important drugs while also holding down costs.

Integration of Pharmacy and Medical Benefits

Because of their complex nature and their delivery mechanism, specialty drugs are often covered through the medical, rather than the pharmacy benefit. This distinction has interfered with the incorporation of traditional cost-sharing mechanisms by obscuring prescribing and utilization patterns across the two benefits—causing a mismatch between utilization management techniques used for specialty and traditional drugs.

Health plans have begun developing innovative benefit designs that both recognize the unique nature of specialty drugs but allow for appropriate tiers and/or coinsurance rates. More tightly

integrating the pharmacy and medical benefits also allows plans to better track the usage of specialty drugs across its enrollees and identify additional areas for benefit modernization.

Policies to Maximize Treatment Adherence

Coverage of a specialty drug is ultimately fruitless if patients have poor adherence to a dosing regimen, or if they discontinue use after filling the prescription—leading to poor outcomes and higher costs. Health plans are helping to ensure that patients are taking their medications by engaging them about the disease and the therapeutic process. Health plans are also helping patients understand how to take their medications correctly by coordinating with providers and making sure that patients understand the guidelines for using the medication and any potential side effects.

Growing Role of Specialty Pharmacies

Many health plans now contract with specialty pharmacies that supply enrollees with the specialty drugs they need and coordinate the often complex delivery and treatment processes associated with these drugs. These pharmacies are better suited than traditional pharmacies to monitor and track the use of specialty drugs and have the necessary training and expertise to handle their distribution. Specialty pharmacies also employ dedicated teams of health care specialists that can help enrollees understand how to manage their medication and can help ensure that these drugs are administered at the most appropriate site of care.

Utilization and Pharmacy Management

By covering specialty drugs for their intended uses and monitoring the effectiveness and any side effects that occur during the therapy session, health plans can help to ensure that individuals are receiving safe and cost-effective care.

Policy Options to Promote High-Quality, Cost-Effective Drug Coverage

Although health plan efforts at lowering the growth of spending on specialty drugs have shown signs of success, substantial reforms are needed at the state and federal levels to better control specialty drug spending and promote patient safety in the aggregate and over the long term. Policymakers can leverage the experience of health plans and other payers to enact policies that strike the appropriate balance between cost control and promoting individual's access to effective treatments. These policy options include:

- ▶ **Encouraging alternative payments and incentive structures—such as coverage with evidence development—for new drugs and technologies.** Such payment strategies can assure access to new drugs while generating additional evidence on the value to patients of these new medications. As part of a broader value-based purchasing strategy, these alternative arrangements—such as outcomes-based contracting strategies—provide enhanced financial incentives for manufacturers of new drugs and medical technologies that are contingent on agreed-upon standards for quality care, performance, and health outcomes. Greater use and availability of comparative effectiveness data is a key element in the future growth of these innovative payment arrangements.
- ▶ **Shortening the exclusivity period for generic biologics—to promote greater price competition and earlier access to lower-cost specialty drugs or bio-similars.** Congress should shorten the exclusivity period for biologics to allow for more competition from follow-on or generic biologics—similar to the patent protections afforded to traditional, small molecule prescription drugs. By shortening the exclusivity period, this proposal would facilitate the entry of lower-cost, generic biologic drugs—reducing costs throughout the health care system. While specialty

and other breakthrough drugs can offer lifesaving treatments to patients with serious medical conditions, there are opportunities to help reduce costs and improve efficiency in delivering high-quality, cost-effective treatments to patients. By shortening the exclusivity period, policymakers can ensure greater price competition in the specialty drug area and help alleviate cost pressures for payers and consumers.

The U.S. Federal Trade Commission (FTC) has concluded that the current 12-year exclusivity period is “unnecessary to promote innovation by pioneer biologic drug manufacturers” and may harm consumers by “directing scarce research and development dollars toward developing low-risk clinical and safety data for drug products with proven mechanisms of action rather than toward new medical inventions to address unmet medical needs.”¹⁰ President Obama—as part of the Administration’s most recent budget proposal to Congress—supports a shorter exclusivity period as a way to strike an appropriate balance between “promoting affordable access to medication while at the same time encouraging innovation to develop needed therapies.”¹¹ The Administration estimates that reducing the exclusivity period will save \$3.3 billion over ten years.¹²

► **Remove barriers at the state level that restrict the use of biosimilars.** While the Affordable Care Act authorized the FDA to develop an abbreviated licensure pathway for biosimilar drugs, it has yet to issue final standards that will determine when a biosimilar drug is truly interchangeable with an already approved biologic. Ahead of these standards, some states have already adopted legislation that may restrict the availability of biosimilars before they even reach the market. These proposals will limit patient access to drugs that are not clinically different, yet cost substantially more than their brand-name counterparts.

► **Expanding agencies’ authority to consider research on treatment effectiveness.**

Consumers and providers have a right to know which treatments and drug regimens work and which are less effective. In the absence of a national process for measuring the cost-effectiveness of procedures and drugs, many providers are attempting to control costs by basing coverage decisions on the relative costs of similar treatments. Sloane-Kettering Cancer Center announced in 2012 that it would not provide patients with Zaltrap—a drug used to treat advanced colorectal cancer – because it cost more than double the price of Avastin while offering no clinical advantages.¹³ Health care systems in Europe have also begun pursuing policies that reimburse only for drugs that have been clinically proven to work.

To expand this evidence base in America, Congress should provide new authorizing language for the Patient-Centered Outcomes Research Institute (PCORI) that explicitly allows it to consider research on cost-effectiveness as a valid component of patient outcomes research. PCORI and the Agency for Healthcare Research and Quality (AHRQ), in their funding of research on the effectiveness of treatments and technologies and their dissemination of the results of that research, should prioritize the establishment of a multi-stakeholder, deliberative process that can use such research to provide trustworthy recommendations on high-value and low-value care options to providers, payers, and patients.

► **Adopting policies that encourage value-based benefit designs across public and private payers.** Many health care consumers lack the information and financial incentives necessary to make more informed health care choices, despite facing substantial and growing out-of-pocket costs. Evidence about clinical effectiveness, safety, quality, and cost can also be incorporated into benefit designs to help health care consumers make better choices. The emerging area of value-based insurance

design can help promote better outcomes and quality of care—by lowering or eliminating cost-sharing for proven, high-quality and effective pharmaceutical treatments—while discouraging low-value, high-cost care through the use of appropriately tailored financial incentives. Value-based purchasing and benefit designs are being pioneered in the private commercial sector and some states have also instituted these innovations in public programs—such as Oregon and Washington. For example, Washington State's evidence-based prescription drug program has yielded savings of \$20-\$30 million a year—representing a 5% reduction in drug spending across state-administered programs including state employees, the basic health program, and Medicaid.¹⁴ By incorporating rigorous reviews of evidence on the effectiveness of medicines and related therapies and providing incentives for physicians to prescribe preferred drugs, the state has promoted access to evidence-based treatments for patients while reducing unnecessary costs. By building on best practices in the private sector and the states, these efforts should be expanded more broadly to assure access to high-quality and cost-effective treatments—based on the best available medical evidence and clinical guidelines.

► **Adopting a “least costly alternative” (LCA) standard for certain drugs covered under Medicare Part B.** CMS should be provided the flexibility to set a single payment rate for groups of clinically similar drugs based on the lowest cost item. Similar to reference pricing strategies used successfully in many countries, these policies encourage cost-effective drug coverage and savings to consumers by setting a price ceiling for drugs within a category of drugs considered clinically equivalent and interchangeable.¹⁵ Consumers and patients selecting a higher-cost drug would be responsible for any cost-differential between the drug selected and the lowest cost, clinically

equivalent drug within a class. These policies work best in drug categories where there is sufficient competition and alternative drugs and treatments available to patients. In addition to reducing costs, this policy would also reduce incentives for physicians to prescribe more costly drugs when comparable lower cost alternatives are available.

An analysis by the Office of the Inspector General at the Department of Health and Human Services found that implementing a LCA policy with respect to certain prostate cancer drugs administered under Medicare Part B would have saved \$33.3 million over the course of a year. Additionally, the OIG found that when LCA policies for Part B drugs were removed in 2010, utilization patterns shifted “dramatically” toward more expensive drugs with the same clinical purpose.¹⁶

► **Prohibiting patent settlements between drug companies.** Congress should bar certain anti-competitive settlements that prevent generics from entering the market in a timely manner—thereby expanding the availability of low-cost, but equally effective, generic drugs. Both the FTC and the Obama Administration has cited these arrangements as anti-competitive and the Administration has supported legislative efforts to remove these barriers to competition as a way to promote lower-cost generic drugs to patients and consumers. The Congressional Budget Office estimates that prohibiting these settlements would save \$4.4 billion from 2014-2023.¹⁷

Conclusion

The skyrocketing cost of specialty drugs remains a critical concern for policymakers and payers—given the current trajectory of pricing trends in this fast-growing and emerging area. These recommendations represent actionable steps that could be implemented to ensure the efficient and effective use of these high-cost treatments while—at the same time—promoting continued medical advances and innovations that offer promise and benefit patients and consumers.

- 1 Express Scripts: Drug Trend Report, Specialty Therapy Class Forecast. Available at: <http://www.drugtrendreport.com/commercial/specialty-forecast-by-therapy-class>
- 2 Brian Schilling. “Purchasing High Performance: Specialty Drugs Poised to Skyrocket but Many Employers Have Yet To Take Notice.” *The Commonwealth Fund*; April 2012
- 3 National Health Spending in 2012: Rat of Health Spending Growth Remained Low For the Fourth Consecutive Year. *Health Affairs*. January 2014. Available at: <http://content.healthaffairs.org/content/33/1/167.full.pdf>
- 4 National health Expenditure Projections, 2012-2022. The Department of Health and Human Services, Centers for Medicare & Medicaid Services. Available at: <http://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/Downloads/Proj2012.pdf>
- 5 Rx Price Watch Report: Trends In Retail Prices of Specialty Prescription Drugs Widely Used by Medicare Beneficiaries, 2005 to 2009. AARP Public Policy Institute. Available at:
- 6 Express Scripts: Drug Trend Report, Specialty Therapy Class Forecast.
- 7 URAC Specialty Pharmacy White Paper. 2012.
- 8 Medicare: Information on Highest-Expenditure Part B Drugs. GAO Testimony before the Subcommittee on Health, Committee on Energy and Commerce, House of Representatives. June 28, 2013. Available at: <http://www.gao.gov/assets/660/655608.pdf>
- 9 Ha T. Tu and Divya R. Samuel. “Limited Options to Manage Specialty Drug Spending.” *Center for Studying Health System Change*; April 2012
- 10 Federal Trade Commission Report. “Emerging Health Care Issues: Follow-On Biologic Drug Competition.” June 2009.
- 11 Obama Administration’s FY 2014 Budget Proposal.
- 12 Fiscal Year 2014 Budget, Page 198. Available at: http://www.aarp.org/content/dam/aarp/research/public_policy_institute/health/2011/rx-pricewatch-01-2012.pdf <http://www.whitehouse.gov/sites/default/files/omb/budget/fy2014/assets/budget.pdf>
- 13 Peter Bach, Leonard Saltz, and Robert Wittes. “In Cancer Care, Cost Matters.” October 14, 2012. Available at: http://www.nytimes.com/2012/10/15/opinion/a-hospital-says-no-to-an-11000-a-month-cancer-drug.html?_r=1&
- 14 Washington State: An Integrated Approach to Evidence-Based Drug Purchasing. *The Commonwealth Fund* <http://www.commonwealthfund.org/Newsletters/States-in-Action/2006/Mar/March-2006/Profiles--In-Depth-Looks-at-Initiatives-that-Are-Making-a-Difference/Washington-State--An-Integrated-Approach-to-Evidence-Based-Drug-Purchasing.aspx>
- 15 Ha T. Tu and Divya R. Samuel. “Limited Options to Manage Specialty Drug Spending.” *Center for Studying Health System Change*; April 2012
- 16 Least Costly Alternative Policies: Impact on Prostate Cancer Drugs Covered Under Medicare Part B. Department of Health and Human Services, Office of the Inspector General. November 2012. Available at: <http://oig.hhs.gov/oei/reports/oei-12-12-00210.pdf>
- 17 Estimates Effects on Direct Spending and Revenues for Health Care Programs of Proposals in the President’s 2014 Budget. Available at: http://www.cbo.gov/sites/default/files/cbofiles/attachments/44247_APB_HealthCarePrograms.pdf



America's Health Insurance Plans
601 Pennsylvania Ave., NW
South Building Suite 500
Washington, DC 20004

202.778.3200 · www.ahip.org

For additional information or questions about this Issue Brief, contact our Federal Affairs Department at 202.778.3200. Copyright ©2014 by America's Health Insurance Plans. All rights reserved.