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Specialty Drugs: Background and Policy Concerns

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Summary

Specialty drugs are one of the fastest-growing areas of health care spending. There is no one set definition of specialty drugs, although insurers and other health care payers often characterize them as prescription products requiring extra handling or administration that are used to treat complex diseases including hepatitis C, multiple sclerosis, and cancer. High cost can trigger a specialty drug designation. Biologics, or drugs derived from living cells, are often but not always deemed to be specialty drugs.

Over the past several years, spending for specialty drugs has been growing at a faster rate than spending for other pharmaceuticals. For example, in 2014, U.S. prescription drug spending rose by 13% from the previous year—the fastest pace since 2001—led by a 26.5% increase in spending for specialty drugs (with much of that spending for drugs to treat hepatitis C), according to industry and government data. Specialty medications now account for about one-third of total U.S. prescription drug spending, and some analysts predict they could make up as much as one-half of total drug spending by 2018.

Insurers have tried to control spending for specialty drugs, in part, by increasing cost sharing for beneficiaries of their health care plans and taking other steps to limit access under their policies. Consumer advocates say that these efforts have undercut some of the recent gains in prescription drug insurance coverage. During the past decade, Congress has expanded consumer access to prescription drugs by enacting the Medicare Part D prescription drug program, requiring certain health plans to provide prescription drug benefits as part of the Patient Protection and Affordable Care Act (ACA; P.L. 111-148, as amended), and expanding the state-federal Medicaid program. Some lawmakers now are focused on ensuring that private and public health care payers do not structure insurance benefits in such a way that certain enrollees cannot afford to fill prescriptions for high-cost medications. During the 113th Congress, for example, lawmakers introduced legislation to cap out-of-pocket spending by insured consumers for specialty drugs. In recent years, states such as New York and Delaware have enacted laws that limit consumer cost sharing for prescription drugs. Dozens of states also have passed laws that bar insurers from imposing higher out-of-pocket charges for oral specialty cancer drugs than for traditional treatments. Others have debated bills to require insurers to detail drug development costs.

Although some of the legislative proposals can reduce consumer out-of-pocket costs, they do not address the overall price of specialty drugs. Congress historically has attempted to improve prescription drug affordability by providing incentives to increase supply and market competition. Lawmakers fund basic drug research through the National Institutes of Health and provide nonrefundable tax credits for qualified research spending. Federal laws including the Orphan Drug Act of 1982 (P.L. 97-414), the Drug Price Competition and Patent Term Restoration Act of 1984 (Hatch-Waxman; P.L. 98-417), and the ACA provide financial incentives for both new, breakthrough drugs and lower-cost substitutes. Those incentives may provide some financial relief in the longer term, but in the short run federal programs and private payers face high up-front prices and spending for new specialty therapies, such as recently introduced treatments for hepatitis C and cancer.

This report provides background on specialty drugs. To put specialty drug development, distribution, and spending in context, the report provides information about broader U.S. prescription drug pricing, insurance, and regulatory trends.

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Introduction

Specialty drugs are one of the fastest-growing areas of health care spending.[1] Although there is no commonly accepted definition of specialty drugs, insurers and other health care payers generally characterize them as expensive prescription products requiring extra handling or administration (such as injection or infusion) that are used to treat complex diseases including multiple sclerosis, cancer, and hepatitis C. Biologics—complex drugs derived from living organisms[2]—are often but not always specialty drugs, as are so-called orphan drugs, which are targeted at rare diseases or disorders.[3]

U.S. spending for specialty drugs increased by 26.5% in 2014 as new drugs for treating hepatitis C came to the market, according to a broad analysis of pharmaceutical market data.[4] Other, more targeted studies also show rapid growth for specialty products.[5] According to one of the largest U.S. pharmacy benefit managers (PBMs),[6] specialty drugs account for less than 1% of U.S. prescriptions but about one-third of prescription drug spending. [7] Some health care industry

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[1] IMS Institute for Healthcare Informatics, *Medicine Use and Shifting Costs of Healthcare: A Review of the Use of Medicines in the U.S. in 2013*, April 2014, p. 40; at http://www.imshealth.com/portal/site/imshealth/menuitem.762a961826aad98f53c753c71ad8c22a/?vgnextoid=2684d47626745410VgnVCM10000076192ca2RCRD IMS Institute, *Medicines Use and Spending Shifts: A Review of the Use of Medicines in the U.S. in 2014*, April 2015, p. 8, at http://www.imshealth.com/portal/site/imshealth/menuitem.762a961826aad98f53c753c71ad8c22a/?vgnextoid=3f140a4331e8c410VgnVCM1000000e2e2ca2RCRD&vgnextchannel=736de5fda6370410VgnVCM10000076192ca2RCRD&vgnextfmt=default; and UnitedHealthcare Center for Health Reform & Modernization, *The Growth of Specialty Pharmacy: Current Trends and Future Opportunities*, April 2014, p.1, at http://www.unitedhealthgroup.com/Modernization/KeyIssues/SpecialtyPharmacy.aspx. U.S. health care spending grew by a projected 5.5% in 2014, while prescription drug spending rose 12.6%, driven by specialty drugs and increased prescription drug use. Centers for Medicare & Medicaid Services, N*ational Health Expenditure Projections 2014-2024*, p. 2, at http://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/Downloads/proj2014.pdf.

[2] CRS Report R41483, *Follow-On Biologics: The Law and Intellectual Property Issues*, by John R. Thomas. Also see "Biologics" below. By comparison, traditional drugs are chemically manufactured (small-molecule) substances that often come in tablet or capsule form.

[3] See "Orphan Drugs" below.

[4] IMS Institute, *Medicines Use and Spending Shifts: A Review of the Use of Medicines in the U.S. in 2014*, April 2015, p. 8. The IMS data are based on actual claims and spending by pharmacies, clinics, hospitals, and other health care providers. The data cover 100% of the retail and non-retail channels for national drug sales at actual transaction prices and are matched with other analytic tools used to track patient drug activity over time. IMS said prescription drug spending increased by 13% in 2014, the highest annual increase since 2001. The increase was driven by increased spending for specialty drugs and a slower pace of patent expirations for existing drugs, which can promote use of less expensive generic drugs.

[5] Pharmacy Benefits Manager (PBM) Catamaran, which manages prescription drug spending for 35 million enrollees in U.S. health plans offered by businesses and other health providers, recorded a 20% increase in specialty drug spending in 2014, compared with a 14.3% increase in 2013, driven by increased costs for hepatitis C drugs. Catamaran, *2015 Informed Trends: Moments of Opportunity*, April 2014, p. 16, at http://catamaranrx.com/Insights/Trend-Report/. The data exclude some clients such as those in cash card, workers' compensation, state insurance exchanges, Medicaid, and Medicare Part D. PBM Express Scripts said that specialty drug costs among its commercially insured members rose by about 17% annually on average from 2010 to 2013. In 2014, traditional drug spending for commercially insured members rose by 6.4% and spending for specialty drugs rose by 30.9%, for an overall increase of 13.1%. Express Scripts, *The 2014 Drug Trend Report*, March 2015, p. 11, at http://lab.express-scripts.com/drug-trend-report. Trend and other measures are calculated separately for those members with commercial insurance coverage, and for Medicaid and for Medicare beneficiaries. The analysis does not include drugs billed through the medical benefit (treatment offered in a health care setting such as a physician's office) rather than the pharmacy benefit of an insurance plan.

[6] See text box entitled "What is a Pharmacy Benefit Manager?"

[7] For example, see Express Scripts, "U.S. Rx Spending Increased 13.1% in 2014," March 10, 2015, at

analysts and PBMs predict that specialty drugs could account for half of all annual prescription drug spending before the end of the decade.[8] To date, the rapid increases in specialty spending have been driven mainly by price inflation, although utilization is starting to play a larger role as manufacturers bring a wider array of specialty drugs to market, including products with broad applications, such as the treatments for hepatitis C.[9]

What Is a Pharmacy Benefit Manager?

Pharmacy benefit managers (PBMs) serve as intermediaries between drug manufacturers and health care payers, such as self-insured businesses; insurance companies, including insurers that participate in Medicaid and Medicare; and union-run health plans. PBMs handle prescription billing; negotiate drug prices with drug companies; and create retail pharmacy networks for insurers, including contracting with mail-order pharmacies and negotiating reimbursement rates with them. PBMs also design insurance formularies, which are lists of drugs covered by an insurance plan.

PBMs oversee prescription drug benefits for more than 210 million Americans. The largest PBMs include Express Scripts, with 28% market share; CVS Caremark, with 27% market share; UnitedHealth Group, with 10% market share; and Catamaran, with 7 % market share.[10] Other large PBMs include Prime Therapeutics, MedImpact, and Cigna.[11]

Consolidation in the PBM industry is ongoing. Drug retailers have merged with PBMs to provide integrated health services. CVS Health is a retail pharmacy chain and a PBM. The Rite Aid drug store chain in 2015 purchased the PBM EnvisionRx.[12] Some insurers operate their own PBMs, such as a group of Blue Cross and Blue Shield plans that owns Prime Therapeutics, and UnitedHealthcare, which owns OptumRx. (Catamaran and OptumRx combined in 2015.)[13]

The growing use of specialty drugs poses complex issues for private insurers and government health programs such as Medicare, Medicaid, and the Veterans Health Administration.[14] Specialty drugs can provide marked improvement or a cure for individuals with serious diseases, thereby

http://lab.express-scripts.com/insights/industry-updates/us-rx-spending-increased-13-percent-in-2014. Express Scripts said specialty drugs made up 31.8% of drug spending in 2014.

[8] The findings are based on data from both prescription drug pharmacy claims and medical claims. Artemetrx, *An Evaluation of Specialty Drug Pricing Under the Pharmacy and Medical Benefit*, March 2014, p. 3, at http://www.artemetrx.com/wp-content/uploads/2014/08/artemetrx-evaluation-of-specialty-drug-pricing.pdf; and Prime Therapeutics, "Specialty Drugs are Forecasted to be 50% of All Drug Expenditures in 2018," April 4, 2013, at http://cdn2.content.compendiumblog.com/uploads/user/e7c690e8-6ff9-102a-ac6d-e4aebca50425/accf0d87-0d14-4aa7-bbf3-193b90c8d68c/File/1f8476cb4a60eb472893ddf381c9c3ec/prediction_that_by_2018__more_than_50_percent_of_all_drug_expenditures_will_be_specialty.pdf. The Prime Therapeutics data encompass drug spending through both pharmacy claims and medical claims for 6.8 million commercially insured beneficiaries.

[9] For example, the number of patients starting treatment with specialty hepatitis C medications rose from 17,000 in 2013 to 161,000 in 2014, according to the IMS Institute, *Medicines Use and Spending Shifts: A Review of the Use of Medicines in the U.S. in 2014*, April 2015, p. 10.

[10] Leah Goddard, *Pharmacy Benefit Management in the US*, IBISWorld Industry Report OD4620, July 2015.

[11] For more background on PBMs, see U.S. Department of Labor, "2014 ERISA Council PBM Compensation and Fee Disclosure," at http://www.dol.gov/ebsa/publications/2014ACreport1.html#4.

[12] *Business Wire*, "Rite Aid Completes Acquisition of Leading Independent Pharmacy Benefit Manager (PBM) EnvisionRx," June 24, 2015, at http://www.businesswire.com/news/home/20150624005906/en/Rite-Aid-Completes-Acquisition-Leading-Independent-Pharmacy#.VbuGqvmZOew.

[13] Optum, "OptumRx, Catamaran Complete Combination," July 23, 2015, at https://www.optum.com/news-events/news/optumrx-catamaran-complete-combination.html.

[14] Medicare is an entitlement program that provides health insurance to individuals aged 65 and older, and it has been expanded over the years to include certain disabled individuals under the age of 65. Medicaid is a state-federal, means-tested entitlement program that finances the delivery of primary and acute medical services as well as long-term services and supports. The Veterans Health Administration (VHA), within the Department of Veterans Affairs (VA), operates the nation's largest integrated health care delivery system to provide care for veteran patients.

reducing the need for hospitalizations and other health services. The potential long-term benefits of specialty drugs may not always offset their higher up-front costs, however. In addition, because the U.S. health care system is decentralized and consumers may change insurance providers, one insurer may bear the cost of the drugs while another insurer may reap the benefit of reduced health care spending for an enrollee treated with specialty pharmaceuticals.[15]

Insurers and employers, and the PBMs with which they contract, control drug costs in part by negotiating discounts and rebates with manufacturers. Because there are no ready substitutes for many newly introduced specialty drugs, health payers may have less ability to negotiate significant price reductions. To contain spending, many health care payers also control enrollees' access to specialty drugs under their plans. Insurers may require enrollees to obtain prior authorization for specialty prescriptions (meaning the insurer must review and approve the prescription before paying for it), impose higher cost sharing for the drugs (charge higher out-of-pocket amounts to fill a prescription), or cover the drugs for only the sickest patients. The net result is uneven access to the products for consumers in private insurance plans and some government programs, in terms of both availability and cost.[16]

Congress, which plays a major role in the prescription drug market, has attempted to address broad issues regarding prescription drug price and availability by expanding insurance coverage and providing incentives for pharmaceutical manufacturers to increase the supply of drugs. In the past decade, Congress has provided subsidized drug coverage to tens of millions of consumers by implementing the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA; P.L. 108-173), which created the Medicare Part D prescription drug program[17] and the Patient Protection and Affordable Care Act (ACA; P.L. 111-148, as amended), which requires insurers to provide basic prescription drug benefits in qualified-individual and small-group health plans sold on exchanges. In addition, as part of the ACA, Congress expanded Medicaid,[18] which also provides prescription drug benefits. Reflecting the increasing government role, the federal share of U.S. prescription drug spending rose to a projected 41% in 2014 from 25% in 2005—the year before Medicare Part D took full effect. [19]

Lawmakers also have provided patent protection and other financial incentives for pharmaceutical manufacturers to develop drugs through the Orphan Drug Act of 1982 (P.L. 97-414); the Drug Price Competition and Patent Term Restoration Act of 1984 (Hatch-Waxman; P.L.

[15] See "Costs and Benefits of Specialty Drugs."

[16] National Health Law Program, "NHeLP and The AIDS Institute File HIV/AIDS Discrimination Complaint Against Florida Health Insurers," press release, May 29, 2014, at http://www.healthlaw.org/news/press-releases/224-nhelp-and-the-aids-institute-file-hivaids-discrimination-complaint-against-florida-health-insurers. Caroline Pearson, Avalere Health, "Exchange Plans Increase Costs of Specialty Drugs for Patients in 2015," press release, December 2, 2014, at http://avalere.com/news/exchange-plans-increase-costs-of-specialty-drugs-for-patients-in-2015.

[17] The Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA; P.L. 108-173). Medicare Part D is a voluntary program under which private insurers bid to offer prescription drug benefits to Medicare enrollees. Although the MMA was passed in 2003, Part D did not take full effect until 2006.

[18] CRS Report R43778, *Medicaid Prescription Drug Pricing and Policy*, by Cliff Binder.

[19] Centers for Medicare & Medicaid Services (CMS), *National Health Expenditure Accounts, Historical Tables*, at http://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/NationalHealthAccountsHistorical.html.

98-417);[20] and the ACA.[21] Lawmakers fund basic research through the National Institutes of Health[22] and provide nonrefundable tax credits to the industry for qualified research spending.[23]

Common Insurance Terms Used in This Report

Co-payment: A fixed dollar amount that an enrollee in a health care plan pays for a product or service covered by the plan. For example, an insurer may charge a $20 co-payment for a physician visit or a $5 co-payment for a prescription drug.

Coinsurance: The percentage share that an enrollee in a health insurance plan pays for a product or service covered by the plan. An insurer may charge 10% coinsurance for a $100 prescription drug, meaning the consumer's out-of-pocket cost is $10.

Deductible: The amount an enrollee is required to pay for health care services or products before his or her insurance plan begins to provide coverage. An enrollee in an insurance plan with a $500 deductible would be responsible for paying for the first $500 in health care services. In some insurance plans, the deductible does not apply to certain services, such as preventive care. Insurance plans vary regarding whether beneficiaries must meet a deductible for prescription drug coverage.

Formulary: A list of prescription drugs covered by an insurance plan. In an effort to control costs, insurers are imposing what are known as *tight formularies* that include a more limited number of drugs. Insurers also are using *tiered formularies*, under which patients are charged lower co-payments or coinsurance for less expensive generic drugs and certain brand-name drugs[24] that are designated by the plan as preferred drugs because they are lower cost or deemed by an insurer to be safer or more effective. Under these tiered formularies, patients are charged higher co-payments or coinsurance for more expensive drugs or drugs that the plan deems to be less effective.

Out-of-Pocket Costs: The total amount an insured consumer pays each year for covered health care services that are not reimbursed by an insurance plan. Out-of-pocket costs can include deductibles, co-payments, and coinsurance.

Out-of-Pocket Maximum: The maximum amount an enrollee must pay before his or her health insurance plan covers 100% of health benefits. Certain costs, such as premiums, generally are not counted toward an out-of-pocket cap.

Pharmacy Network: A group of retail, mail-order, and specialty pharmacies that contract with health insurers to dispense covered drugs at a set price. Network pharmacies also may provide other services under contract, such as monitoring patient adherence to drugs. Some states require insurance companies to contract with any willing pharmacy that agrees to meet their financial terms. In other states, insurers may contract with a smaller network of preferred pharmacies. Insurers say they have more ability to negotiate price concessions in preferred networks.

Premium: The amount an enrollee pays for health insurance coverage. Many plans charge monthly premiums, but premiums also can be assessed on a quarterly or annual basis.

Sources: Information from Congressional Research Service (CRS) reports, Healthcare.gov, and other sources.

Recently, there has been an increased focus on specialty drugs. During the 114th Congress, lawmakers introduced bills to cap insured consumers' out-of-pocket spending for high-priced drugs.[25] Some state governments have enacted laws to limit consumer out-of-pocket spending for

[20] CRS Report R41114, *The Hatch-Waxman Act: Over a Quarter Century Later*, by Wendy H. Schacht and John R. Thomas.

[21] CRS Report R41483, *Follow-On Biologics: The Law and Intellectual Property Issues*, by John R. Thomas.

[22] CRS Report R41705, *The National Institutes of Health (NIH): Background and Congressional Issues*, by Judith A. Johnson; and CRS Report CRS Report RL32324, *Federal R&D, Drug Discovery, and Pricing: Insights from the NIH-University-Industry Relationship*, by Wendy H. Schacht.

[23] CRS Report RL31181, *Research Tax Credit: Current Law and Policy Issues for the 114th Congress*, by Gary Guenther.

[24] The U.S. Food and Drug Administration (FDA) defines a brand-name drug as a drug marketed under a proprietary, trademark-protected name. Generic drugs are identical to traditional brand-name drugs in dosage, safety, strength, route of administration, quality, performance characteristics, and intended use.

[25] Examples of such legislation in the 114th Congress include H.R. 2739, S. 1566, and H.R. 1600.

specialty drugs,[26] and many have prohibited insurers from charging consumers higher cost sharing for certain newer specialty cancer drugs than for existing treatments.[27] In addition, some ACA state health insurance exchanges have moved to limit prescription drug cost sharing in exchange-offered insurance plans.[28] This report provides information about the specialty drug market and insurance coverage. To put specialty drug development, distribution, and spending in context, the report also provides information about broader U.S. prescription drug pricing, insurance, and regulatory trends.

U.S. Prescription Drug Market

The United States is the world's largest pharmaceutical market, making up more than one-third of total global drug spending.[29] Roughly 10 cents of every U.S. health care dollar is spent on prescription drugs ($305 billion in 2014).[30]

According to federal data, from 1980 through 2007, U.S. prescription drug spending rose by about 11% annually, on average.[31] From 2008 through 2013, the pace of annual drug spending slowed to about 2% on average.[32] (See **Figure 1** for annual growth rates.) There were several

[26] Health insurance is mainly regulated by the states. See Alex Wayne, "Drugmakers Turn Heat on Insurers by Backing Copay Limits," *Bloomberg*, March 12, 2015, at http://www.bloomberg.com/news/articles/2015-03-12/drugmakers-turn-heat-on-insurers-by-backing-copay-limits-health;and Patrick Connole, "States Target Prescription Drugs in 'Cap The Copay' Bills; Analysts Warn of Offsets," *AIS Health*, April 20, 2015, at http://aishealth.com/archive/nhpw042015-02.

[27] Michelle Andrews, "Some States Mandate Better Coverage of Oral Cancer Drugs," *Kaiser Health News*, May 14, 2012, at http://www.kaiserhealthnews.org/features/insuring-your-health/2012/cancer-drugs-by-pill-instead-of-iv-michelle-andrews-051512.aspx; and Bo Wang, Steven Joffe, and Aaron Kesselheim,"Chemotherapy Parity Laws: A Remedy for High Drug Costs?," *JAMA Internal Medicine*, November 2014, at http://archinte.jamanetwork.com/article.aspx?articleid=1907003.

[28] Covered California, "Covered California Board Protects Consumers Against Skyrocketing Specialty Drug Costs To Ensure Access To Vital Medications," May 21, 2015, http://news.coveredca.com/2015/05/covered-california-board-protects.html.

[29] IMS Institute for Health Informatics, *Global Outlook for Medicines Through 2018*, November 2014, p. 1, at http://www.imshealth.com/portal/site/imshealth/menuitem.762a961826aad98f53c753c71ad8c22a/?vgnextoid=266e05267aea9410VgnVCM10000076192ca2RCRD.

[30] CMS, *National Health Expenditure Projections, 2014-2024- Tables*, Table 2, at https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/NationalHealthAccountsProjected.html. The United States is projected to spend $305 billion on prescription drugs in 2014, out of total health care spending of about $3.08 trillion. The National Health Expenditure estimates are based on U.S. Census Bureau and IMS drug data. The figures include retail sales of prescription drugs, subtract manufacturer rebates, and add in government spending for drugs provided by government-owned mail-order facilities. They do not include drugs dispensed in institutional settings such as hospitals or clinics. See "National Health Expenditure Accounts Methodology Paper 2010: Definitions, Sources and Methods," at http://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/downloads/dsm-10.pdf.

[31] CMS, *National Health Expenditure Projections, 2014-2024* and *Historical Data*, at http://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/NationalHealthAccountsProjected.html. For additional background, see Centers for Disease Control, *Health United States 2013*, May 14, 2014, p. 30, at http://www.cdc.gov/nchs/data/hus/hus13.pdf#listfigures. By comparison, overall health care spending grew by a forecast 5.5% in 2014, with hospital services growing by 4.4% and physician and clinical services growing by 4.8%.

[32] CMS, *National Health Expenditure Projections, 2014-2024*, Table 11, at http://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/NationalHealthAccountsProjected.html. For additional analysis of prescription drug spending, see Glen Schumock, Edward Li, Katie Suda, Linda Matusiak, Robert Hunkler, Lee Vermeulen, and James Hoffman, "National Trends in Prescription Drug Expenditures and Projections for 2014," *American Journal of Health-System Pharmacy*, vol. 71 (2014), pp. 6-23, at

reasons for the recent slowdown, including the 2007 economic recession, which also helped reduce overall U.S. health care costs;[33] the increasing use of insurer drug-utilization controls; and the introduction of fewer blockbuster, brand-name drugs than in previous years. Rising utilization of lower-cost generic drugs was a major factor in holding down costs, as patents for a number of best-selling brand name drugs expired.[34]

Hatch-Waxman Act

In 1984, Congress enacted the Hatch-Waxman Act to spur the development of lower-cost generic drugs. Generic drugs are identical to traditional brand-name drugs in dosage, safety, strength, route of administration, quality, performance characteristics, and intended use.[35] The act provided manufacturers of innovative prescription drugs with patent protection and a period of marketing exclusivity; created a generic drug approval process to help companies bring products to the market more quickly once the patent for an original brand-name drug expired; and established procedures for resolving patent disputes arising from applications to market generic drugs.[36] Consumers and health care payers can realize significant savings from generic drugs, which can cost 75%-80% less than an original brand-name drug.[37] The average price of a brand-name drug also may decline after a generic comes to the market. Only 19% of prescriptions were filled with generics when Hatch-Waxman was enacted. The generic market share rose to 86% in 2013 and accounted for 28% of U.S. drug spending.

In its latest forecast for national health spending, the Centers for Medicare & Medicaid Services (CMS) projected that U.S. prescription drug spending rose by 12.6% in 2014, due in part to increased specialty drug use, and would average 6.3% annual growth from 2015 through 2024.[38] (See **Figure 1**.) CMS says ACA implementation is helping drive the higher spending, as millions of Americans become newly insured or obtain more comprehensive coverage, including prescription drug benefits.[39] The improving economy and improved drug adherence are other

http://www.ashpmedia.org/AJHP/DrugExpenditures-2014.pdf.

[33] Prescription drug demand declined after the onset of the 2007 recession and recovered slowly. IMS Institute for Healthcare Informatics, *Medicine Use and Shifting Costs of Healthcare: A Review of the Use of Medicines in the U.S. in 2013*, April 2014, p. 7, at http://www.imshealth.com/portal/site/imshealth/menuitem.762a961826aad98f53c753c71ad8c22a/?vgnextoid=2684d47626745410VgnVCM10000076192ca2RCRD.See also Andrea Sisko, et al., "National Health Expenditure Projections, 2013–23: Faster Growth Expected With Expanded Coverage And Improving Economy," *Health Affairs*, vol. 33, no. 10 (October 2014), pp. 1-10.

[34] IMS Institute for Healthcare Informatics, "M*edicine Use and Spending Shifts: A Review of the Use of Medicines in the U.S. in 2014*, April 2015, p. 6, at http://www.imshealth.com/portal/site/imshealth/menuitem.762a961826aad98f53c753c71ad8c22a/?vgnextoid=2684d47626745410VgnVCM10000076192ca2RCRD. Over the past several years, patents for a record number of top-selling traditional drugs expired, a situation that market analysts dubbed the *patent cliff*. The patent expirations paved the way for manufacturers to market an array of new generic substitutes, helping to hold down overall drug prices.

[35] FDA, "Understanding Generic Drugs," at http://www.fda.gov/Drugs/ResourcesForYou/Consumers/BuyingUsingMedicineSafely/UnderstandingGenericDrugs/.

[36] CRS Report R41114, *The Hatch-Waxman Act: Over a Quarter Century Later*, by Wendy H. Schacht and John R. Thomas.

[37] Congressional Budget Office, *Effects of Using Generic Drugs on Medicare's Prescription Drug Spending*, September 15, 2010, at https://www.cbo.gov/publication/21800.

[38] CMS, *National Health Expenditure Projections, 2014-2024, Forecast Summary*, at http://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/Downloads/proj2014.pdf. By comparison, overall health care spending grew by a forecast 5.5% in 2014, with hospital services growing by 4.4% and physician and clinical services growing by 4.8%.

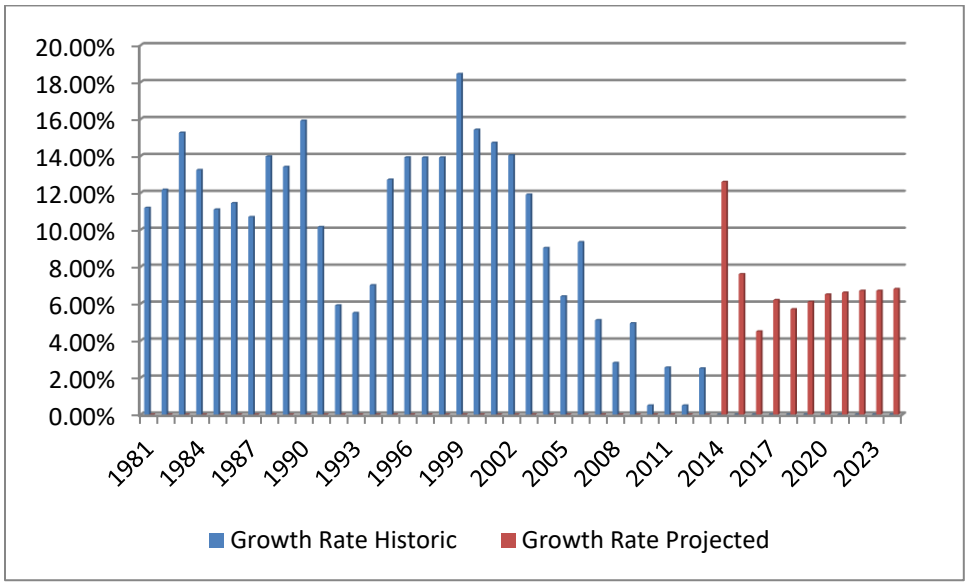
[39] See CRS Report R42663, *Health Insurance Exchanges Under the Patient Protection and Affordable Care Act (ACA)*, by Bernadette Fernandez and Annie L. Mach. Patient Protection and Affordable Care Act (ACA; P.L. 111-148, as amended) coverage was effective January 1, 2014. The ACA also expanded the Medicare Part D benefit. CRS Report R41196, *Medicare Provisions in the Patient Protection and Affordable Care Act (PPACA): Summary and Timeline*,

noted factors, along with the fact that Americans are using more prescription drugs for longer periods of time to treat chronic ailments such as diabetes or heart disease.

Some analysts predict that generic drug utilization will level off at about 91%-92% of filled prescriptions in the next several years, meaning generic substitution could play a smaller role in limiting drug spending. [40] In addition to the fact that fewer blockbuster, traditional drugs will lose patent protection than has been the case during the past several years, a greater share of drugs under development are biologics for which there are not many lower-cost substitutes. Manufacturers also have increased prices for a number of existing brand-name and generic drugs.[41]

Figure 1. U.S. Prescription Drug Spending 1980-2024

(annual percentage change from previous year)



Sources: Centers for Medicare & Medicaid Services (CMS), *National Health Expenditure Projections, 2014-2024*, Table 11, and CMS Historical Data.

Specialty Drugs

As previously noted, specialty drugs are broadly described as prescription drugs that are expensive; need special handling or administration, such as drugs that are infused or injected; have limited distribution; are targeted at a narrow group of chronic diseases; or are biologics.[42]

coordinated by Patricia A. Davis.

[40] Catamaran, *2015 Informed Trends: Moments of Opportunity*, April, 2014, at http://catamaranrx.com/Insights/Trend-Report/.

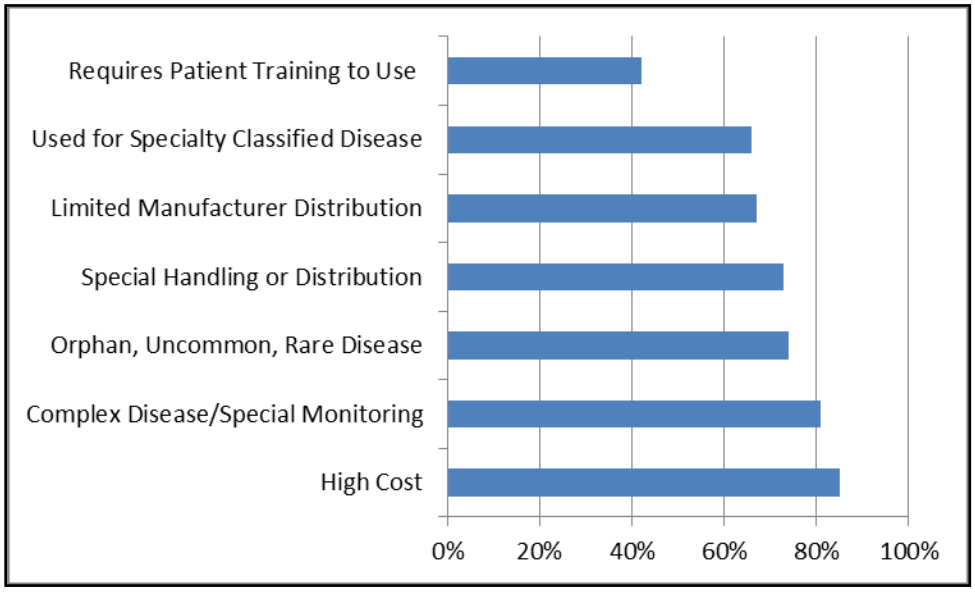
[41] Senate Committee on Health, Education, Labor and Pensions, Subcommittee on Primary Health and Aging, "Why Are Some Generic Drugs Skyrocketing in Price," November 20, 2014, at http://www.help.senate.gov/hearings/hearing/?id=a7beb0ef-5056-a032-521e-c63f76dda7f3.

[42] Gayle C. Johnston and Miguel Rodriguez, "The Facts, Figures and Trends in U.S. Pharmaceutical Distribution," Distribution Management Association's Center for Healthcare Supply Chain Research, powerpoint, March 8-11, 2015, p. 22, at http://www.hcsupplychainresearch.org/projects/pdfs/2012-SpecPharm-ExecSummary.pdf https://www.google.com/search?q=2013Specialty+Pharmaceuticals+Facts%2C+Figures+and+Trends+in+Healthcare&ie=utf-8&oe=utf-8.

(See **Figure 2**.) Even those general categorizations do not hold across all insurers and government health care programs. In the voluntary Medicare Part D program, for example, price is the main factor used to determine whether an insurer may classify a drug as a specialty product and impose higher cost sharing.[43]

Figure 2. Factors Determining Specialty Drug Designation

(leading criteria for specialty drug determination cited by managed care plans)



Source: EMD Serono *Specialty Digest,* 10th Edition, p. 10.

Notes: Data are based on a survey of 91 Medicare Advantage and commercial managed care health plans representing 124 million covered lives. For those plans that cited high cost as a factor, 86% defined high cost as more than $600 per month.

Biologics

Many specialty drugs are biologics. Biologics are products derived from a living organism that can be many times the size of a conventional (small-molecule) drug and have a more complex structure. Biologics may be sensitive to heat and contamination, making them more difficult to ship and store. Biologics often must be injected, although a growing number are available in oral form. Examples of biologics include monoclonal antibodies for treating cancer,[44] botox, and shingles and flu vaccines.[45] Pharmaceutical firms are focusing on development of biologic drugs,

[43] 42 C.F.R 423.578(a)(7). CMS regulations allow private insurers that offer Part D plans to place prescriptions with a negotiated price of $600 per month or more on a specialty price tier, where the insurers can charge enrollees up to 33% of the price of the drug. The $600 threshold was instituted in 2008 and has not been raised since that time. See CMS, "Medicare Part D Specialty Tier," April 2014, at http://www.cms.gov/Medicare/Prescription-Drug-Coverage/PrescriptionDrugCovGenIn/Downloads/SpecialtyTierMethodology.pdf. Also see "Tiered Formularies."

[44] Antibodies are proteins produced by the immune system in response to foreign proteins, or antigens. Monoclonal antibodies are developed to target specific antigens, such as those on cancer cells.

[45] FDA, "Overview of Biologic Products," webinar, at http://www.slideshare.net/robpuopolo/061713-biological-product-fda-basics-webinar-2.

which accounted for about 22% of sales by the world's top pharmaceutical companies in 2013, according to research.[46]

Congress has provided 12 years of product exclusivity for certain biologic drugs,[47] which limits manufacturers' initial market competition and increases their pricing power. Lawmakers also have attempted to spur development of lower-cost *biosimilar* products, similar to earlier efforts to stimulate development of generic products. Congress enacted the Biologics Price Competition and Innovation Act of 2009 (BPCIA) as Title VII of the ACA.[48] The ACA/BPCIA gives the U.S. Food and Drug Administration (FDA) authority to license products shown to be biosimilar to or interchangeable with an FDA-licensed biological product.[49] The Congressional Budget Office (CBO) has estimated that the ACA/BPCIA eventually could reduce insurer and consumer spending for biologics.[50]

Orphan Drugs

Many orphan drugs (which often are biologics) are classified as specialty drugs by insurers and other payers. An orphan drug is a drug targeted at a rare disease or condition (1) affecting fewer than 200,000 persons in the United States or (2) affecting more than 200,000 persons in the United States but for which there is no reasonable expectation that the sales of the drug will be sufficient to offset the costs. [51] The Orphan Drug Act of 1982 (P.L. 97-142) provides seven years of marketing exclusivity, tax credits, and FDA assistance with the review process as incentives for pharmaceutical firms to develop such drugs.[52]

Of the 41 novel new drugs approved by the FDA in calendar year 2014, 41% were orphan drugs, the highest annual total since passage of the Orphan Drug Act.[53]

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[46] *The Economist*, "Going Large," January 3, 2015, http://www.economist.com/news/business/21637387-wave-new-medicines-known-biologics-will-be-good-drugmakers-may-not-be-so-good; and Sarah Turk, IBISWorld Industry Report 32541, "Brand Name Pharmaceutical Manufacturing in the US," April 2015.

[47] For information see CRS Report R42890, *The Role of Patents and Regulatory Exclusivities in Pharmaceutical Innovation*, by John R. Thomas.

[48] CRS Report R41483, *Follow-On Biologics: The Law and Intellectual Property Issues*, by John R. Thomas.

[49] Ibid. A biosimilar is defined as a biological product if it is "highly similar to the reference product notwithstanding minor differences in clinically inactive components" and "there are no clinically meaningful differences between the [biosimilar] and the reference product in terms of safety, purity, and potency of the product." The ACA provides a period of exclusivity for manufacturers of certain biologic brand-name drugs and biosimilar products. (42 .U.S.C. §262(i)(2).) The FDA has a major role in setting standards for biosimilar drugs and has begun the process of implementing the ACA provisions. See also FDA, "Biosimilars," at http://www.fda.gov/drugs/developmentapprovalprocess/howdrugsaredevelopedandapproved/approvalapplications/therapeuticbiologicapplications/biosimilars/default.htm.

[50] Congressional Budget Office, "S. 1965: Biologics Price Competition and Innovation Act of 2007," at http://www.cbo.gov/sites/default/files/cbofiles/ftpdocs/94xx/doc9496/s1695.pdf; and Department of Health and Human Services, Office of the Assistant Secretary for Planning and Evaluation, *Expanding the Use of Generic Drugs*, December 2010, p. 10, at http://aspe.hhs.gov/sp/reports/2010/genericdrugs/ib.pdf.

[51] FDA, "Developing Products for Rare Diseases and Conditions," at http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/ucm2005525.htm.

[52] CRS Report R42890, *The Role of Patents and Regulatory Exclusivities in Pharmaceutical Innovation*, by John R. Thomas.

[53] FDA, "CDER New Drug Review: 2014 Update," December 11, 2014, at http://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ucm074833.htmand http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugInnovation/ucm429873.htm.

Manufacturers often set high prices for orphan drugs.[54] Pharmaceutical companies point out that they have a narrow patient population from which to recoup development and marketing costs. Some orphan drugs expand beyond their target market if they are effective for treating other conditions that were not part of the original FDA approval process (a situation known as off-label use).[55] Some orphan drugs have reached blockbuster status, meaning they have sales of more than $1 billion per year. [56]

Specialty Drug Spending Trends

Specialty medications grew from 23% of total U.S. prescription drug spending in 2010 to 33% in 2014 and accounted for about 73% of overall drug spending growth during that period, according to one analysis.[57] PBM data show that specialty drug spending has been growing much faster than spending on traditional drugs. For example, businesses and commercial insurers served by PBM Express Scripts posted a 30.9% increase in specialty drug spending in 2014, compared with a 6.4% rise for traditional drugs.[58]

Price inflation has been the main driver of specialty drug spending in recent years, but volume growth played a larger role in 2014 because 161,000 people began treatment with hepatitis C drugs. According to IMS Health, spending for hepatitis C drugs amounted to $12.3 billion in 2014, with $11.3 billion of that total coming from spending on newly introduced hepatitis C drugs.[59] Medicare Part D spent $4.5 billion on new hepatitis C medications in 2014, compared with $286 million that the program spent on earlier-generation hepatitis C drugs in 2013.[60]

Specialty drug spending in private and public health plans has been concentrated on a relatively narrow range of therapy areas including oncology, autoimmune diseases, HIV/AIDs, multiple sclerosis, hepatitis C, growth factors, and hormones for red cell production.[61]

For many insurers, the comparatively small share of enrollees who use specialty drugs accounts for a disproportionately large share of total prescription drug spending. For example, in Medicare Part D, specialty tier drugs made up 0.25% of prescriptions filled by enrollees in 2013 but more

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[54] Catamaran, *2014 Informed Trends: Moments of Opportunity*, p. 13, April 16, 2014, p. 8, at https://trendreport.catamaranrx.com/Catamaran. According to the report, a sampling of orphan drugs launched from 2012 to 2013 found at least five with annual costs close to or exceeding $150,000 per patient. Costs for another three orphan drugs approached $300,000 or more per patient per year.

[55] Aaron Kesselheim, Jessica Myers, Daniel Solomon, Wolfgang Winkelmayer, Raisa Levin, and Jerry Avorn, "The Prevalence and Cost of Unapproved Uses of Top-Selling Orphan Drugs," *PLoS ONE*, vol. 7, no. 2 (February 21, 2012), at http://www.ncbi.nlm.nih.gov/pmc/articles/PMC3283698/.

[56] EvaluatePharma, "Orphan Drug Report 2014," October 2014, at http://info.evaluategroup.com/rs/evaluatepharmaltd/images/2014OD.pdf; and Sara Reardon, "Regulators Adopt More Orphan Drugs," *Nature*, April 1, 2014, at http://www.nature.com/news/regulators-adopt-more-orphan-drugs-1.14970.

[57] IMS Institute for Healthcare Informatics, *Medicines Use and Spending Shifts: A Review of the Use of Medicines in the U.S. in 2014*, April 2015, p. 8.

[58] Express Scripts, *The 2014 Drug Trend Report*, March 2015, p. 11, at http://lab.express-scripts.com/drug-trend-report.

[59] IMS Institute for Healthcare Informatics, *Medicines Use and Spending Shifts: A Review of the Use of Medicines in the U.S. in 2014*, April 2015, pp. 8-9.

[60] Charles Ornstein, "New Hepatitis C Drugs are Costing Medicare Billions," *ProPublica*, March 29, 2015, reprinted in the *Washington Post* at http://www.washingtonpost.com/national/health-science/medicare-spent-45-billion-on-new-hepatitis-c-drugs-last-year-data-shows/2015/03/29/66952dde-d32a-11e4-a62f-ee745911a4ff_story.html.

[61] Waseem Noor and Michael Kleinrock, "Pharma 50 Insight: The Accelerating Growth of Specialty Markets," *Pharmaceutical Executive*, June 2014, at http://www.imshealth.com/deployedfiles/consulting/Global/Content/How%20We%20Help/Strategy%20&%20Portfolio/pharma50_2014.pdf.

than 11% of total Part D drug spending.[62] In the private sector, PBM Prime Therapeutics estimated that specialty prescriptions made up less than one-half of 1% of commercial insurance claims in 2013 but 20% of pharmacy benefit spending for the Blue Cross and Blue Shield plans it served. According to CVS Caremark, 3.6% of its enrollees in the health plans it served used specialty drugs in 2013, which accounted for 20% of prescription drug spending at retail pharmacies and nearly the same share of pharmacy spending in hospitals and other institutions.[63]

Health care payers have been scrambling to adjust to the changing drug marketplace. For example, health care actuaries have been having difficulty projecting future costs for specialty drugs, which in turn affects insurers' ability to accurately bid to offer prescription drug coverage to consumers.[64]

Costs and Benefits of Specialty Drugs

Manufacturers justify specialty drug prices based on the cost of bringing a new product to market and the potential benefits of the drugs. Specialty pharmaceuticals may improve a patient's quality of life or provide a cure, which, in turn, can provide offsetting savings to the health care system by way of fewer hospitalizations and other medical procedures.

Although publicly traded pharmaceutical manufacturers release information regarding aggregate company research and development spending, detailed information on the costs of developing specific drugs generally is not readily available. An oft-cited study put the average cost of developing a new prescription drug at about $802 million in 2001. The study was updated in 2014 to a cost of $2.6 billion.[65] There has been considerable debate among researchers about the estimate,[66] with a number of analysts saying that the true cost is likely to be lower.[67] Further, development costs for different drugs vary.[68]

Some targeted studies have looked at the costs and benefits of specific biologic and high-cost drugs. For example, a 2008 study found that using biologic drugs to treat rheumatoid arthritis and multiple sclerosis reduced the use of some other types of medical services. In this case, the savings did not offset the full cost of the drugs.[69] A study of biologics used to treat colorectal

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[62] CMS, "Medicare Part D Specialty Tier," April 7, 2014, at http://www.cms.gov/Medicare/Prescription-Drug-Coverage/PrescriptionDrugCovGenIn/Downloads/SpecialtyTierMethodology.pdf.

[63] See Prime Therapeutics, *Looking Back Moving Forward, 2014 Report on Prescription Drug Costs*, p. 14; and CVS Caremark, *Specialty Trend Management, Where to Go Next*, November 20, 2013, p. 3, at http://www.cvshealth.com/sites/default/files/Insights%202013.pdf.

[64] American Academy of Actuaries, Letter to Centers on Medicare & Medicaid Services, November 11, 2014, at http://www.actuary.org/files/Medicaid_capitation_rates_and_BTD_medications_Letter_to_CMS_Nov%2011.pdf.

[65] Tufts Center for the Study of Drug Development, "Cost to Develop and Win Marketing Approval for a New Drug Is $2.6 Billion," November 18, 2014, at http://csdd.tufts.edu/news/complete_story/pr_tufts_csdd_2014_cost_study.

[66] Christopher Adams and Van Brantner,"Estimating the Cost of New Drug Development. Is it Really $802 Million?" *Health Affairs*, vol. 25, no. 2 (March 2006), pp. 420-428, at http://content.healthaffairs.org/content/25/2/420.long; Donald Light and Rebecca Warburton, "Demythologizing the High Costs of Pharmaceutical Research," *BioSocieties*, London School of Economics, 1-17, 2011, at http://www.pharmamyths.net/files/Biosocieties_2011_Myths_of_High_Drug_Research_Costs.pdf.

[67] CRS Report R41705, *The National Institutes of Health (NIH): Background and Congressional Issues*, by Judith A. Johnson.

[68] David Austin, *Research and Development in the Pharmaceutical Industry*, Congressional Budget Office, 2006, at http://www.cbo.gov/sites/default/files/cbofiles/ftpdocs/76xx/doc7615/10-02-drugr-d.pdf.

[69] Geoffrey Joyce, Dana Goldman, Pinar Karaca-Mandic and Grant Lawless, "Impact of Specialty Drugs on Use of Other Medical Services," *American Journal of Managed Care*, vol. 14, vo. 12 (December 2008), pp. 821-828, at

cancer indicated that the drugs improved outcomes and life expectancy but created large increases in total expenditures and did not substitute for other medical services.[70]

A recent study[71] examined the benefit-cost ratio of Sovaldi and another specialty hepatitis C drug, Harvoni. The study, which assumed an 11% average price discount for the drugs across payers,[72] found that the drugs, which can provide a cure, were cost-effective in selected patient groups at a threshold where each additional quality-of-life year was valued at $50,000 and were cost-effective for most patients at a $100,000 threshold.[73] However, the study noted that the resources needed to treat a large number of eligible patients could be "immense and unsustainable."

Recent research indicates that the price of new cancer therapies, many of which are specialty drugs, increased by 10% a year (adjusted for inflation and health benefits) from 1995 to 2013. The authors posit that manufacturers were able to set the prices of new products at or slightly above the prices of existing therapies. Government-required rebates and other discounts may have contributed to higher launch prices as manufacturers tried to make up for the discounts by raising prices in other parts of the market.[74]

There are increased efforts to provide research on the possible benefits and costs of specialty drugs. During the next two years, for example, the Institute for Clinical and Economic Review plans to produce 15 to 20 public reports on newly approved FDA high-impact drugs. The reports will analyze the drugs' comparative effectiveness, cost-effectiveness, and potential budget impact.[75]

Specialty Drug Spending Controls

Private employers and insurers are the nation's largest purchasers of prescription drugs, accounting for a projected 43% of annual spending in 2013, followed by the federal and state governments at about 41% and consumer out-of-pocket spending at about 16%. (See **Figure 3** for

http://www.ncbi.nlm.nih.gov/pmc/articles/PMC3767569/. The authors suggested that insurers would be better off finding ways to manage utilization so that patients who would benefit from the drugs had access to them, rather than restricting access through high patient cost sharing or formulary requirements designed to deter use by all patients, regardless of clinical need.

[70] Pinar Karaca-Mandic, Jeffrey McCullough, Mustaqeem Siddiqui, Holly Van Houten, and Nilay Shah, "Impact of New Drugs and Biologics on Colorectal Cancer Treatment and Costs," *Journal of Oncology Practice*, vol. 7, no. 3 (May 2011), pp. e30s–e37s, at http://www.ncbi.nlm.nih.gov/pmc/articles/PMC3092464/. David Howard, John Kauh, and Joseph Lipscomb, "The Value of New Chemotherapeutic Agents for Metastatic Colorectal Cancer," *Archives of Internal Medicine*, vol. 170, no. 6 (March 22, 2010); pp.537-542, at http://www.ncbi.nlm.nih.gov/pubmed/20233802/.

[71] Jagpreet Chhatwal, Fasiha Kanwal, Mark Roberts, and Michael Dunn; "Cost-Effectiveness and Budget Impact of Hepatitis C Virus Treatment with Sofosbuvir and Ledipasvir in the United States," *Annals of Internal Medicine*, vol. 162, no. 6 (March 17, 2015), pp. 397-406, at http://annals.org/article.aspx?articleid=2197176.

[72] Ibid. The study estimated the average discount on drugs given to payers based on the discounts for Sovaldi. According to the study, private, VA, Medicare, and Medicaid discounts were 14%, 44%, 0% and 23%, respectively. By then adjusting for the number of patients under each insurance type, the study estimated the average discount was 11%. The authors used projections of expected quality-adjusted life-years, total lifetime costs, and cost of antiviral drugs to estimate the incremental cost-effectiveness of new drugs compared with previous treatments. See the study for more information.

[73] See "Sovaldi as Case Study of Payer Negotiations."

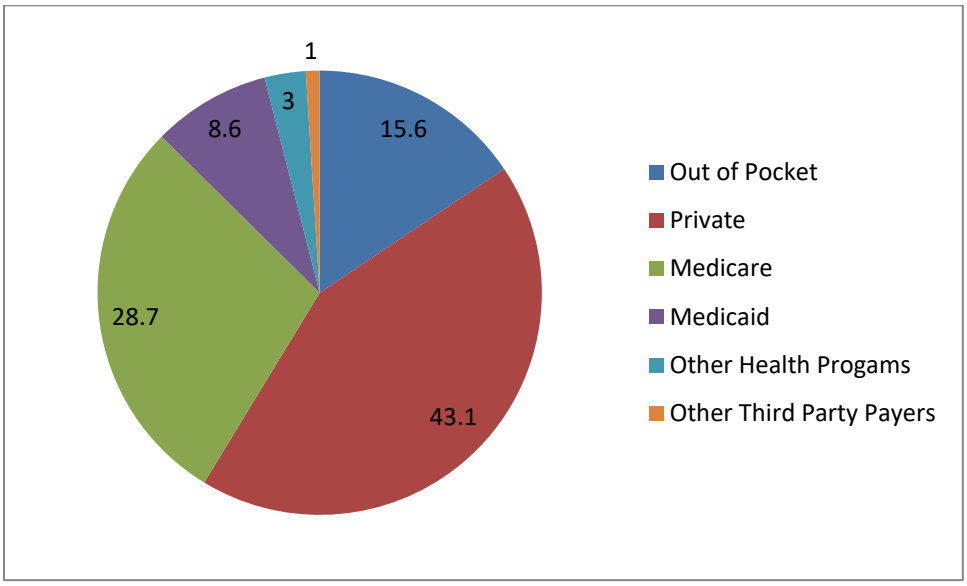
[74] David Howard, Peter Bach, Ernst Berndt, and Rena Conti,"Pricing in the Market for Anticancer Drugs," *Journal of Economic Perspectives*, vol. 29, no. 1(Winter 2015), pp. 139–162, at http://pubs.aeaweb.org/doi/pdfplus/10.1257/jep.29.1.139.

[75] Institute for Clinical and Economic Review, "Emerging Therapy Assessment and Pricing," at http://www.icer-review.org/etap/.

prescription drug spending by source.) The health payers use a variety of strategies to control specialty drug spending.

Figure 3. 2014 U.S. Spending for Prescription Drugs by Source

(by percentage of total spending)



Source: Centers for Medicare & Medicaid Services, Office of the Actuary, *National Health Expenditures 2014-2024*, Table 11.

Notes: The category *other health programs* includes the state Children's Health Insurance Program, Department of Defense, and Department of Veterans Affairs. *Other third party payers* includes worksite health care, other private revenues, Indian Health Service, workers' compensation, general assistance, maternal and child health, vocational rehabilitation, other federal programs, Substance Abuse and Mental Health Services Administration, other state and local programs, and school health.

Enrollee Utilization Policies

Health care payers pass on a portion of specialty drug costs to enrollees through plan premiums and annual deductibles.[76] Many payers also use targeted management tools including (1) requiring enrollees to pay higher cost sharing for expensive drugs (tiered formularies); (2) requiring prior authorization before covering certain medications; (3) mandating that enrollees try

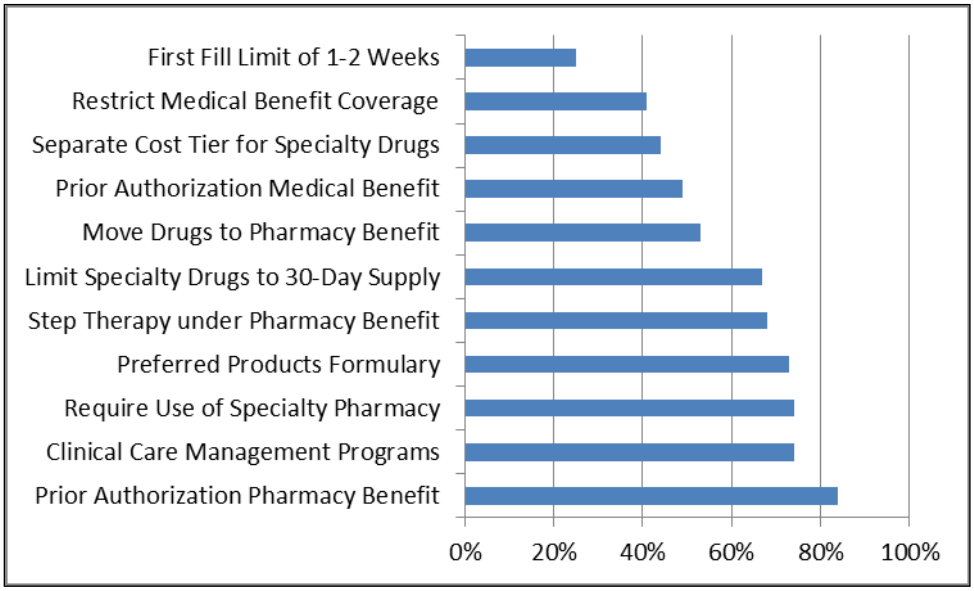
[76] About 20% of insured consumers are now enrolled in high-deductible health plans (HDHP) that have lower premiums and higher deductibles than traditional insurance plans. Some HDHPs cover preventive care, including vaccinations and certain drugs, before an enrollee has met the plan deductible. In other HDHP plans, employees must cover prescription costs out-of-pocket until they reach a set-dollar deductible. According to America's Health Insurance Plans (AHIP), there were nearly 17.4 million enrollees in HDHPs in January 2014, up from 11.4 million in January 2011. (AHIP, "New Census Survey Shows Increased Growth in HSA Enrollment," July 9, 2014, at https://www.ahip.org/Press-Room/2014/HSA-Census-Survey/.) HDHP enrollees may pay for drugs and other services with tax-advantaged health savings accounts or may have access to other, employer-provided health reimbursement. See CRS Report RS21573, *Tax-Advantaged Accounts for Health Care Expenses: Side-by-Side Comparison, 2013*, by Carol Rapaport. Some health insurance plans sold on state exchanges impose deductibles for prescription drugs or include prescription drugs in their overall deductibles. Average prescription-drug-only deductibles ranged from $134 to $465 in 2015. Kaiser Family Foundation, "Medical and Prescription Drug Deductibles for Plans Offered in Federally Facilitated and Partnership Marketplaces for 2015," November 18, 2014, at http://kff.org/health-reform/fact-sheet/medical-and-prescription-drug-deductibles-for-plans-offered-in-federally-facilitated-and-partnership-marketplaces-for-2015/.

a less expensive drug before moving to a more expensive prescription product (step therapy); (4) limiting the length of an initial prescription to assess whether a drug works as intended; (5) requiring use of a specialty pharmacy; (6) offering only a limited formulary; (7) moving a drug to a pharmacy (retail) benefit from an institutional (medical) benefit to cut overhead; and (8) requiring closer oversight and monitoring of patients using specialty drugs.[77] (See **Figure 4**.)

This report will look at some of the most commonly used strategies.

Figure 4. Percentage of Insurers Using Strategies for Managing Specialty Drug Use

(2012 commercial insurance plan data)



Source: Walgreens/Pharmacy Benefit Management Institute, *2013 Specialty Drug Benefit Report*.

Note: Based on survey of insurers and other plan sponsors covering 17.6 million enrollees.

Tiered Formularies

Payers commonly include tiered pricing to induce enrollees to use drugs that are less expensive or that are considered more effective. Under tiered pricing, a generic or preferred brand-name drug[78] is put on a tier that requires a comparatively low co-payment, and drugs that are more expensive or deemed less effective are put on tiers requiring comparatively higher co-payments or coinsurance. In 2014, 80% of consumers with employer-sponsored insurance were in plans with three or more drug tiers, and 20% were in plans with four or more tiers.[79]

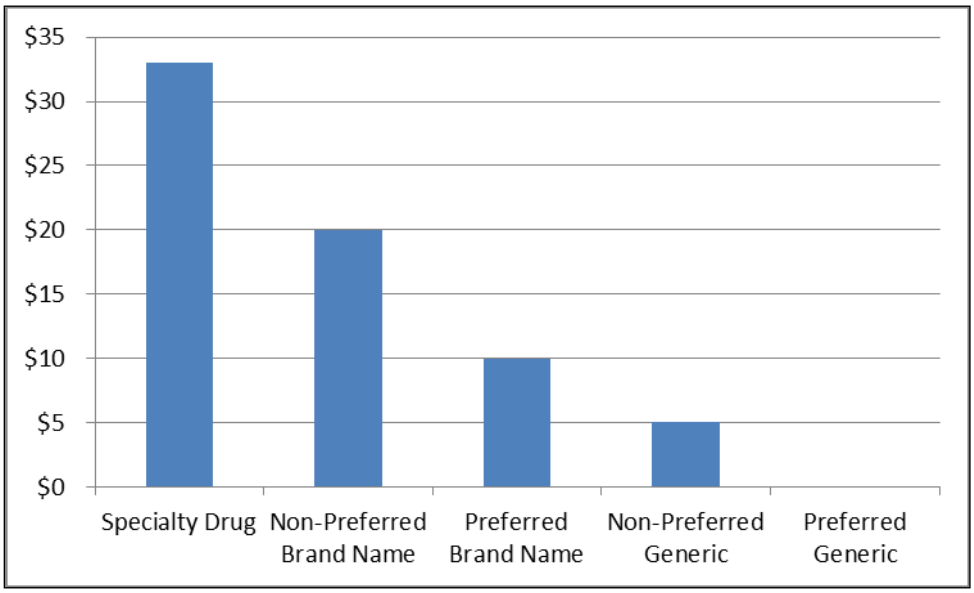
[77] Walgreens/Pharmacy Benefit Management Institute, *2014 PBMI Specialty Drug Benefit Report*, at http://reports.pbmi.com/report.php?id=5. (Registration required) The report is based on responses from 306 U.S. employers, health plans, and other plan sponsors representing 17.6 million covered lives.

[78] The FDA defines a brand-name drug as a drug marketed under a proprietary, trademark-protected name.

[79] Kaiser Family Foundation, *2014 Employer Health Benefits Survey, Section Nine: Prescription Drug Benefits*, September 10, 2014, at http://kff.org/report-section/ehbs-2014-section-nine-prescription-drug-benefits/.

Figure 5. Tiered Formulary

(example of pricing under a tiered formulary for a drug with a $100 price tag)



Source: CRS.

Notes: For purposes of this graphic, the preferred generic tier has a $0 co-payment; non-preferred generics have a $5 co-payment, preferred brand names have a $10 co-payment, non-preferred brand names have a $20 co-payment, and specialty drugs have 33% coinsurance.

Payers often place specialty drugs on a tier that requires enrollees to pay coinsurance rather than a co-payment, which helps the payer keep pace with price inflation for expensive drugs and discourages use of the drugs in cases where substitutes are available.[80] For example, a payer could impose a flat $20 co-payment for a $100 drug or it could charge 33% coinsurance for the product, which would result in $33 in out-of-pocket spending.[81] Over time, the cost differential between price tiers has widened, imposing a greater burden on enrollees prescribed higher-priced drugs.[82]

While many consumers focus on the cost of monthly premiums when deciding whether a health plan is affordable, prescription drug tiers can have a major impact on the total cost of coverage. A recent analysis of non-group health plans[83] sold through ACA insurance exchanges[84] found that

[80] Stephanie Regnier, "How Does Drug Coverage Vary by Insurance Type? Analysis of Drug Formularies in the United States," *The American Journal of Managed Care*, April 21, 2014, at http://www.ajmc.com/publications/issue/2014/2014-vol20-n4/how-does-drug-coverage-vary-by-insurance-type-analysis-of-drug-formularies-in-the-united-states.

[81] Kaiser Family Foundation, *2014 Employer Health Benefits Survey, Section Nine: Prescription Drug Benefits*, September 10, 2014, at http://kff.org/report-section/ehbs-2014-section-nine-prescription-drug-benefits/.

[82] Kaiser Family Foundation, *2014 Employer Health Benefits Survey, Section Nine*, Exhibit 9.4, at http://kff.org/report-section/ehbs-2014-section-nine-prescription-drug-benefits/. There also was wider pricing for insurers requiring coinsurance. Shelly Carrey, Pharmacy Benefit Management Institute, "8 Ways Pharmacy Benefit Design May Evolve in 2013," The Institute for Health Care Consumerism, at http://www.theihcc.com/en/communities/pharmacy_benefit_management/8-ways-pharmacy-benefit-design-may-evolve-in-2013_hfpq9mpi.html.

[83] CRS Report R42069, *Private Health Insurance Market Reforms in the Affordable Care Act (ACA)*, by Annie L. Mach and Bernadette Fernandez. On average, Bronze plans will cover 60% of a base benefit package; Silver plans, 70%; Gold plans, 80%; and Platinum plans, 90%. (Qualified health insurance plans must be certified by a state exchange and must provide a minimum level of benefits, although insurers may offer more comprehensive coverage. To receive ACA cost sharing and premium subsidies, consumers must buy qualified plans through an exchange.)

[84] Exchanges are special marketplaces where consumers can buy health plans that meet set federal requirements and

60% of the least-comprehensive Bronze plans imposed co-insurance of 30% or more for drugs on specialty tiers, with 25% requiring coinsurance of 50% or more.[85] About 23% percent of Silver plans, which cover a larger share of the federally required benefits, still had specialty-tier coinsurance of 30% of more, as did 33% of Gold plans and 10% of the most expansive Platinum plans.[86] A few plans concentrated all drugs for treating certain conditions on a specialty tier that imposed coinsurance requirements, including not just the highest-cost drugs but also less expensive drugs used to treat the conditions, raising concerns about possible discrimination against certain classes of enrollees.[87] In addition, the plans may require beneficiaries to meet a deductible before covering prescription drugs.

A number of studies have shown that when insurers impose higher cost sharing for prescription drugs, fewer enrollees fill or continue their prescriptions.[88] There is some evidence that higher cost sharing does not affect prescription adherence for specialty drugs as much it does for traditional drugs. A 2006 analysis of 50 commercial health plans found that increases in specialty drug cost sharing reduced usage by 1%-21%, compared with a 25%-45% reduction for traditional drugs.[89] A separate review analyzed private health insurance claims for biologic drugs used to treat rheumatoid arthritis. If insurers doubled the average out-of-pocket payment for the arthritis treatment, the odds that a person would not use the drugs increased by about 9%. Consumers were much more likely not to fill or not to complete a prescription for traditional drugs when out-of-pocket payments doubled.[90]

Prescription Drug Coupons

Pharmaceutical manufacturers try to mitigate the impact of tiered formularies on consumers by offering drug coupons that reduce or eliminate required co-payments or coinsurance. Manufacturers may offer their coupons through their websites, in magazines and other media, or through a physician's office. A manufacturer might offer a coupon that limits a consumer's co-payment to $50 for a drug that has a negotiated price of $500, even if the consumer's health plan requires a $150 co-payment. In this case, the manufacturer provides a $100 subsidy to cover the difference between the coupon price limit and the co-payment amount.

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also qualify for federal premium subsidies to defray part of the cost of the plans. See CRS Report R42663, *Health Insurance Exchanges Under the Patient Protection and Affordable Care Act (ACA)*, by Bernadette Fernandez and Annie L. Mach.

[85] Avalere, "Exchange Plans Increase Costs of Specialty Drugs for Patients in 2015." Link to study available at Caroline Pearson, Avalere Health, "Consumers Likely Face High Out-Of-Pocket Costs for Specialty Drugs In Exchange Plans," at http://avalerehealth.net/expertise/managed-care/insights/consumers-likely-face-high-out-of-pocket-costs-for-specialty-drugs-in-excha.The study looked at 603 plan designs offered by 60 different carriers in 19 states.

[86] Ibid.

[87] See "Outstanding Issues."

[88] Dana Goldman, Geoffrey Joyce, Yuhui Zheng, "Prescription Drug Cost Sharing: Associations With Medication and Medical Utilization and Spending and Health," *Journal of the American Medical Association*, vol. 298, no. 1 (July 4, 2007), at http://jama.jamanetwork.com/article.aspx?articleid=207805.

[89] RAND Health, "Prescription Drug Cost-Sharing: A Powerful Policy Lever to Use with Care," 2009, at http://www.rand.org/content/dam/rand/pubs/research_briefs/2009/RAND_RB9474.pdf; and Dana Goldman, Geoffrey Joyce, Grant Lawless, William Crown, and Vincent Willey, "Benefit Design and Specialty Drug Use," *Health Affairs*, vol. 25, no. 5 (September 2006), pp. 1319-1331, at http://content.healthaffairs.org/content/25/5/1319.full.

[90] Pinar Karaca-Mandic, Geoffrey Joyce, Dana Goldman, and Marianne Laouri, "Cost Sharing, Family Health Care Burden, and the Use of Specialty Drugs for Rheumatoid Arthritis," *Health Service Research*, Oct 2010; vol. 45, no. 5, part I (October 2010), pp. 1227–1250, at http://www.ncbi.nlm.nih.gov/pmc/articles/PMC2965502/.

Coupons appear to be particularly important for consumers using specialty drugs. PBM Prime Therapeutics found the insurance beneficiaries it served used manufacturer coupons to cover $21.2 million out of a total of $35.3 million in annual out-of-pocket costs for specialty drugs in 2013.[91] The beneficiaries used coupons to reduce the average out-of-pocket spending for anti-inflammatory drugs by nearly 77% and to realize nearly 61% in savings on drugs for treating multiple sclerosis. The coupons helped increase prescription adherence (whether patients take their drugs as prescribed, such as three times daily, and whether they continue the full course of treatment). However, because the coupons were very effective in lowering required co-payments, they also may have induced some consumers to use more expensive drugs when less expensive substitutes may have been available, according to the study.

Some health payers and PBMs prohibit enrollees from using coupons for certain drugs.[92] Pharmaceutical manufacturers may be liable under the federal anti-kickback statute if they offer coupons to induce consumers to purchase their companies' products under federal programs, such as the Medicare Part D prescription drug benefit. The federal anti-kickback statute[93] makes it a felony for a person to knowingly and willfully offer, pay, solicit, or receive anything of value (i.e., *remuneration*) in return for a referral or to induce generation of business reimbursable under a federal health care program.

Site of Care

Individuals covered by health insurance may obtain prescription drugs through their plan's pharmacy benefit (which covers outpatient prescriptions filled in drug stores) or through its medical benefit (which covers drugs administered by a health care provider in an institutional setting—such as a hospital outpatient center, a doctor's office, a freestanding infusion center—or in a patient's home by a special provider). As a general rule of thumb, oral drugs often are covered under a pharmacy benefit whereas infused or injected drugs, such as infused oncology drugs, are covered under a medical benefit.

According to analytics and consulting firm Artemetrx, about half of total specialty drug spending reported by commercial insurers is processed through the pharmacy benefit and half is processed through the medical benefit.[94] A number of consultants say this dual system can make it more difficult to track and control spending on specialty drugs. Because of technical differences in drug coding and reporting under the two systems, a health insurer may have difficulty monitoring the total regimen of drugs used for a patient. Insurers also may have difficulty imposing utilization

[91] Catherine Starner, G. Caleb Alexander, Kevin Bowen, Yang Qiu, Peter Wickersham, and Patrick Gleason, "Specialty Drug Coupons Lower Out-Of-Pocket Costs And May Improve Adherence At The Risk Of Increasing Premiums," *Health Affairs*, vol. 33, no. 10 (October 2014), pp. 1761-1769, at http://content.healthaffairs.org/content/33/10/1761.abstract.

[92] Andrew Pollack, "Health Insurers Pressing Down on Drug Prices," *The New York Times*, June 20, 2014, at http://www.nytimes.com/2014/06/21/business/health-plans-bring-pressure-to-bear-on-drug-prices.html.

[93] §1128B(b) of the Social Security Act. For background see CRS Legal Sidebar WSLG246, *Earning Rewards when Buying Drugs Covered by Medicare and Medicaid: HHS Gives the Green Light*, by Jennifer A. Staman.

[94] Artemetrx, *Specialty Drug Trend Across the Pharmacy and Medical Benefit*, 2013, p. 2, at http://www.artemetrx.com/docs/ARTEMETRX_Specialty_Trend_Rpt.pdf; and Prime Therapeutics, "Specialty Drugs are Forecasted to be 50% of All Drug Expenditures in 2018," at http://cdn2.content.compendiumblog.com/uploads/user/e7c690e8-6ff9-102a-ac6d-e4aebca50425/accf0d87-0d14-4aa7-bbf3-193b90c8d68c/File/1f8476cb4a60eb472893ddf381c9c3ec/prediction_that_by_2018__more_than_50_percent_of_all_drug_expenditures_will_be_specialty.pdf.

controls, such as requiring prior authorization, for drugs purchased by and administered in a physician's office.[95]

In an effort to control drug spending, some insurers have moved to coordinated drug benefits.[96] Some studies caution that potential cost savings from integrating the pharmacy and medical benefits may have been oversold. A recent analysis by Artemetrx found that prices for certain drugs actually were lower when the drugs were dispensed in a physician's office.[97] From the consumer perspective, out-of-pocket costs may be higher when a drug is covered under a tiered pharmacy benefit rather than as part of a medical service. For example, an insurer may charge 33% coinsurance under the pharmacy benefit, as opposed to a smaller, flat co-payment for a drug provided as part of a medical benefit. That has become an issue as more specialty drugs, particularly cancer drugs, are offered in oral form rather than as infused products. Dozens of states have passed laws to prohibit insurers from imposing higher out-of-pocket payments for oral cancer drugs purchased at a pharmacy than for similar infused products delivered in a medical setting.[98]

Specialty Pharmacies

Specialty drugs often are distributed through specialty pharmacies[99] that offer a range of services beyond distribution or sale, including helping to administer complex drugs that must be infused or that can have serious side effects, performing patient education, and monitoring patients' reactions to prescribed medications. Specialty pharmacies may handle paperwork associated with insurer reimbursement, manufacturer data reporting, and FDA reporting requirements. Insurers use specialty pharmacies to more closely monitor and counsel patients during a course of treatment to improve medication adherence, which is a major concern given the high cost of many specialty drugs.

A number of PBMs and insurers have purchased specialty pharmacies, and some require that the insured beneficiaries they serve fill their prescriptions through those pharmacies. Payers may be able to secure better prices through their own specialty distribution system and to monitor enrollee outcomes more closely. Some drug manufacturers have contracted directly with a limited number of specialty pharmacies to limit distribution and better control the supply chain.[100]

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[95] See "Understanding Specialty Pharmacy Management and Cost Control," Pharmaceutical Strategies Group, June 2010; and Peter Wehrwein,"Should Specialty Drugs Be Shifted From the Medical to Pharmacy Benefit?" *Managed Care*, January 2015, at http://www.managedcaremag.com/archives/2015/1/should-specialty-drugs-be-shifted-medical-pharmacy-benefit. Specialty drug spending estimates can vary depending on whether analysts include drugs purchased under both the pharmacy and medical benefit.

[96] Brenda Motheral and Corey Belkin, "An Evaluation of Specialty Drug Pricing Under the Pharmacy and Medical Benefit," Artemetrx Specialty Drug Solutions, March 2014.

[97] Ibid.

[98] Jordan Shapiro, "Missouri Governor Signs Oral Chemotherapy Bill," *Associated Press*, March 19, 2014, at http://www.newstribune.com/news/2014/mar/19/missouri-governor-signs-oral-chemotherapy-bill/; and Compass Health Analytics, "Actuarial Assessment of the Report of the Committee to Evaluate the Parity between Oral and Intravenous Chemotherapy," prepared for the State of New Hampshire Insurance Department, August 9, 2012, at http://www.nh.gov/insurance/reports/documents/nhid_oralchemoanalysis2012.pdf.

[99] The URAC, formerly known as the Utilization Review Accreditation Commission, and the Accreditation Commission for Health Care (ACHC) are organizations accrediting specialty pharmacies. See https://www.urac.org/about-urac/about-urac/.

[100] Katie Thomas and Andrew Pollack, "Specialty Pharmacies Proliferate, Along with Questions," *The New York Times*, July 15, 2015, at http://www.nytimes.com/2015/07/16/business/specialty-pharmacies-proliferate-along-with-questions.html?ref=us&_r=0. There are questions about whether PBMs that own specialty pharmacies and make money

Manufacturer-Payer Negotiations

Even though pharmaceutical manufacturers announce a price for newly developed drugs, the actual price paid is likely to vary among public and private health care payers, which in turn affects how much insured consumers pay out of pocket and the level of government and private insurer spending for a product.

In the private sector (and in some government programs), insurers and other payers and the PBMs with which they contract negotiate price concessions with manufacturers. Private health payers secure rebates from manufacturers based on the volume of drugs their enrollees are predicted to use, for example. Larger payers with a higher volume of prescription activity are likely to secure more generous rebates. Insurers and PBMs may receive a price reduction if they include a single manufacturer's drug on their formulary, while excluding competing drugs.[101] Payers may secure price concessions if they put a drug on a tier that carries a lower co-payment or coinsurance than other, similar drugs. However, in the case of specialty drugs that have few substitutes, payers may have less leverage to negotiate, at least initially.[102]

In addition to private-sector negotiations, U.S. government agencies operate a range of payment systems across and within health care programs. Among the public payer systems,

- The state-federal Medicaid program requires participating drug manufacturers to provide a minimum 23.1% price rebate to the government for innovator drugs[103] and limits annual price increases for prescription products to the rate of consumer inflation.[104] To participate in Medicaid, manufacturers must take part in the 340B Drug Pricing Program, which requires discounts on outpatient drugs to eligible health care organizations and entities.[105]
- The Veterans Health Administration (VA) operates a centralized system for buying drugs, including a central formulary.[106] The Department of Defense (DOD) and the VA use certain federal pricing arrangements for these direct purchases, including Federal Supply Schedule prices and prices available to the four largest federal purchasers. Prime vendors used by DOD and VA provide a fixed percentage discount off the lowest price otherwise available for each drug.
- The Medicare Part D program prohibits the Secretary of Health and Human Services (HHS) from negotiating drug prices with manufacturers or creating a set formulary.[107] Part D private insurers, often working through PBMs, negotiate drug prices and administer benefits. Manufacturers must provide a 50% discount

dispensing the drugs face potential conflicts of interest.

[101] "Catamaran Makes Formulary Cuts, But Preserves Specialty Access," *Drug Benefit News*, vol. 15, no. 13 (July 11, 2014), at http://aishealth.com/sites/all/files/marketplace_pdf_samples/sampledbn.pdf.

[102] See "Sovaldi as Case Study of Payer Negotiations."

[103] Innovator drugs include single-source products (typically brand-name products that have no available generic versions) and innovator multiple-source products (typically brand-name drugs that have available generic versions).

[104] Medicaid.gov, "Medicaid Drug Rebate Program," at http://www.medicaid.gov/Medicaid-CHIP-Program-Information/By-Topics/Benefits/Prescription-Drugs/Medicaid-Drug-Rebate-Program.html.

[105] CRS Report CRS Report R43778, *Medicaid Prescription Drug Pricing and Policy*, by Cliff Binder.

[106] Government Accountability Office, *Prescription Drugs: Comparison of DOD and VA Direct Purchase Prices*, April 2013, at http://www.gao.gov/assets/660/654019.pdf.

[107] CRS Report R40611, *Medicare Part D Prescription Drug Benefit*, by Suzanne M. Kirchhoff and Patricia A. Davis.

for brand-name drugs purchased by enrollees who have reached the Part D "doughnut hole," or coverage gap.[108]

Prices for brand-name prescription drugs often are higher in the United States than in European and other countries that have government-run health care systems, including a centralized mechanism for pricing and purchasing drugs.[109]

Sovaldi as Case Study of Payer Negotiations

The introduction of specialty drugs for treating hepatitis C provides an example of payer-manufacturer negotiations. In late 2013, drug maker Gilead received FDA approval for Sovaldi, a drug that offers a high cure rate for hepatitis C patients, and brought it to the market at a list price of $84,000 for a 12-week course of treatment.[110] Sovaldi's initial price was not as high as for some other specialty drugs. Whereas many specialty drugs are targeted at a narrow population, an estimated 3.2 million Americans are infected with the hepatitis C virus.[111] Private and public health payers faced the prospect of billions of dollars in up-front costs for Sovaldi, as compared with other treatments. Although the drug is expected to produce some long-term cost savings by way of reduced hospitalizations or other services, there are uncertainties about the size and timing of the reductions.[112] Further, many U.S. consumers may switch health insurance plans on an annual basis, meaning one insurer could bear the entire up-front cost of Sovaldi for an enrollee who then moved to another health plan, which then could benefit from the fact that the individual did not face ongoing medical costs for treating hepatitis C.

Public and private payers have tried to limit spending for the drug by restricting coverage for Sovaldi to those in advanced stages of the disease and requiring prior authorization, including proof that a prospective patient has not used drugs or alcohol for a certain period of time.[113] Payers entered into price negotiations with the manufacturer, Gilead, and some imposed mandatory price reductions (i.e., Medicaid requires mandatory new drug rebates of at least 23.1%, and many state Medicaid programs imposed additional controls such as prior

[108] Ibid. Beneficiaries also receive coverage for drugs under Medicare Part B, which covers certain drugs furnished in a doctor's office or as part of a medical service, and drugs that usually are not self-administered, such as drugs given by infusion or injection. Generally, Medicare reimburses physicians and other providers, such as hospital outpatient clinics, for covered Part B drugs and biologics at 106% of a drug manufacturer's average sales price. Health care providers also are paid separately for administering Medicare Part B drugs. Medicare pays 80% of the amount paid to providers, and beneficiaries are responsible for the remaining 20%. See CRS Report R40425, *Medicare Primer*, coordinated by Patricia A. Davis and Scott R. Talaga.

[109] Panos Kanavos, Alessandra Ferrario, Sotiris Vandoros, and Gerard Anderson, "Higher U.S. Branded Drug Prices and Spending Compared to Other Countries May Stem Partly from Quick Uptake of New Drugs," *Health Affairs*, vol. 32, no. 4 (April 2013), pp. 753-761.

[110] Gilead, "U.S. Food and Drug Administration Approves Gilead's Sovaldi (Sofosbuvir) for the Treatment of Chronic Hepatitis C," December 6, 2013, at http://www.gilead.com/news/press-releases/2013/12/us-food-and-drug-administration-approves-gileads-sovaldi-sofosbuvir-for-the-treatment-of-chronic-hepatitis-c. The cost of the treatment can be higher if Sovaldi is used in conjunction with other hepatitis C drugs.

[111] Centers for Disease Control, "Hepatitis C FAQs for the Public," at http://www.cdc.gov/hepatitis/c/cfaq.htm.

[112] California Technology Assessment Forum, *The Comparative Clinical Effectiveness and Value of Simeprevir and Sofosbuvir in the Treatment of Chronic Hepatitis C Infection*, Final Report, April 15, 2014, at http://ctaf.org/sites/default/files/assessments/CTAF_Hep_C_Apr14_final.pdf; and Jagpreet Chhatwal, Fasiha Kanwal, Mark Roberts, and Michael Dunn, "Cost-Effectiveness and Budget Impact of Hepatitis C Virus Treatment With Sofosbuvir and Ledipasvir in the United States," *Annals of Internal Medicine*, vol. 162, no. 6 (March 17, 2015), pp. 397-406, at http://annals.org/article.aspx?articleid=2197176.

[113] Viohl and Associates for the Medicaid Health Plans of America, "The Sovaldi® Squeeze: High Costs Force Tough State Decisions," September 29, 2014, at www.mhpa.org/_upload/SovaldiSqueeze-Oct2014.pdf.

authorization requirements).[114] Gilead also offered discount coupons for insured patients and other assistance for certain uninsured patients.[115] Gilead has said that its average discount for Sovaldi was 22% in 2014.[116] In addition to reductions in the U.S. market, Gilead provided deep discounts to payers, including government programs, in other countries.[117]

In October 2014, Gilead secured FDA approval for a second hepatitis C therapy called Harvoni, which it initially priced at around $94,000.[118] In December 2014, manufacturer Abbvie secured FDA approval for a competing hepatitis C drug called Viekira Pak, which it initially priced at $83,319.[119] As competition emerged, payers became more aggressive in price negotiations. PBM Express Scripts announced that Viekira Pak would be its exclusive option for specialty drugs to treat hepatitis C patients starting January 1, 2015.[120] Gilead secured contracts with other PBMs and payers for its drugs, but company executives said in a February 2015 conference call that they expected to provide average price discounts of about 46% on their hepatitis C drugs in 2015, compared with the average 22% discounts in 2014.[121]

Outstanding Issues

Consumer Insurance Coverage of Specialty Drugs

One of the thorniest issues facing health care payers is how to control specialty drug spending without restricting coverage to the point that some insured enrollees cannot afford needed drugs. Public and private payers say that utilization strategies such as tiered pricing merely provide an incentive for consumers to use less expensive or more effective drugs. However, many specialty therapies are one-of-a-kind products with no ready substitutes, meaning that tiered pricing mainly serves to increase cost sharing for enrollees prescribed such drugs (thus lowering plan spending).[122] That outcome, in turn, can affect health outcomes given studies showing that drug adherence diminishes as price sharing increases.

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[114] Ibid.

[115] Gilead, "Support Path for Sovaldi and Harvoni," at http://www.gilead.com/responsibility/us-patient-access/support%20path%20for%20sovaldi%20and%20harvoni.

[116] Seeking Alpha, "Gilead Sciences' (GILD) CEO John Martin on Q4 2014 Results – Earnings Call Transcript," February 3, 2015, at http://seekingalpha.com/article/2880996-gilead-sciences-gild-ceo-john-martin-on-q4-2014-results-earnings-call-transcript.

[117] Ed Silverman, "Ooh La La: France Negotiates a Steep Discount for Sovaldi From Gilead," *Wall Street Journal*, November 20, 2014, at http://blogs.wsj.com/pharmalot/2014/11/20/ooh-la-la-france-negotiates-a-steep-discount-for-sovaldi/.

[118] FDA, "FDA Approves First Combination Pill to Treat Hepatitis C," press release, October 10, 2014, at http://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm418365.htm.

[119] FDA, "FDA Approves Viekira Pak to Treat Hepatitis C," press release, December 19, 2014, at http://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm427530.htm.

[120] Andrew Pollack, "AbbVie Deal Heralds Changed Landscape for Hepatitis Drugs," *New York Times*, December 22, 2014, at http://www.nytimes.com/2014/12/22/business/pharmacy-deal-heralds-changed-landscape-for-hepatitis-drugs.html and PR Newswire, "Express Scripts and AbbVie Make Hepatitis C Cure Available to Millions of Patients in Need," December 22, 2014, http://www.prnewswire.com/news-releases/express-scripts-and-abbvie-make-hepatitis-c-cure-available-to-millions-of-patients-in-need-300012889.html.

[121] Seeking Alpha, "Gilead Sciences' (GILD) CEO John Martin on Q4 2014 Results – Earnings Call Transcript," February 3, 2015, at http://seekingalpha.com/article/2880996-gilead-sciences-gild-ceo-john-martin-on-q4-2014-results-earnings-call-transcript.

[122] Erin Allday, "Kaiser Reverses Plan to Sharply Boost Patient Cost of HIV Drugs," *SFGate*, February 20, 2015, at

Recent data indicate that a small share of consumers face high cost sharing for drugs. In 2013, more than half of retail prescriptions filled by all insured consumers cost $5 or less and 20% had no co-payment, including many generics.[123] At the same time, about 2% of prescriptions had co-payments or coinsurance of $70 or more and accounted for nearly 30% of consumer out-of-pocket spending for drugs.[124]

Consumer and health care advocacy groups have charged that some health payers use tiered pricing and formulary management to discourage people with expensive conditions from enrolling in their plans. A coalition of civil rights and AIDS groups filed a lawsuit in Florida in July 2014 charging that four insurers offering coverage through the state's ACA exchange had placed all HIV/AIDS drugs, including generics, on their highest-priced tier, effectively discouraging people with AIDS from buying their policies.[125] State officials negotiated an agreement with one of the insurers under which it agreed to alter drug pricing.[126] A recent study of HIV/AIDS drug pricing in 12 state ACA marketplaces found evidence of potentially discriminatory use of price tiers in 12 of 48 studied insurance plans.[127] In February 2015, CMS published rules that set more stringent standards for individual and small-group insurance plan formularies[128] and warned private insurers that placing most or all drugs for treating a specific condition on the highest cost tiers effectively could discriminate against individuals with those conditions.[129]

In addition, legislation has been introduced at the state level to require "transparency" in drug pricing by requiring manufacturers to provide information on the cost of developing a certain drug, and the rationale used to set the price for the product.[130]

In Congress, lawmakers have introduced legislation to allow beneficiaries to seek an exception for drugs placed on Part D specialty price tiers and to amend federal law so that health plans provide no less favorable coverage for oral or self-injectable cancer drugs used on an outpatient

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http://www.sfgate.com/health/article/Kaiser-reverses-plan-to-sharply-boost-patient-6092204.php.

[123] IMS Health, *Medicine and the Shifting Costs of Healthcare*, April 2014.

[124] Ibid. Many health plans cap annual out-of-pocket spending, but the high co-payments still can be a major burden for consumers who must pay thousands of dollars for their drugs in a matter of months or weeks (or if low income even smaller amounts are more burdensome). While participants in private insurance plans may qualify for manufacturer coupons that reduce cost sharing, pharmaceutical companies may be liable under federal anti-kickback statutes if they offer coupons to induce consumers to buy prescription drugs under federal programs, such as Medicare Part D.

[125] "NHeLP and The AIDS Institute File HIV/AIDS Discrimination Complaint against Florida Health Insurers," press release, May 29, 2014, at http://www.theaidsinstitute.org/sites/default/files/attachments/NHeLP_and_The_AIDS_Institute_File_Civil_Rights_Complaint_Against_Florida_Health_Insurers_FINAL.pdf.

[126] Nicholas Nehmas,"Cigna Agrees to Reduce Cost of HIV/AIDS Drugs," *Miami Herald*, November 7, 2014, at http://www.miamiherald.com/news/local/community/miami-dade/article3622324.html.

[127] Douglas Jacobs and Benjamin Sommers "Using Drugs to Discriminate—Adverse Selection in the Insurance Marketplace," *The New England Journal of Medicine*, vol. 372 January 28, 2015, pp. 399-402, at http://www.nejm.org/doi/full/10.1056/NEJMp1411376.

[128] The rules affect individual and fully insured small-group plans regulated by the ACA.

[129] CMS, "Patient Protection and Affordable Care Act; HHS Notice of Benefit and Payment Parameters for 2016," February 20, 2015, at http://www.cms.gov/CCIIO/Resources/Regulations-and-Guidance/.

[130] Policy and Medicine, "Massachusetts Introduces Drug Cost Transparency Bill That Would Cap Prices For Certain Products; North Carolina and Pennsylvania Also Introduce Bills Requiring Drug Cost Reports," April 23, 2015, at http://www.policymed.com/2015/04/massachusetts-introduces-drug-cost-transparency-bill-that-could-cap-prices-for-certain-products-nort.html; and Caitlin Owens, "How States are Working to Control Prescription Drug Costs," *National Journal*, June 1, 2015; and George Lauer, "Drug Price Transparency Bill Shelved in California, Push Continues," *California Healthline*, May 7, 2015, at http://www.californiahealthline.org/insight/2015/drug-price-transparency-bill-shelved-in-california-push-continues.

basis as for traditional chemotherapy administered in a health care setting.[131] At the state level, dozens of legislatures have debated or enacted legislation to prohibit specialty drug tiers in insurance plans and to limit co-payments and other out-of-pocket costs for specialty drugs.[132] For example, a 2014 Maryland law prohibits insurers from imposing a co-payment or coinsurance for a covered drug that exceeds $150 for a 30-day supply.[133]

Although such proposals provide financial relief to individual consumers, they do not address the overall price of drugs or total spending for the products. Rather, the legislation may shift more of the cost of the drugs on to private and public payers. Those payers, in turn, may then pass on the costs to all enrollees in the form of higher monthly premiums or plan deductibles.

Health care analysts have suggested other approaches to managing specialty drugs that might help to improve patient outcomes and manage costs such as value-based purchasing.[134]

Potential Changes in Drug Payment and Pricing

Health care analysts have recommended various ways to give payers more power to negotiate, impose lower prices for specialty drugs, or make the drug purchasing system more cost-effective in terms of linking drug payments to the efficacy of treatments.

On the government side, lawmakers have introduced legislation that would impose Medicaid rebates on drugs purchased by low-income beneficiaries in the Medicare Part D program, including specialty drugs. President Obama's FY2016 budget proposal recommends that the Secretary of HHS be allowed to negotiate directly with manufacturers to set Medicare Part D prices for biologics and other high-cost drugs that are eligible for specialty tier placement. Pharmaceutical manufacturers seeking to participate in Part D would be required to supply HHS with data and information necessary to come to a price agreement on specialty drugs.[135]

Some analysts have suggested new payment models that would allow insurers to spread the cost of expensive drugs over a period of time. One example would be to create a new credit system with a third party providing loans or insurance to payers.[136]

Certain medical professional organizations, led by oncology specialists, and some health insurers have said they will begin using more rigorous economic cost-benefit analyses to determine which

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[131] Legislation from the 114th Congress includes H.R. 1600, S. 1488, and S. 1566.

[132] Examples include Chapter 536, Laws of New York 2010; Vermont Act 171. Chad Brooker, "Waging War on Specialty Pharmaceutical Tiering in Pharmacy Benefit Design," American University, *Health Law and Policy Brief*, vol 7, no. 2 (2013), pp. 25-48, at http://digitalcommons.wcl.american.edu/cgi/viewcontent.cgi?article=1128&context=hlp.

[133] Maryland House Bill 761. The law defines a specialty drug as a prescription drug prescribed for an individual with a complex or chronic medical condition; that costs $600 or more for a 30-day supply; is not typically stocked at retail pharmacies; and requires difficult or unusual process of delivery to the patient or enhanced patient education, management, or support beyond those required for traditional dispensing before or after administration of the drug.

[134] Ha Tu, Divya Samuel, "Limited Options to Manage Specialty Drug Spending," Center for Studying Health System Change, *HSC Research Brief*, no. 22, April 2012, at http://www.hschange.org/CONTENT/1286/?words=specialty%20drugs.

[135] CRS Report R43934, *President's FY2016 Budget: Centers for Medicare & Medicaid Services (CMS) Legislative Proposals*, coordinated by Alison Mitchell, Kirstin B. Blom, and Patricia A. Davis.

[136] IMS, *Harbingers of Change in Healthcare: Implications for the Role and Use of Medicines*, September 2014, p. 11, at http://www.imshealth.com/vgn-ext-templating/v/index.jsp?vgnextoid=af47009812568410VgnVCM10000076192ca2RCRD.

drugs to cover and prescribe.[137] Other health analysts contend that a growing body of data obtained from electronic health records and additional information systems could be used to help rationalize the drug payment system as described below.

At a recent symposium, the IMS Institute for Health Care Informatics, which has the nation's largest database of pharmacy claims and other prescription drug data, suggested tapping a rapidly growing body of online health care data to examine possible new ways to price drugs and other breakthrough therapies.[138] Possible ideas include paying different prices for drugs depending on how they are used and how well they perform.[139] Another option would be to have the federal government provide conditional approval and pricing for a drug that could be adjusted depending on whether it performed as intended. Patient use, outcomes, and treatment costs could be tracked through electronic health record systems.[140] A number of European countries use similar models.[141]

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[137] American Society of Clinical Oncology Value Care Task Force, "Value in Cancer Care," at http://www.asco.org/practice-research/value-cancer-care.

[138] IMS Institute for Health Care Informatics, "How Much Should Drugs Cost? A Big Data Solution," *Forbes* Healthcare Conference, December 3-4, 2014, at http://www.imshealth.com/portal/site/imshealth/menuitem.762a961826aad98f53c753c71ad8c22a/?vgnextoid=a2ce4596eb11a410VgnVCM10000076192ca2RCRD.

[139] Bijal Nitin Patel and Patricia R. Audet, "A Review of Approaches for the Management of Specialty Pharmaceuticals in the United States," *PharmacoEconomics*, August 14, 2014, at http://www.ncbi.nlm.nih.gov/pmc/articles/PMC4209107/.

[140] CRS Report CRS Report RL32858, *Health Information Technology: Promoting Electronic Connectivity in Healthcare*, by C. Stephen Redhead.

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