

REPORT TO CONGRESS

Prescription Drugs: Innovation, Spending, and Patient Access

December 7, 2016

U.S. Department of Health and Human Services

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Table of Contents

	Page
Executive Summary	3
Chapter 1. Introduction	17
Innovation and Prescription Drug Development	
Chapter 2. Time and Cost for New Drug Development	25
Prescription Drug Spending	
Chapter 3. Medicare Part B	39
Chapter 4. Medicare Part D	53
Chapter 5. Medicaid	69
Chapter 6. Veterans Health Administration	83
Chapter 7. Purchasing Arrangements and Utilization Management	88
Patient Access to Prescription Drugs	
Chapter 8. Access	98
Chapter 9. Satisfaction with Care	116
Chapter 10. Outcomes	129
Chapter 11. Summary	143
Appendix	146

EXECUTIVE SUMMARY

Prescription drugs can effectively treat many acute and chronic diseases leading to improvements in quality of life, life expectancy, and overall population health¹. Policy makers must carefully balance the incentives for stimulating innovation of effective medicines and efforts to assure the affordability of those medicines. On one hand, development of new prescription drugs is expensive, uncertain, and slow; requiring the prospect of financial returns to encourage sponsors to continue investing in innovation. Certain new drugs approved by the Food and Drug Administration (FDA) are granted market exclusivity for a period of time to encourage investment. The FDA's orphan drug program also provides incentives to help address this challenge for rare diseases and conditions. On the other hand, if their promise is to be realized, medicines must be affordable and their prices should reflect their value in terms of patient health outcomes.

During most of the time period analyzed in this report (2003-2014), growth in prescription drug spending was moderated by a number of patent expirations and the resulting increased availability and use of generic versions of top selling brand-name drugs. Nonetheless, growth in prescription drug spending has been rising more quickly than overall health care spending in the United States [1, 2]. In recent years, growth in prescription drug spending has accelerated considerably due to increases in the number of newly available costly drugs, including specialty drugs and biologics. In 2015, there were a higher than average numbers of novel drug approvals.[3]. In addition, spending was accelerated by price increases in existing drugs; a relatively low number of patent expirations; increasing insurance coverage; increasing utilization; and population growth and aging [1, 2]. Prescription drug expenditures are projected to continue rising during the coming decade [2], adding to the nation's total health care bill and placing increasing fiscal pressures on commercial, federal, state, and family budgets. Accumulating evidence suggests that patients with high out-of-pocket costs are more likely to delay or forgo treatments for acute and chronic illnesses or not take treatments as prescribed [4-6], jeopardizing any potential benefits of treatment.

Examination of recent trends in prescription drug use and spending can inform efforts to simultaneously promote innovation, improve quality of care, and reduce costs, while maintaining patient access to life-saving therapies. This report describes overall spending for prescription drugs and recent trends in spending using data from literature reviews and separate quantitative analyses for Medicare Part B, Medicare Part D, Medicaid, and the Veterans Health Administration (VHA) programs. Analyses of the National Health Interview Survey (NHIS) data were also conducted to examine patient access to prescription drugs, satisfaction, and outcomes for each of the four programs.

Key Findings

Key findings regarding innovation and drug development, prescription drug spending, and prescription drug access are summarized below. Findings specific to the government health insurance programs Medicare Part B, Medicare Part D, Medicaid, and the VHA are also presented.

¹ The Secretary of Health and Human Services (HHS) has been directed to submit a report on prescription drug innovation, spending, and access to the Committee on Appropriations of the House of Representatives and the Senate. See Appendix for full request.

Innovation and Drug Development

- Between 2006 and 2015, the Food and Drug Administration approved an average of 29 novel drugs a year, with 45 approvals in 2015 alone [3].
- Published estimates of the cost of new drug development range from \$1.2 billion to \$2.6 billion [7-10] and are highly sensitive to assumptions about pre-clinical and clinical development time, cost of capital, the likelihood of reaching approval following the start of clinical testing, and costs of preclinical development and clinical trials conducted among humans.
- Published estimates of the cost of new drug development are also highly sensitive to the incorporation of recent increases in Orphan drug approvals, which tend to have smaller trial sizes, higher success rates, and tax advantages for the sponsor. Between 2010 and 2015, Orphan drugs increased from 29 percent to 47 percent of new drug approvals. Applying updated information yields mean and median development costs for Orphan drugs of \$1.0 billion and \$0.8 billion, respectively, less than half the mean and median estimates of drug development costs of \$2.6 billion and \$1.9 billion published by DiMasi et al. (2016) [7].

Prescription Drug Spending in the United States

According to estimates from the National Health Expenditure Accounts (NHEA), total spending on retail prescription drugs in the United States was \$305 billion in 2014 [2]. ASPE has estimated that spending on non-retail prescription drugs [1] was \$119 billion in 2014, bringing the total figure for prescription drugs to \$424 billion, or 16.3 percent of overall personal health care services. Insurance coverage of prescription drugs has expanded in the United States, with the introduction of the Medicare Part D program in 2006 and with 20 million newly insured adults under the Affordable Care Act [11].

Patterns of increases in overall prescription drug spending growth varied by program

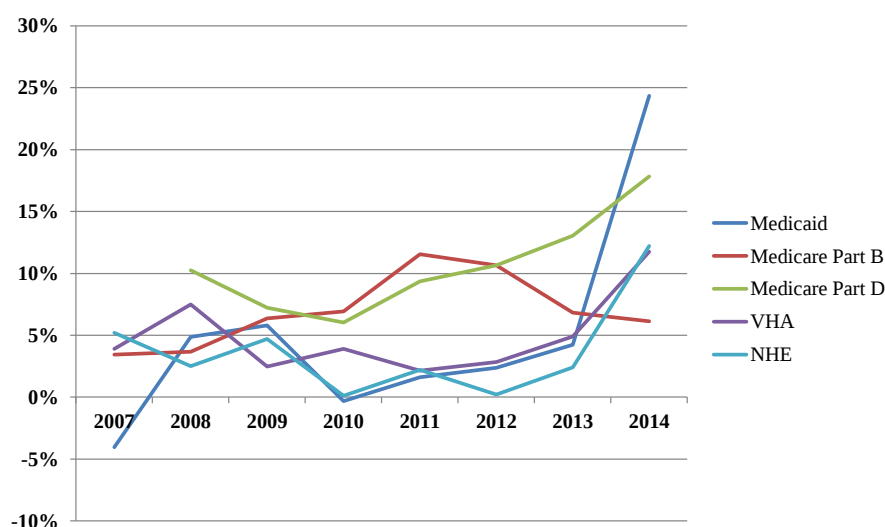
Between 2001 and 2007, estimates from the NHEA showed retail prescription drug spending growing by about 10 percent annually. Growth slowed to about 2 percent annually between 2008 and 2013 and then increased to 12 percent in 2014 [2].

- Medicare Part B prescription drug spending increased from \$10.1 billion in 2006 to \$17.2 billion in 2014. The average annual growth rate in spending was 4.5 percent annually between 2006 and 2009, and then accelerated to 8.4 percent annually between 2009 and 2014. Medicare Part B prescription drug spending as a percentage of total Part B spending remained relatively modest and stable throughout this period, averaging about 6.2 percent.
- Medicare Part D gross drug costs (total payment to pharmacies by Part D plans and beneficiaries) nearly doubled from \$61.9 billion in 2007 to \$121.0 billion in 2014. Between 2007 and 2012, annual spending increases were 7.7 percent, but accelerated to 16.3 percent annually between 2012 and 2014.
- Medicaid prescription drug spending fell sharply after Medicare Part D assumed costs for dual eligible enrollees in 2006. Between 2006 and 2013, Medicaid prescription drug spending net of rebates rose 15.0 percent, to \$22.0 billion. Medicaid spending growth accelerated by 24.3

percent to \$27.3 billion in 2014 due to the expansion of Medicaid coverage as well as uptake of newly available therapies to treat Hepatitis C. Spending per enrollee grew 13.5 percent between 2013 and 2014.

- VHA total spending on prescription drugs rose from \$3.2 billion in 2007 to \$3.6 billion in 2014, with an average annual growth rate of 1.5 percent. Growth in the last year of that period, however, was 11.7 percent.

Annual Percentage Change in Prescription Drug Spending



Medicaid: Net spending based on estimates produced by the Office of the Actuary at the Centers for Medicare & Medicaid Services (CMS). Medicare Part B: Analysis of carrier, durable medical, and outpatient claims for Part B drugs paid under ASP + 6%. Medicare Part D: Analyses of Medicare Part D prescription drug event data to calculate gross spending. National Health Expenditures (NHE): produced by the Office of the Actuary at the CMS. NHE estimates represent net spending.

Spending on specialty drugs and biologics increased rapidly

Overall, specialty drugs represented only 1 percent of retail prescriptions in the United States, but 31.8 percent of prescription drug spending in 2014 [12]. Spending on specialty drugs increased at an average annual rate of 11.2 percent between 2010 and 2014, with annual growth of 22.9 percent between 2013 and 2014 [1]. Spending on specialty drugs is rising more rapidly than spending on other drugs, although estimates vary depending on the way “specialty” is defined. Biologics are an important component of specialty drugs and account for a small share of prescription drug utilization, but a large share of spending. U.S. spending on biologics increased 10 percent annually from 2005 to 2012 [13, 14].

- Increases in spending in the Medicare Part B program have been driven by increases in biologics. Spending on biologics between 2006 and 2014 grew by 13.3 percent annually, whereas spending

on small molecule drugs grew by 0.7 percent annually during the same period. In 2014, biologics accounted for 63 percent of prescription drug spending in Part B.

- Between 2007 and 2014, spending on specialty tier eligible drugs increased from \$6.1 billion to \$35.9 billion in Medicare Part D. Spending increased faster than did utilization: the average annual growth rates for spending was 29 percent as compared with 15 percent for utilization, implying that price increases are responsible for half the growth in spending.
- In the Medicaid program in 2014, biologics accounted for only 3 percent of utilization, but 15.7 percent of gross spending (\$7.3 billion).

Spending by therapeutic class reflects underlying differences in eligibility and prescription drug coverage across programs

Eligibility across government insurance programs varies by age, disability, income, military service, and medical need. Underlying differences in eligibility for each program also affect the prevalence of chronic conditions and medical needs for those with coverage through the Medicare Parts B and D, Medicaid and the VHA programs. Program coverage of prescription drugs also varies. In general, Medicare Part D provides coverage for oral prescription drugs and Medicare Part B covers drugs that are administered by injection or infusion in physician's offices or hospital outpatient departments. Medicaid provides coverage for oral and implanted, infused, inhaled, injected, and instilled drugs. The VHA prescription benefit provides coverage for all Food and Drug Administration (FDA) approved drugs, over the counter medications, and medical supplies.

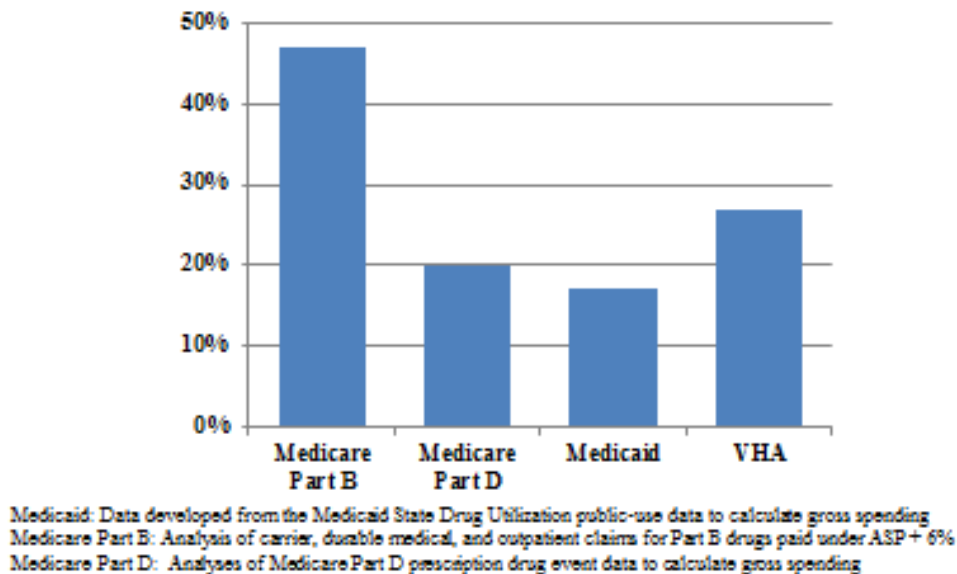
- In 2014, spending in Medicare Part B was dominated by cancer drugs (45 percent). Spending for drugs that treat rheumatoid arthritis (8.7 percent), intravenous immunoglobulin (IVIG) preparations (5.2 percent), and osteoporosis (4.3 percent) was also common. No other therapeutic class represented more than 3 percent of spending. These four classes were also the dominant therapeutic classes for spending in 2006.
- Spending in Medicare Part D was highest for antidiabetics, antipsychotics and antimanics, and antineoplastics and adjunctive therapies in 2014. In 2007, the top three therapeutic classes were antipsychotics and antimanics, antidiabetics, and anticonvulsants. Antidepressants and antidiabetics had the highest utilization in all years.
- Psychotherapeutic drugs have consistently been the largest therapeutic class in spending for the Medicaid program. Gastrointestinal drugs are the largest therapeutic class in units dispensed. Led by Sovaldi, gross Medicaid spending on antivirals rose from \$59.0 million in 2012 to \$1.9 billion in 2014, an increase of 3,092.1 percent.
- In 2014, VHA drug spending was highest for antimicrobials, which include the hepatitis C drugs, and central nervous system medications. Those two categories accounted for about 36 percent of 2014 drug spending. Spending in 2007 was highest for central nervous system medications and cardiovascular medications, which accounted for 41 percent of 2007 drug spending. The hepatitis C drugs were largely responsible for the antimicrobial medications moving into the highest cost drug class for VHA in 2014.

Small numbers of drugs represent disproportionately high spending, although spending concentration varies by program

Analyses of recent trends in prescription drug spending across programs included evaluation of the top 10 drugs in terms of spending. The top 10 drugs vary across programs, reflecting the health care needs of the population served by each program.

- A relatively small number of Medicare Part B drugs account for a significant share of spending. The top 10 drugs account for 47 percent of total spending. Concentrated spending for a relatively small number of drugs has been consistent for the past decade, with Rituximab ranked either first or second from 2006 to 2014 and Ranibizumab entering the top 10 list in 2008 and ranking either first or second from 2010 to 2014.
- In Medicare Part D, the top 10 drugs by gross spending accounted for about 20 percent of total gross drug cost in 2014. This proportion has been consistent since 2007. However, concentrated spending for a relatively small number of drugs may increase with the entry of new expensive drugs. For example, the hepatitis C drug Sovaldi entered the market late in 2013 and moved into the top 10 list with a relatively small number of claims and users.
- In the Medicaid program, the top 10 small-molecule branded drugs by gross spending accounted for about 17 percent of total gross drug spending in 2014. Recent market entrant Sovaldi ranked second and Truvada, approved in 2012, ranked fourth.
- The top 10 drugs by spending in the VHA accounted for 27 percent of all prescription drug spending in 2014, an increase of 8 percent since 2007. Hepatitis C drugs represented two of the top 10 prescription drugs by total spending in 2014.

Percentage of Prescription Drug Spending for Top Ten Drugs in 2014, by Program

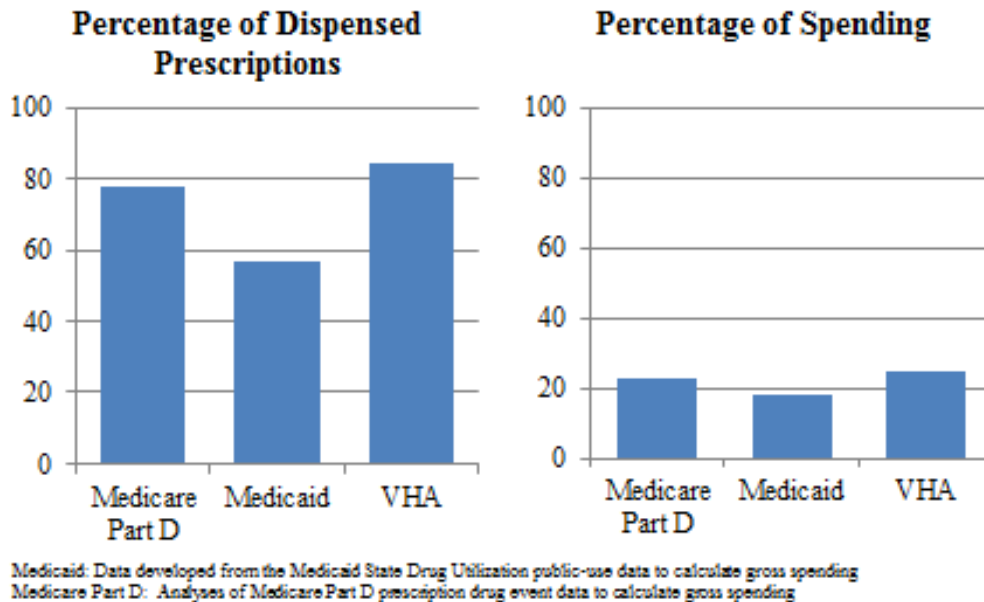


Generic drugs account for the majority of dispensed prescriptions, but a relatively small percentage of spending

The use of generic drugs in the United States increased by an average of 4.2 percent annually between 2006 and 2014. However, within this period, growth has slowed, with increases of more than 6 percent annually between 2006 and 2008 compared to roughly 3 percent annual increases from 2011 to 2014. As of 2014, generic drugs accounted for 88.0 percent of dispensed prescriptions, but only 28.0 percent of drug spending, while in 2006 they made up 63 percent of prescriptions and 20.0 percent of spending [12].

- In the Medicare Part D program, generics increased from 52.8 percent of filled prescriptions in 2007 to 77.5 percent in 2014. As a percentage of gross spending, generics increased from 18.5 percent to 23.0 percent over the same period.
- In 2014 in the Medicaid program, generic drugs represented the majority of drugs used, almost 57 percent of units. However, generics represented only 18.3 percent of gross spending and 32.4 percent of net spending.
- In 2014 in the VHA, generic drugs represented approximately 84 percent of the outpatient 30-day equivalent prescription fills and 25 percent of the prescription drug spending.

Utilization and Spending for Generic Drugs in 2014, by Program



Some government programs use purchasing arrangements and utilization management strategies available in the private sector to promote value and control cost

A number of purchasing arrangements and utilization management strategies are used by commercial insurers and some government programs to promote value and control cost, including negotiation with manufacturers and pharmacies, rebates, use of preferred drug lists or formularies with tiers, prior authorization requirements, step therapy, prescription quantity limits, value-based purchasing and payment, and risk-sharing or outcomes-based arrangements [15].

- Medicare Part B does not currently use any of the purchasing arrangements available in the private sector.
- The Medicare Part D drug benefit is administered through private prescription drug plans, which each separately design and manage benefits and pay claims. The private plans use purchasing arrangements and utilization management, including negotiation of prices with manufacturers and pharmacies, formularies, step therapy, quantity limitations, and prior authorization. All formularies must include “all (with specified exceptions)”² drugs in the immunosuppressant, antidepressant, antipsychotic, anticonvulsant, antiretroviral, and antineoplastic classes to ensure patient access to these 6 protected classes of drugs. CMS is prohibited from interfering in drug price negotiations between prescription drug plans and manufacturers. Prescription drug plans

² The current exceptions are that the formulary does not have to include all therapeutic equivalents (i.e., generics) and can use safety edits to limit quantities (see 42 CFR 423.120(b)(2)(vi)).

are required to cover FDA-approved drugs for medically accepted indications when medically necessary.

- State Medicaid programs and managed care plans use preferred drug lists, prior authorization, and drug utilization review to help ensure value in purchasing of prescription drugs and management of their use. About half of Medicaid gross spending on prescription drugs is returned to the federal government and the states in the form of manufacturer rebates. The Medicaid program cannot deny access to drugs approved by the FDA and manufactured by companies participating in the rebate program when they are prescribed for medically accepted indications.
- VHA purchases prescription drugs directly through a pharmaceutical prime vendor and receives a significant statutory discount on covered drugs (drugs with a new drug application or a biologics license application). Purchasing arrangements include direct negotiation with manufacturers, volume discounts, and rebates. The VHA also uses a wide variety of utilization management strategies, including prior authorization, preference for generic drug options when available, and national criteria-for-use documents with a blend of clinical criteria, step therapy, and quantity limits. VHA also uses value-based approaches that include outcomes-based risk-sharing agreements. Finally, VHA influences prescribers to use cost-effective drugs through academic detailing and national monitoring of cost saving opportunities.

Prescription Drug Purchasing Arrangements, Utilization Management, and Value-Based Approaches in the United States

		Commercial Health Plans and Pharmaceutical Benefits Managers	Veterans Health Administration	Private Prescription Drug Plans on Behalf of Medicare Part D	Medicare Part B	State Medicaid Programs and Managed Care Plans
Purchasing Arrangements	Direct negotiation/volume discounts	X	X	X		X
	Rebates	X	X	X ¹		X ²
Utilization Management and Review	Formulary design	X	X	X		X
	Utilization and management review	X	X	X		X
	Prior authorization	X	X	X		X
	Step therapy	X	X	X		X
	Quantity limits	X	X	X		X
Value-Based Approaches	Indication-based pricing	X				
	Value-based purchasing	X				
	Performance-based risk-sharing/Outcomes based arrangements	X				
	Reference pricing ³				⁴	
	Value-based patient cost-sharing	X	X			

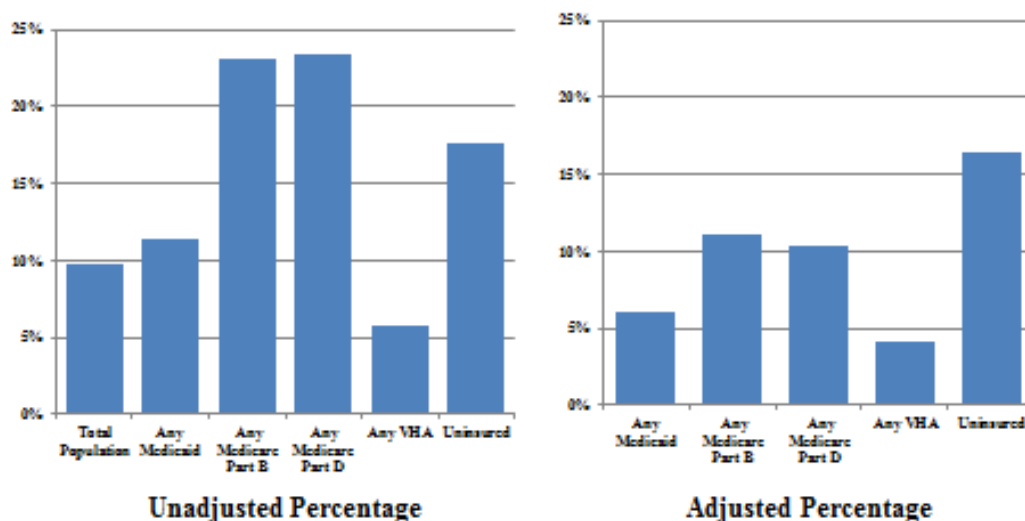
¹ Rebates are negotiated by commercial insurers and pharmacy benefits managers (PBMs) for retail pharmacy drugs; and by providers for drugs administered in offices or hospital outpatient settings. CMS is prohibited from interfering in drug price negotiations between prescription drug plans and manufacturers. However, the program and its beneficiaries benefit from rebates negotiated by the private entities through lower payments and premiums. For example, Medicare Part B reimburses providers using the Average Sales Price (ASP). The ASP is calculated from sales price data sent by manufacturers to the Centers for Medicare & Medicaid Services (CMS), which are required to be net of all rebates and other price concessions. ² About half of Medicaid gross spending on prescription drugs is returned to the federal government and the states in the form of manufacturer rebates. ³ Commercial insurers do not formally use reference pricing. They do, however, frequently employ tiered cost sharing arrangement which can have incentives similar to reference pricing for patients and providers in situations where generics or other therapeutic alternatives are available in different cost-sharing tiers. ⁴ Branded drugs and their generic counterparts are grouped together when calculating the ASP. When generics are available, the ASP for that active substance is a form of reference pricing. However, active substances within pharmacologic class are not grouped together in calculating a broader ASP for the pharmacologic class, as is done in other countries. In addition, pharmacologic classes are not grouped within therapeutic class.

Patient access to prescription drugs

Having health insurance is a strong predictor of patient access to care [16], and recent expansions in health insurance coverage offer the potential for improved population health. However, the out-of-pocket costs associated with some prescription drugs may result in financial hardship for patients and their families, even if they have health insurance [17-19]. Access to prescription drugs varies substantially by age in the United States [18]. Adults ages 18-64 years are twice as likely as older adults ages 65 years and older to report skipping doses, taking less medication, or delaying filling prescription drug medications because of cost in the past 12 months (9.7 percent vs 4.7 percent), despite lower prevalence of chronic conditions and medical need. Data from the National Health Interview Survey were pooled for years 2011-2014 to allow stable estimates of patient access to prescription drugs for each of the four government insurance programs.

- Among adults ages 18-64 years, the prevalence of not taking drugs as prescribed because of cost was 9.7 percent in the United States overall and varied by insurance program: Medicare Part B (23.1 percent) or Part D (23.3 percent), Medicaid (11.5 percent), VHA (5.8 percent), private insurance (6.7 percent) and for the uninsured (17.6 percent). Statistical adjustment for characteristics that vary across programs, such as comorbidity, had a significant effect on estimates of prescription drug access. Adjusted estimates of not taking drugs as prescribed because of cost were closer to that of the general population for Medicare Part D (10.3 percent) or Part B (11.0 percent), Medicaid (6.0 percent), VHA (4.1 percent), and private insurance (7.8 percent), but remained high for the uninsured (16.4 percent).

Problems with Access to Prescription Medication Past 12 Months by Program, Ages 18-64 Years



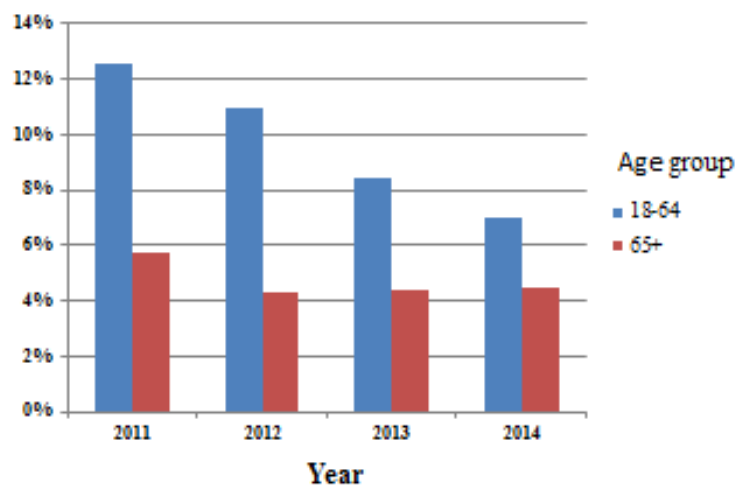
NOTE: Estimates are from National Health Interview Survey (NHIS) data 2011-2014.
Insurance categories are not mutually exclusive.

- Among adults aged 65 years and older, prevalence of not taking drugs as prescribed because of cost was 4.7 percent in the United States overall and was relatively similar across insurance program: Medicare Part D (5.4 percent) or Part B (4.8 percent), Medicaid (6.1 percent), and VHA (2.9 percent). Statistical adjustment for differences in individual characteristics that vary between programs had little effect for those with Medicare Part D, Part B, or VHA coverage. The proportion not taking medications as prescribed because of cost declined for Medicaid enrollees from 6.1 percent to 3.2 percent following adjustment for characteristics, such as comorbidity, that vary between Medicaid enrollees and the United States population aged 65 years and older.
- Patient assistance programs, individual drug couponing, and savings card programs are increasingly common as a means to reduce patient out-of-pocket cost and increase access to prescription drugs [20, 21]. However, due to the federal anti-kickback statute, Medicare, Medicaid, and VHA beneficiaries are not eligible to participate in programs sponsored by pharmaceutical companies or use coupons or savings card programs that apply only to a specific drug.

Access to prescription drugs has improved since 2011

- The percentage of adults not taking drugs as prescribed because of cost declined between 2011 and 2014, from 12.5 percent to 7.0 percent of adults ages 18-64 and from 5.7 percent to 4.4 percent of adults ages 65 years and older. Improvements in access to prescription drugs during this period likely reflect increased availability of health insurance coverage for the population ages 18-64 years and efforts to close the Medicare Part D coverage gap in the population ages 65 years and older.

Percentage of Adults Skipping Doses, Taking Less Medicine or Delaying Filling Prescriptions, 2011 to 2014



NOTE: Estimates are from National Health Interview Survey (NHIS) 2011-2014

Published research shows that patient out-of-pocket spending for prescription drugs has declined for the uninsured gaining insurance coverage and for the insured taking non-specialty medications

- Between 2013 and 2014, uninsured individuals who gained private insurance filled 28 percent more prescriptions and had 29 percent lower out-of-pocket spending per prescription. Uninsured individuals who gained Medicaid coverage filled 79 percent more prescriptions and had 58 percent lower out-of-pocket spending per prescription [22].
- Between 2003 and 2014, median monthly out-of-pocket spending for privately insured users of non-specialty drugs has declined, even though patient out-of-pocket spending for specialty drugs has increased [23]. Because a relatively small proportion of individuals use specialty drugs, these findings are consistent with overall improvements in patient access to prescription drugs, despite increasing prescription drug spending.

Overall satisfaction with health care was highest for adults with access to prescription drugs

Adults in the United States report high levels of satisfaction with health care received in the past 12 months.

- More than 80 percent of adults ages 18-64 years with any Medicare Part B or Part D, any Medicaid, or any VHA coverage reported satisfaction with health care received in the past 12 months. Among the uninsured, only 44.0 percent reported being satisfied and 9.2 percent reported being dissatisfied, but nearly half (46.8 percent) reported no healthcare utilization in the past 12 months. After adjustment for sociodemographic factors that vary across programs, the percentages of adults expressing satisfaction with health care increased slightly for all groups, except for those with coverage through the VHA.
- Satisfaction varied little by insurance program for the population ages 65 years and older, with at least 89 percent of those with any Medicare Part B or Part D, any Medicaid, or any VHA coverage reporting being satisfied with health care. Adjustment for sociodemographic factors that vary across programs had very little effect on these percentages.
- Satisfaction with health care was highest for individuals with access to prescription drugs and lowest for those who reported not taking medication as prescribed due to cost. This finding was consistent across age groups and by insurance program.

Self-reported health outcomes were better for individuals with access to prescription drugs

Large majorities of adults in the United States report good, very good, or excellent health, regardless of age group.

- Almost 90 percent of adults ages 18-64 years reported good, very good, or excellent health, and having good to excellent self-reported health varied by insurance program: Medicare Part B (37.5 percent) or Part D (36.0 percent), Medicaid (69.5 percent), VHA (67.8 percent), and uninsured (86.9 percent). After adjustment for individual characteristics that vary by program, self-reported health was more similar across

programs, with at least 70 percent reporting good, very good, or excellent health: Medicare Part B (70.0 percent) or Part D (71.3 percent), Medicaid (82.6 percent), VHA (82.4 percent), and uninsured (88.3 percent).

- Overall, 78.1 percent of adults ages 65 years and older reported good, very good or excellent health, although percentages varied by insurance program: Medicare Part B (77.4 percent) or Part D (76.0 percent), Medicaid (50.7 percent), and VHA (69.1 percent). After adjustment for characteristics that vary across programs, distributions of self-reported health were more similar across insurance programs, with at least two-thirds (66.5 percent) reporting good, very good, or excellent health.
- Individuals who reported their health was very good or excellent were more likely to report access to prescription drugs than individuals who reported their health was fair or poor. This finding was consistent across age groups and by insurance program.

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CHAPTER 1: INTRODUCTION

The Secretary of Health and Human Services (HHS) has been directed to submit a prescription drug report to the Committee on Appropriations of the House of Representatives and the Senate (see Appendix). In response, the Assistant Secretary for Planning and Evaluation (ASPE) developed this report containing data and analyses related to innovation and prescription drug development, prescription drug spending, and patient access to prescription drugs. The section on innovation provides an overview of new prescription drug approvals and the clinical trials process and an evaluation of the current cost and length of time necessary to bring new drugs to market. The section on prescription drug spending contains data and analysis concerning trends in annual spending since 2003 and spending for the most frequently prescribed and highest cost drugs for government health insurance programs, including: 1) the Medicare program under part B of title XVIII of the Social Security Act, 2) the Medicare prescription drug program under part D of title XVIII of the Social Security Act, 3) the Medicaid program under title XIX of the Social Security Act, and 4) the Department of Veterans Affairs. For each program, strategies to control costs since 2001 and the current use of formularies and utilization management are reviewed. Finally, in the access section, data and analyses pertaining to patient access to drugs, satisfaction with care, and outcomes are presented for each of the four government health insurance programs.

Innovation, Prescription Drug Development, and Affordability

Prescription drugs can effectively treat many acute and chronic diseases leading to improvements in quality of life, life expectancy, and overall population health. Innovation through new prescription drug development is ongoing, with 45 novel drug approvals in 2015 [1]. Of the novel drug approvals in 2015, 16 were “first in class” drugs, which often have a new mechanism for treating an indication. In addition, the Food and Drug Administration (FDA) designated 10 of the new drugs as “breakthrough” therapies, which is done when a drug is intended to treat a serious condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement on a clinically significant endpoint over available therapies. There are also indications of continued innovation as biotech and pharmaceutical companies continue to increase their investments in research and development (R&D) [2] and success rates for new drugs in development rise [3]. However, development of new prescription drugs is expensive, uncertain, and slow. The high costs of new drug development require the prospect of financial returns to encourage sponsors to continue investing in innovation. To encourage investment, sponsors of certain drugs approved by the Food and Drug Administration (FDA) are granted exclusive rights to market their drug for a period of time. The Orphan Drug Act also provides incentives, including grants, tax credits, and an additional period of market exclusivity to encourage investment in treatments for rare diseases or conditions. Examining the R&D costs and time to develop a new drug can inform understanding of trends in prescription drug list prices and overall spending.

Because new medicines can improve the health of individuals and the population more broadly, the incentives for innovation described above are important. At the same time, policy makers must balance these incentives with assuring that the new medicines are affordable and reflect

their value in terms of improving patient health outcomes. Different countries rely on different strategies to achieve this balance by either: relying on the interaction of private market participants; relying on public price controls or negotiation; or using a combination of both. To the extent that the U.S. considers strategies for assuring both innovation and affordability, new information on pharmaceutical trends can inform the debate.

Prescription Drug Spending in the United States

Prescription drug spending has increased over the past decade in the United States, with accelerated growth in recent years [4, 5]. One of the most commonly cited sources of information about health care spending is the National Health Expenditure Accounts (NHEA) [6]. The NHEA provide estimates of retail prescription drug spending at outlets that directly serve patients. Non-retail prescription drug spending, or spending by medical providers for drugs they provide directly to patients, however, is classified under the spending category corresponding to the provider purchasing the drugs, such as hospital or physician services. Estimates that omit the non-retail portion of drug spending present an incomplete picture of total prescription drug spending. According to estimates from the NHEA, total spending on retail prescription drugs in the United States was \$305 billion in 2014 [6]. ASPE has estimated that spending on non-retail prescription drugs [5] was \$119 billion in 2014, bringing the total figure for prescription drugs to \$424 billion in 2014, or 16.3 percent of overall personal health care services [5].

Between 2001 and 2007, estimates from the NHEA showed retail prescription drug spending growing by about 10 percent annually. The period 2008 to 2012 had unusually slow growth in drug spending—about 2 percent per year. The slower growth in retail spending on prescription drugs during this period was associated with a number of patent expirations for brand-name drugs and the resulting increased availability and use of generic versions of widely used brand-name drugs. More recently, however, growth in retail prescription drug spending—12.6 percent in 2014—has been rising more quickly than overall health care spending in the United States [6]. This recent acceleration is due to a number of factors. Insurance coverage of prescription drugs has expanded in the United States, with the introduction of Medicare Part D program in 2006 and access increased with the addition of 20 million newly insured adults under the Affordable Care Act [7]. The population in the United States is both growing and aging as well, and prescription drug use is highest in the elderly [8]. In addition, increases in availability and use of expensive specialty drugs and biologics, price increases in existing drugs, and a relatively low number of patent expirations, reducing the rate of new generic drug entry, also played a critical role in the increased growth in spending [5, 6].

Specialty drugs and biologics

Specialty drugs are typically used to treat chronic, complex conditions that require intensive monitoring or dosing adjustments, patient training and compliance assistance, and specialized handling or administration [4]. They may also be complex to manufacture. Importantly, specialty drugs also tend to be costly and are sometimes defined by high cost alone.

Overall, specialty drugs represented only 1 percent of prescriptions, but 31.8 percent of prescription drug spending in 2014 [4]. Spending on specialty drugs increased at an average

annual rate of 11.2 percent between 2010 and 2014, with annual growth of 22.9 percent between 2013 and 2014 [5]. Spending on specialty drugs is rising more rapidly than spending on other drugs, although estimates vary depending on the definition of “specialty”. Biologics are a type of specialty drugs and account for a small share of prescription drug utilization but a large share of spending. U.S. spending on biologics increased 10 percent annually from 2005 to 2012 [9, 10].

Generics

Generic drugs are copies of brand-name small molecule drugs. The brand-name and corresponding generic drugs have the same active ingredient and are the same in dosage, form, safety, strength, route of administration, quality, performance characteristics and intended use. They may also be bioequivalent. In 1984, the Drug Price Competition and Patent Term Restoration Act (“Hatch-Waxman”) eliminated the requirement that generic drugs complete the same clinical trial process as their brand drug counterpart with the goal of making generic drugs more available. The use of generic drugs in the United States has increased by an average of 4.2 percent annually between 2006 and 2014. Growth slowed over the latter part of this period, with increases of more than 6 percent annually between 2006 and 2008 compared to roughly 3 percent annually from 2011 to 2014. As of 2014, generic drugs accounted for 88.0 percent of dispensed prescriptions, but only 28.0 percent of drug spending, while in 2006 they made up 63.0 percent of prescriptions and 20.0 percent of spending [4].

Prescription Drug Spending in Government Health Insurance Programs

Trends in prescription drug utilization and spending vary by Medicare Part B, Medicare Part D, Medicaid, and the Veterans Health Administration (VHA) government insurance programs. Differences in utilization and spending reflect underlying variation in eligibility for each program, including age, disability, income, military service, and medical need. The Medicare Program provides health insurance coverage for individuals aged 65 years and older as well as certain younger individuals with disabilities or with End-Stage Renal Disease. The Medicaid program is the joint Federal-State program that provides coverage for individuals and families with low incomes; some individuals with incomes above these limits may also qualify due to high medical expenses. The VHA provides health insurance coverage for eligible individuals after discharge from active military service based on medical need and status-based criteria.

In addition to populations served, these programs and different parts of these programs also vary in the coverage offered for prescription drugs. Medicare Part D and Medicare Part B provide coverage for different types of prescription drugs: in general, Medicare Part D provides coverage for oral prescription drugs and Medicare Part B covers drugs that are administered by injection or infusion in physician’s offices or hospital outpatient departments. Medicaid provides coverage for oral and implanted, infused, inhaled, injected and instilled drugs. The VHA prescription benefit provides coverage for all Food and Drug Administration (FDA) approved drugs, over-the-counter medications, and medical supplies.

Prescription drug expenditures are projected to continue rising during the coming decade [5, 6], placing increasing fiscal pressures on commercial, federal, and state budgets. Increases in prescription drug spending are not expected to be uniform across government program, however, in part due to differences in eligibility and coverage across program. Another important factor underlying differential projected increases in prescription drug spending is variation in use of

purchasing arrangements, utilization management strategies, and value-based approaches by the different government programs.

Government Insurance Program Efforts to Control Costs and Promote Value

A number of purchasing arrangements and utilization management strategies are used by commercial insurers and some government programs to promote value and control cost, including negotiation with manufacturers and pharmacies, rebates, use of preferred drug lists or formularies with tiers, prior authorization requirements, step therapy, prescription quantity limits, value-based purchasing and payment, and risk-sharing or outcomes-based arrangements. Current use of these strategies varies substantially between Medicare Part B, Medicare Part D, Medicaid, and VHA programs. In addition to controlling costs, some of these strategies may restrict or limit patient access to specific prescription medications. For example, many specialty drugs do not have lower-cost alternative treatments, and patients may be faced with the highest levels of cost-sharing for these medications, potentially leading to cost-related barriers or problems with access to prescription drugs. Thus, although these utilization management strategies, formularies, and value-based benefit designs are important tools for controlling costs and promoting value, they may also have adverse effects on patient access to prescription medication.

Patient Access to Prescription Drugs

Having and maintaining health insurance is a strong predictor of patient access to care, and the recent expansion in the number of people with health insurance in the United States offers the potential for improved population health. However, the costs associated with some prescription drug therapies can place a financial strain on patients who might face high out-of-pocket costs even if they have health insurance [11-13]. Accumulating evidence suggests that patients with higher levels of cost-sharing are more likely to delay or forgo prescription medications for acute and chronic illness or not take medication as prescribed [14-16], jeopardizing any potential benefits of treatment. Cost-related medication non-adherence is also associated with higher rates of emergency room visits [17], potentially avoidable hospitalizations [18,19], and poorer patient outcomes [18, 20].

A recent nationally-representative poll of more than 1,200 adults found that the affordability of prescription drugs tops the public's list of priorities for the President and Congress. Identified priorities included "making sure that high-cost drugs are affordable to those who need them" and "government action to lower prescription drug prices". Notably, 77 percent of U.S. adults believe that "making sure that high-cost drugs for chronic conditions, such as HIV, hepatitis, mental illness and cancer, are affordable to those who need them" is a top priority [21].

Contents of this Report

ASPE developed this report in consultation with experts from the Department of Health and Human Services, including Centers for Medicare & Medicaid Services and Department of Veteran's Affairs. The report is divided into three sections: 1) prescription drug innovation, 2) prescription drug spending, and 3) patient access to prescription drugs. In the first section, Chapter 2 discusses prescription drug innovation and provides an analysis of the cost and length

of time to bring a new drug to market. It describes key features of the drug development process that affect timing and cost. Analyses conducted specifically for this report use estimates from the Food and Drug Administration (FDA) and the published literature to evaluate the derivation of published estimates of the time and costs associated with new drug development.

In the second section, multiple aspects of prescription drug spending are described for Medicare Part B in Chapter 3, Medicare Part D in Chapter 4, Medicaid in Chapter 5, and the Veterans Health Administration in Chapter 6. In each of these chapters, the program is described and historical efforts to control costs are detailed. Data and analyses are presented addressing trends in annual spending, with detailed evaluation of trends in spending for specialty drugs and biologics, top 10 drugs, therapeutic classes, and generics. Factors associated with trends and efforts to improve access and value are also noted. An analysis of current use of purchasing arrangements, utilization management, and value-based approaches is reviewed for each the government health insurance programs in Chapter 7.

In the third section, data and analyses pertaining to patient access to prescription drugs, satisfaction with care, and outcomes are presented for each of the four government health insurance programs, Medicare Part B, Medicare Part D, Medicaid, and the Veterans Health Administration. Following reviews of the published literature, results of analyses of the National Health Interview Survey data conducted specifically for this report are reported. Chapter 8 presents data on the prevalence of chronic conditions and medical need by broad age group (18-64 years and 65 years and older), providing background for the evaluation and analysis of access, satisfaction, and outcomes. This chapter also reviews the published literature about access to care, access to prescription drugs and describes components of patient cost-sharing for each program. Chapters 9 and 10 discuss satisfaction with health care and outcomes by program. All three chapters provide estimates of access, satisfaction, and outcomes, respectively, for each of the four programs using standard measures of access to prescription drugs and the most recently available nationally representative data.

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CHAPTER 2: COST AND LENGTH OF TIME TO BRING A NEW DRUG TO MARKET

This chapter discusses prescription drug innovation and the cost and length of time to bring new drugs to market. It provides an overview of the clinical trials process and describes key features of the drug development process that affect timing and cost using estimates from the Food and Drug Administration (FDA) and the published literature. Analyses conducted specifically for this report used published data to evaluate the effects of key assumptions and estimates on the time and costs associated with new drug development. Limitations of existing estimates and methodologies are also noted.

Key Findings

- Between 2006 and 2015, the Food and Drug Administration approved an average of 29 novel drugs a year, with 45 approvals in 2015 alone³.
 - Published estimates of the cost of new drug development range from \$1.2 to \$2.6 billion [1-4] and are highly sensitive to assumptions about pre-clinical and clinical development time, cost of capital, the likelihood of reaching approval following the start of clinical testing, and costs of preclinical development and clinical trials conducted among humans.
 - Published estimates of the cost of new drug development are also highly sensitive to the incorporation of recent increases in Orphan drug approvals, which tend to have smaller trial sizes, higher success rates, and tax advantages for the sponsor. Between 2010 and 2015, Orphan drugs increased from 29 percent of newly approved drugs to 47 percent of approvals. Applying updated information yields mean and median drug development costs of \$1.0 billion and \$0.8 billion for Orphan drugs, respectively, which are less than half the recently published mean and median estimates of \$2.6 billion and \$1.9 billion from DiMasi et al. (2016) [1].
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Background

Effective new drugs offer tremendous societal value by extending life and improving quality of life. Effective drug treatment has transformed HIV/AIDS from a terminal to a chronic infectious disease. The availability of new childhood vaccines have led to the eradication of smallpox, and once common childhood killers, like measles and mumps, are now rare. Continuing innovation in drug development has the potential to deliver ongoing improvements in societal health. However, the development of new drugs is expensive, uncertain, and slow. The high costs of new drug development require the prospect of financial returns to encourage sponsors to continue investing in innovation. To encourage investment in new drugs, sponsors of certain drugs approved by the Food and Drug Administration (FDA) are granted exclusive rights to market their drug for a period of time. The Orphan Drug Act also provides incentives, including grants, tax credits, and an additional period of market exclusivity to encourage investment in treatments for Orphan drugs. A drug qualifies for Orphan status if it is intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the

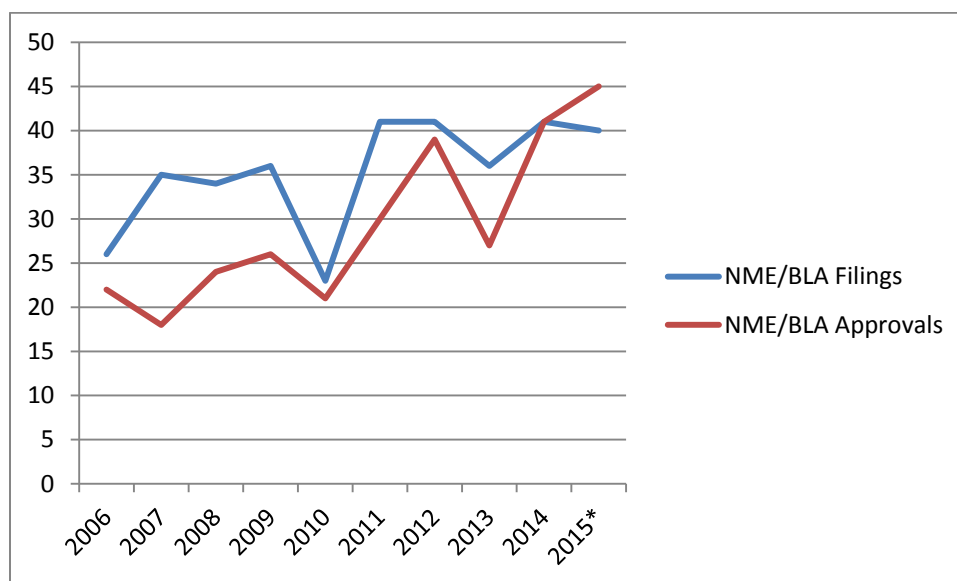
³ Novel drugs are classified as new molecular entities (“NMEs”) by the FDA. NMEs either contain active moieties that have not been approved by FDA previously, or are characterized as NMEs for administrative purposes. For example, CDER classifies biological products submitted in an application under section 351(a) of the Public Health Service Act as NMEs for purposes of FDA review.
(<http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugInnovation/ucm20025676.htm>).

U.S., or that affect more than 200,000 persons but are not expected to recover the costs of developing and marketing a treatment drug. .

Recent Drug Approvals

From 2006 to 2015, the FDA’s Center for Drug Evaluation and Research (CDER) approved an average of 29 novel drugs annually⁴. There were 45 novel drug approvals in 2015, 16 of which represent “first in class” drugs, which often have a new mechanism of action to treat the indication. The FDA designated 10 of the new drugs approved in 2015 “breakthrough” therapies, is done when a drug is intended to treat a serious condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement on a clinically significant endpoint over available therapies. Breakthrough therapies receive more intensive FDA guidance on their development program, and may receive other actions to expedite review. Examples include Tagrisso and Alecenza, both treatments for non-small cell lung cancer. Figure 1 shows the number of novel drugs approved from 2006 to 2015 and the number of novel drugs applications filed with the CDER [5].

Figure 1: New Molecular Entity/Biologic Filings and Approvals from 2006 to 2015



U.S. FDA, 2016 [5]

As described above, the past decade has represented a period of growing innovation, with many new drugs designated as “breakthrough” and “first in class.” There are also indications of continued innovation as biotech and pharmaceutical companies are continuing to increase their investments in research and development (R&D) [6] and a recent study showed rising success rates for new drugs in development [7]. However, this innovation is accompanied by high price tags and rising spending on prescription drugs, placing increasing fiscal pressures on commercial, federal, state, and family budgets.

⁴ Novel drugs are classified as new molecular entities (“NMEs”) by the FDA. NMEs either contain active moieties that have not been approved by the FDA previously, or are characterized as NMEs for administrative purposes. For example, CDER classifies biological products submitted in an application under section 351(a) of the Public Health Service Act as NMEs for purposes of FDA review. (<http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugInnovation/ucm20025676.htm>).

Drug manufacturers often point to high drug development costs as a justification for high drug prices and understanding the R&D costs and time to develop a new drug is important. However, the relationship between R&D costs and drug prices is subject to a number of misconceptions. In reality, the prices charged for drugs are unrelated to their development costs. Drug manufacturers set prices to maximize profits [8]. At the time of marketing, R&D costs have already occurred and do not affect the calculation of a profit-maximizing price.

Lower drug development costs, however, do help to spur innovation in drug development [9]. When drug manufacturers consider prospectively whether to invest in developing a new drug, they weigh the costs of development against future returns. Shorter development times and lower R&D costs make investing in developing new drugs more attractive by increasing expected net returns.

The following sections describe the clinical trial process used for new drug development and features of the development process that affect timing and cost of new drug development, including clinical development time, preclinical development time, cost of capital, success rates, preclinical costs, and clinical trial costs. Published estimates are presented for each component. Also described are analyses of key inputs for calculating time and costs of new drug development that were conducted specifically for this report.

Phases of New Drug Development

Before a new drug begins clinical trials, it undergoes prehuman or preclinical testing, which does not involve use of the drug in humans. This phase includes discovery and development of the molecule as a potential drug and preclinical testing for safety and toxicity in the in vitro and in vivo (animal) settings. Often, animal testing in this phase provides necessary information on a drug's risks of birth defects.

Clinical trials are the backbone for the collection of safety and efficacy data required by the FDA to approve a new drug. A clinical trial is defined as a research study in which one or more human subjects are prospectively assigned to one or more drugs (which may include a placebo or other usual care controls) to evaluate the effects of that drug on health-related biomedical or behavioral outcomes [2]. Clinical testing is often described in terms of phases for simplicity, although the actual development process may be much more complex without clear distinctions between phases.

Phases 1, 2, and 3 involve testing in humans that is carried out before approval to demonstrate safety and efficacy. Phase 1 is the first clinical phase of development and usually involves 20 to 100 healthy volunteers or people with the disease or condition to be treated to assess safety/toxicity and to identify potential therapeutic dosages that could be studied in larger clinical trials. In Phase 2, the drug is studied in up to several hundred people with the disease or condition to obtain data regarding efficacy and to further characterize adverse events. Often, these studies are not large enough to demonstrate the full benefits or risks of the drug. Phase 3 studies are conducted to determine whether the drug offers a treatment benefit to a specific population. These studies typically involve 300 to 3,000 participants, although in some cases Phase 3 studies may be much larger. Phase 3 studies are generally conducted in patients randomized to receive either the study drug or a placebo or usual care treatment. Phase 3 studies provide most of the efficacy and safety data on a drug that is submitted to FDA for approval, and tend to be longer than Phase 2 studies.

After approval of a drug, post-marketing studies (or Phase 4 studies) are sometimes conducted (or required to be conducted by the FDA) to assess the drug's safety (and sometimes, efficacy). Drug sponsors also may voluntarily undertake Phase 4 studies to increase utilization and exposure of their product to a broader group of providers and patients. The following sections describe the time to develop new drugs.

Time to Develop New Drugs

Estimates of the length of time required to develop a new drug requires considering two separate periods: time spent in clinical development, and time spent in preclinical development. Below estimates of each are presented based on an analysis of internal FDA data and previously published literature on the topic.

Clinical Development Time (CDT)

Clinical development time (CDT) is defined as the time from the filing of the key investigational new drug (IND) application with the FDA to the time of FDA approval. The key IND is the IND under which the sponsor conducted the primary development and regulatory interactions that led to the submission of the marketing application⁵.

Data on new drug approvals⁶ from Oct 1, 2007 through February 29, 2016 from an internal FDA database were used⁷ to determine the CDT for approved new drugs. During this time period, there were 297 approvals for either a new drug or new indication (for approximately 269 unique drugs).

Among the 297 approvals, four drugs had no CDT; that is, their sponsor did not submit an IND to FDA prior to submitting a marketing application so CDT could not be calculated⁸. For the remaining 293 approvals, CDT ranged from 1.5 years to 43.9 years with an average of 8.4 years and a median of 7.4 years. When adjusted to include only the oldest key IND date per unique drug, the mean CDT was 8.6 years and the median was 7.5 years. The distribution of adjusted CDTs is summarized in Figure 2.

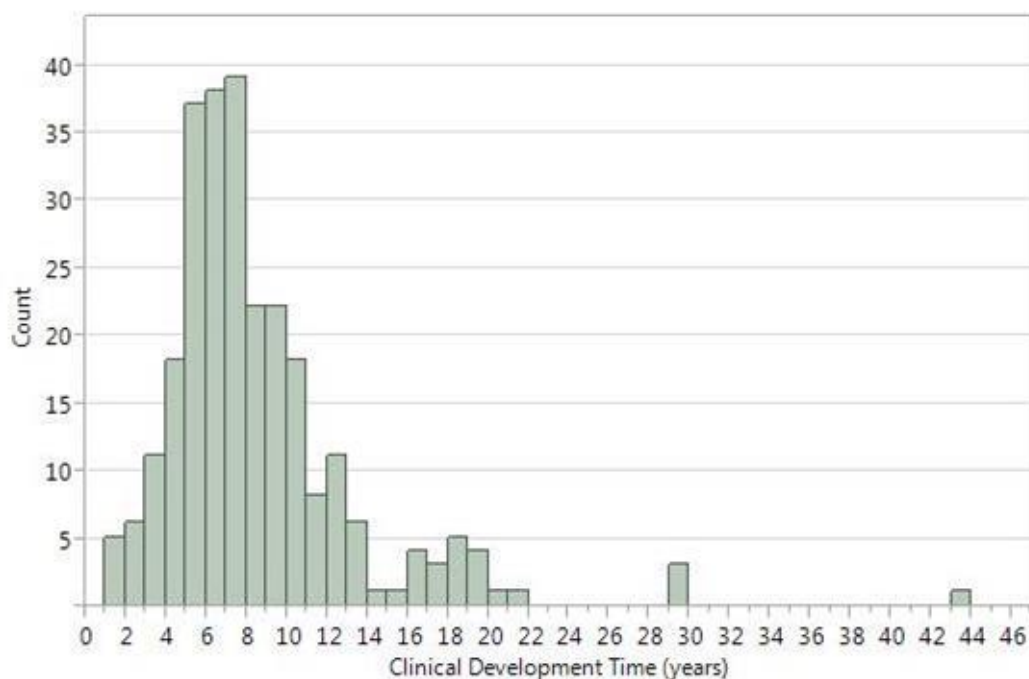
⁵ A limitation to this approach is that there may be multiple INDs for a given drug. For example, sponsors may choose to submit new INDs for different indications submitted to FDA, or a new IND may be opened when the IND changes hands, such as transfer from one sponsor to another. Thus, the key IND does not always represent the first administration of the investigational drug under IND in the United States. For the drugs that received more than one indication at time of first approval, more than one CDT would be noted for a unique drug (e.g., for the miltefosine example, three CDTs for the three drug + indications would be noted). In order not to multi-count CDTs in this analysis, an adjusted CDT was calculated such that only one CDT per unique drug was included. In some cases, the key INDs for the different indications were different and the drug may have had more than one CDT (that is, one indication developed under one key IND, the other under a different key IND). In these cases, the older key IND was used for the adjusted CDT since it would more closely approximate first contact with FDA for drug development.

⁶ New molecular entity New Drug Approvals (NDAs) and original Biologics License Applications (BLAs)

⁷ This data set contains a compilation of CDER's regulatory science metadata that is identified and extracted by FDA staff with extensive regulatory and drug development experience and training and an experienced contractor. Consistency is maintained through a detailed standard operating procedure with data definitions, and through adjudication of some of the information by a committee; however, there is an unavoidable subjective component to some of the information (such as what constitutes the efficacy population for a clinical development program), and caution is advised when interpreting the data since opinions may vary.

⁸ This situation may be due to the sponsor relying on foreign clinical trials for its new drug application to the FDA.

Figure 2: Distribution of Clinical Development Time for New Drug Approvals



This estimate of the average CDT is largely consistent with estimates reported in the published literature. DiMasi et al (2016) [1] uses a mean CDT of 8.1 years for a sample of drugs that began clinical development between 1995 and 2007. Paul et al (2010) [10] estimates a mean CDT of 8 years for drugs in the pipeline from approximately 1997 to 2007. Kaitin and DiMasi (2011) [11] estimate CDTs of 8.1 years for drugs with priority review versus 7.7 years for standard approvals based on data on novel drugs approved from 2005 to 2009. Use of the common definition of CDT – from IND to approval – likely leads to the generally consistent estimate of approximately 8 years for CDT.

Preclinical Development Time (PDT)

Time spent in preclinical development time (PDT) is much less clearly defined than the time spent in CDT, making it much more difficult to develop reliable estimates. Activities included in PDT include developing and validating a drug target and animal testing. Paul et al (2010) [10] used a benchmark PDT of 5.5 years, but excluded activities like discovering and validating a target. More recently, DiMasi et al (2016) [1] estimated an average time from synthesis to initial human testing of 31 months or 2.6 years. Poor understanding of PDT is a significant issue in developing reliable estimates of new drug development costs.

Factors associated with costs of bringing a new drug to market

Time spent in development is an important factor in estimating the cost of developing a new drug. Other important factors are the cost of capital, success rates, preclinical costs, and clinical trial costs.

Cost of capital

The drug development process is very long, which means that resources devoted to development are not available for other uses for many years. As a result, the opportunity cost of these resources is significant. To account for opportunity costs, calculation of the cost of drug development includes the cost of capital. Most commonly, the cost of capital is calculated based on the average expected returns for stock market investments using a Capital Asset Pricing Model (CAPM) [1, 12, 13]. The CAPM is based on: 1) the

estimated risk-free cost of capital, 2) the equity market risk premium, and 3) the non-diversifiable risk⁹. The cost of capital in the biopharmaceutical industry is unusually high relative to most other industries. A recent analysis by Koijen et al (2016) [14] found a “medical innovation premium” of 4-6 percent annually for equity returns in the medical R&D sector. This premium means that financial returns in the medical R&D sector have exceeded overall market returns. Recent estimates of the cost of capital for the biopharmaceutical industry have used a cost of capital of 10.5 or 11 percent [1, 15]. However, some analysts have argued that using the cost of debt or even excluding the opportunity costs for invested resources is a more appropriate approach [16, 17].

Because drug development timelines are long, the choice of whether to include the cost of capital in estimating the cost of drug development and, if so, what value to use for the cost of capital has a significant impact on the total cost estimate. The long development times and the high costs of capital means that the out-of-pocket estimates (excluding the cost of capital) for the cost of drug development are much lower than the capitalized cost estimates (including the cost of capital).

Success rates

For each new drug that successfully reaches the market, many potential drugs begin development, but fail before approval. Typically, the cost of developing a new drug refers not only to the cost of bringing a single new drug successfully from discovery to marketing, but includes the costs of failed potential new drugs that do not reach the market. The success rate for a new drug from the beginning of human testing to marketing varies considerably. For example, Hay et al (2014) [18] investigated success rates (the likelihood of reaching approval from the start of clinical testing) by indication and found rates ranging from 6.7 percent for oncology indications to 45.9 percent for infectious disease indications. A success rate of 6.7 percent means that approximately 15 molecules need to enter the pipeline of drugs in development for every approved new oncology drug versus approximately two molecules needed for a 45.9 percent success rate for a new infectious disease drug. Smietana et al (2016) [7] investigated success rates from Phase 1 to marketing and found that rates have increased in 2012-2014 to 11.6 percent from a low of 7.5 percent in 2008-2011, although still down from a high of 16.4 percent in 1996-1999.

Success rates have a large impact on new drug development costs as the costs for each of the failures is incorporated into the cost of a success. A challenge with using these success rates to estimate drug development costs, however, is that early phase development costs appear to be higher for new drugs that go on to receive marketing approval. Therefore, estimating costs of failed candidates based on clinical trial costs for successful drugs may bias cost estimates. Also, because sponsors halt development of investigational new drugs for many reasons, not just scientific, failure rates also reflect business decisions on the part of the sponsor [7].

Preclinical costs

Pre-clinical and discovery costs are the costs to discover a potential new drug with sufficient promise to enter clinical trials. This includes in vivo and animal testing, as well as basic research into understanding the mechanism of disease. Allocating this spending to a specific drug is challenging, as this research may support multiple therapeutic areas or potential new drugs. Additionally, because there is a long time between investing these resources and the payoff, incorporating the cost of capital can make preclinical costs a very large contributor to the overall cost. For example, in a recent estimate of the costs to develop a new drug, preclinical costs were \$1.1 billion of an estimated \$2.6 billion in total R&D costs [1].

⁹ Nondiversifiable risk is risk that is common to an entire class of assets.

Clinical trial costs

Clinical trial costs are perhaps the most easily measured element of new drug development because they are the most concrete and clearly defined. Clinical trial costs vary with the number of sites where patients are treated, number of patients enrolled, length of the trial, and complexity of the clinical trial protocols. A recent study looked at mean costs by phase and therapeutic area for clinical trials and found that the average Phase 3 trial costs ranged from \$11.5 million (dermatology) to \$52.9 million (pain and anesthesia) [19]. However, some clinical trials, such as trials for certain indications that enroll tens of thousands of patients, may have considerably higher costs.

Estimates of research and development (R&D) costs in bringing a new drug to market

Mean cost estimates

Table 1 presents estimates of the mean research and development (R&D) costs to develop a new drug based on research published since 2003. The mean costs range from \$1.1 to \$2.6 billion (in 2013 dollars). However, care is necessary in interpreting mean costs; R&D costs vary systematically by therapeutic area, firm size, whether the drug is licensed (“licensed-in”) ¹⁰ from another company or company originated (“self-originated”), and Orphan status. In addition, licensed-in and Orphan drugs are frequently under-represented in the data sets on which the drugs cost estimates rely. Also, the distribution of costs is skewed, with mean costs typically higher than the median costs [1, 15].

Table 1: Estimates of R&D Costs in the Literature

	Study Period	Cost of Capital	Probability of Success from Phase 1	Mean R&D Cost (2013\$)
DiMasi et al, 2003 [2]	First in humans, 1983-1994	11.0%	21.5%	\$1,064
Adams and Brantner, 2006 [4]	First in humans, 1989-2002	11.0%	24.0%	\$1,152
Adams and Brantner, 2010 [3]	First in humans, 1989-2002	11.0%	24.0%	\$1,610
Paul et al, 2010 [10]	1997-~2007	11.0%	11.7%	\$1,927
Mestre-Ferrandez, 2012 [15]	In clinical development, 1997-1999, followed until 2012	11.0%	10.7%	\$1,554
DiMasi et al, 2016 [1]	Initial human testing 1995-2007	10.5%	11.8%	\$2,558

To illustrate the challenges of developing an up-to-date and generalizable estimate representing development costs, DiMasi et al’s (2016) [1] costs of development estimate is evaluated under different scenarios. Table 2 shows key study parameters and the mean and median capitalized and out-of-pocket estimates of the costs of development. The researchers base their estimate on a sample of 106 drugs (87 small molecule and 19 biological drugs) in development from 10 pharmaceutical companies initially tested in humans between 1995 and 2007. The sample does not include licensed-in drugs where partner cost data were not available. The sample includes only two Orphan drugs, although Orphan drug

¹⁰ Licensed-in drugs begin development with one company and then are acquired by the sponsor. In contrast, self-originated drugs go through all the phases of development with the sponsor.

designation may occur at any point in development. As a result, some of the drugs sampled in earlier stages of development may later be designated Orphan drugs.

Table 2: DiMasi et al 2016 [1] Means and Medians for Out-of Pocket and Capitalized Costs (in Millions)

	Time to Next Phase	Probability of Success	Out of Pocket –Mean	Capitalized –Mean	Out-of Pocket –Median	Capitalized – Median
Pre-human	31.2		\$430	\$1,097	\$324	\$829
Phase 1	19.8	60%	\$214	\$418	\$146	\$286
Phase 2	30.3	36%	\$295	\$480	\$225	\$367
Phase 3	30.7	62%	\$456	\$561	\$357	\$439
Approval	16.0	90%				
Cost of Capital				10.5%		10.5%
Total			\$1,395	\$2,556	\$1,053	\$1,921

Source: DiMasi et al. (2016) [1]

Alternative estimates

Orphan drugs are a large and increasing proportion of new approvals, representing 47 percent of new approvals in 2015, up from 29 percent in 2010. The cost of developing Orphan drugs is important for understanding overall drug costs. Table 3 shows the upward trend in the percent of Orphan approvals, accounting for 28 percent of approvals in 2010 and 47 percent in 2015. Because the DiMasi (2016) [1] sample is lacking in Orphan drugs, likely estimates of the key parameters for Orphan drug development were added from other sources to improve understanding of the cost of developing Orphan drugs.

Table 3 Trend in Orphan Drug Approvals

Year	Orphan Approvals	Novel drug Approvals	Percent Orphan Approvals
2010	6	21	29%
2011	11	30	37%
2012	13	39	33%
2013	9	27	33%
2014	17	41	41%
2015	21	45	47%
Mean	77	203	38%

Source: FDA Novel Drug Summaries 2011-2015[5, 20-23] and Mullard, 2011 [6]

There are several reasons to believe that Orphan drug development is likely to be less costly than non-Orphan drugs. First, Orphan drugs have higher success rates than non-Orphan drugs. Hay et al (2014)

[18] estimated an overall success rate of 32.9 percent for Orphan drugs, in contrast to a 10.4 percent success rates for the entire sample¹¹.

In addition to having higher success rates, Orphan drug approvals, although they have the same standards for efficacy and safety, on average, are typically based on smaller and fewer efficacy and safety trials [24, 25]. Table 4 shows the results of an analysis of the FDA approval data, Orphan drug efficacy trials had a mean of 335 participants and a median of 219; non-Orphan efficacy trials had a mean of 2,495 participants and a median of 1,333. O’Connell and Pariser (2014) [24] looked at population sizes for safety trials for Orphan and non-Orphan approval and found a difference of approximately nine-fold: Orphan drugs safety trials had a median of 282 and mean of 383 patients, while non-Orphan drugs had a median of 2,359 patients and mean of 3,432 patients. Evaluate Pharma (2015) [25] estimated that average phase 3 costs for Orphan drugs are roughly half of the Phase 3 costs for non-Orphan drugs, \$103 million versus \$193 million. Factoring in the U.S. tax breaks, Evaluate Pharma estimated that Orphan drugs Phase 3 costs could average as little as a quarter of Phase 3 costs for non-Orphan drugs. In addition, Evaluate Pharma also estimated average Phase 3 trial sizes of 761 for Orphan drugs and 3,549 for non-Orphan drugs.

Table 4 Efficacy Trial Sizes for Orphan and Non-Orphan Novel Drug Approvals

Efficacy Trials	Enrolled Population
<i>not Orphan</i>	
Mean	2495
Median	1333
<i>Orphan</i>	
Mean	338
Median	219

Based on analysis of FDA data

Orphan drugs are also eligible for a number of incentives, including waived user fees, tax credits, grants, and marketing exclusivity extensions. The value of the Orphan drug tax credit was estimated at \$800,000,000 in total in 2015 and is projected to grow to \$1.3 billion in 2019 [26]. In addition, Orphan drugs in some therapeutic categories may be eligible for other incentives. For example, Impavido, an Orphan drug, received a priority review voucher (PRV) for rare and tropical diseases. Knight Therapeutics, the sponsor, sold the PRV for \$125 million [27]. Vimizim, also approved in 2014, received a rare pediatric disease PRV, which the sponsor, BioMarin sold for \$67 million. In 2015, five of the 21 Orphan drug approvals received PRVs, with one voucher sold for \$350 million by United Therapeutics [27].

Table 5 shows the means and medians adjusted for Orphan drugs. If the parameters from DiMasi et al (2016) [1] are altered using the success rates for Orphan drugs from Hay et al (2014) [18] and reducing Phase 2 and 3 costs by 50 percent (assuming the lower numbers of patients translate into lower costs), mean and median costs of new drug development for Orphan drugs become \$1,007 and \$762 million,

¹¹ This study may overestimate the actual difference in success rates. Drugs may be designated with Orphan status at any point in development, so this may overestimate success rates in early phases.

respectively. This is less than forty percent of the estimated mean and median costs of \$2,556 and \$1,921 million from DiMasi et al (2016) [1].

Table 5. DiMasi et al (2016) [1] Means and Medians Adjusted for Orphan Drugs (in Millions)

	Time to Next Phase	Probability of Success	Out-of-Pocket – Mean	Capitalized – Mean	Out-of-pocket – Median	Capitalized – Median
Pre-human	31.2		\$173	\$442	\$132	\$337
Phase 1	19.8	87%	\$77	\$150	\$52	\$103
Phase 2	30.3	70%	\$77	\$126	\$59	\$96
Phase 3	30.7	67%	\$235	\$289	\$184	\$227
Approval	16.0	81%				
Cost of Capital				10.5%		10.5%
Total			\$562	\$1,007	\$428	\$762

Source: DiMasi et al (2016) [1], Hay et al (2014) [18], Evaluate Pharma (2015) [25], analysis of FDA data

Licensed-in versus self-originated drugs

Licensed-in drugs are important to consider in estimating R&D costs because the majority of drugs in development are licensed-in, and the trend toward more licensed-in drugs continues.[15] These drugs tend to have higher success rates than self-originated drugs and so would be expected to have lower associated costs of development. It is notable, therefore, that the DiMasi et al. (2016)[1] sample previously described excludes licensed-in drugs.

Another study by DiMasi et al, conducted in 2010 [28] evaluated success rates for self-originated and licensed-in drugs using a sample of drugs that began clinical testing anywhere in the world between 1993 and 2004. Overall, 70 percent of the drugs were self-originated. They found that the overall estimated success rates were substantially higher (27 percent) for licensed-in drugs than for self-originated drugs (16 percent). A more recent analysis of success rates also found much higher success rates for partnered compounds (defined as having at least one licensee during development) versus non-partnered compounds (defined as developed by a single owner company). Smietana et al (2016) [7] found that partnered compounds were 8 percentage points more likely to reach approval than nonpartnered compounds. If an 8 percent higher success rate is applied to the other parameters used in DiMasi et al (2016) [1], then the mean and median costs of development fall to \$1,967 and \$1,487 million, respectively (Table 6).

Table 6. DiMasi et al (2016) [1] Means and Medians with Licensed-in Probability of Success (in Millions)

	Time to Next Phase	Probability of Success	Out-of-Pocket – Mean	Capitalized – Mean	Out-of-Pocket - Median	Capitalized - Median
Pre-human	31.2		\$335	\$857	\$255	\$651
Phase 1	19.8	68%	\$136	\$266	\$93	\$182
Phase 2	30.3	44%	\$213	\$347	\$163	\$265
Phase 3	30.7	70%	\$404	\$497	\$316	\$389
Approval	16.0	90%				
Cost of Capital				10.5%		10.5%
Total			\$1,088	\$1,967	\$827	\$1,487

Source: DiMasi et al. (2016) [1], Smietana et al (2016) [7]

Trends in drug development costs

The DiMasi et al (2016) [1] estimates of R&D costs use the same methodology and are comparable to the results of DiMasi et al (2003) [2], which looked at drugs first studied in humans from 1983 to 1994 and found an overall cost of R&D of \$1,064 million (in 2013 dollars). This estimate represents a compound annual growth rate of 9.3 percent in out of pocket costs and 8.5 percent in capitalized costs. DiMasi et al (2016) [1] attributes the cost increase to a number of factors including increasing failure rates, increasing clinical trial complexity, larger clinical trials, inflation in cost of inputs and payer demands for comparative effectiveness data.

An alternative measure of changes in the cost of doing biomedical research, including drug research and development is the biomedical research and development price index (BRDPI). BRDPI measures changes in the weighted average of the inputs purchased with the NIH budget to support research [29]. Because the BRDPI is indexed to NIH spending, it is not a perfect indicator of private sector price changes. However, it does measure similar inputs to activities undertaken by the private sector to develop new drugs [29]. Table 9 provides a comparison of the annual percent changes in the gross domestic price (GDP) price index and BRDPI since 1985. BRDPI shows a growth rate greater than the GDP price index for every year except 2012, but considerably lower than the growth rate implied by comparison of the 2003 and 2016 DiMasi estimates. The BRDPI measures only the changes in the prices of the inputs and does not capture other changes in research and development of new drugs, like changes in success rates, complexity of clinical trials, and shifts in company portfolios.

Table 7. Annual Percent Changes in the Cost of Biomedical Research

Fiscal Year	BRDPI	GDP Price Index
2000	3.7%	2.1%
2001	3.3%	2.4%
2002	3.3%	1.6%
2003	3.5%	1.9%
2004	3.7%	2.5%
2005	3.9%	3.1%
2006	4.6%	3.3%
2007	3.8%	2.7%
2008	4.7%	2.1%
2009	2.9%	1.2%
2010	3.0%	0.9%
2011	2.9%	2.0%
2012	1.3%	1.9%
2013	1.9%	1.7%
2014	2.2%	1.7%

U.S. NIH, 2016 [29], GDP=Gross domestic product, BRDPI=Biomedical research and development price index

Summary

Innovation in new prescription drug development is ongoing, with large numbers of newly approved drugs designated as “first in class” and “breakthroughs”. Between 2006 and 2015, the Food and Drug Administration approved an average of 29 novel drugs a year, with 45 approvals in 2015 alone. Many new drugs are in the development pipeline. Recent published estimates of the cost of new drug development range from \$1.2 billion to \$2.6 billion. These estimates are highly sensitive to assumptions about pre-clinical and clinical development time, cost of capital, the likelihood of reaching approval following the start of clinical testing, and costs of preclinical development and clinical trials conducted among humans. Characteristics of the drugs also play a key role in estimating the cost of new drug development. As Orphan drugs increasingly represent a higher proportion of new drug approvals (47 percent in 2015), consideration of the smaller size of trials, higher success rates, and accompanying tax advantages have a large influence on estimates of R&D costs. Applying updated information yields mean and median development costs of \$1.0 billion and \$0.8 billion for Orphan drugs, respectively, less than half the estimates of drug development costs of \$2.6 billion and \$1.9 billion from DiMasi et al (2016) [1]. Understanding the costs of drug development is useful in assessing public policies to stimulate drug development. However, given the heterogeneity in costs associated with bringing a new drug to market and the limitations of recent studies on this topic, it is important to use these estimates cautiously.

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CHAPTER 3: MEDICARE PART B

This chapter presents information about prescription drugs in Medicare Part B. It describes measures to contain costs through payment policies and the system through which providers are currently reimbursed for prescription drugs. It also presents data and findings from quantitative analyses conducted specifically for this report for overall spending for prescription drugs and recent trends in spending, including spending for biologics, top ten drugs, and by therapeutic classes.

Key Findings

- Medicare Part B prescription drug spending increased from \$10.1 billion in 2006 to \$17.2 billion in 2014, representing an average annual growth rate of 6.9 percent. Part B drug spending grew 4.5 percent annually between 2006 and 2009, then accelerated to 8.4 percent annually between 2009 and 2014. Medicare Part B prescription drug spending as a percentage of total Medicare Part B spending remained relatively modest and stable, averaging about 6.2 percent.
 - Increases in Medicare Part B drug spending have been driven by increases in biologics. Spending on biologics between 2006 and 2014 grew by 13.3 percent annually, whereas spending on small molecule drugs grew by 0.7 percent annually during the same period. In 2014, biologics accounted for 63 percent of Part B drug spending, up from 39 percent in 2005.
 - In 2014, spending was dominated by cancer drugs (45 percent). Spending for drugs that treat rheumatoid arthritis (8.7 percent), intravenous immunoglobulin (IVIG) preparations (5.2 percent), and osteoporosis (4.3 percent) was also common. No other therapeutic class represented more than 3 percent of spending. These four classes were also the dominant therapeutic classes for spending in 2006. Over the past decade, a relatively small number of Medicare Part B drugs have accounted for a significant share of spending. For example, the top 10 drugs in terms of spending accounted for 47 percent of total spending in 2014.
 - Beneficiaries using these high cost drugs potentially faced high cost-sharing.¹² Because there is a 20 percent coinsurance requirement for Part B drugs, a beneficiary using Rituximab, one of the top 2 spending drugs since 2005, would incur cost sharing expected to be \$4,430 for that drug alone in 2014. Even Part B drugs that are not on the top 10 list could impose very heavy burden on the beneficiary; a user of Ipilimumab, ranked 15th, would be expected to incur over \$18,000 of cost sharing in 2014.
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¹² Most beneficiaries have some insurance protection from cost sharing liabilities. About 90 percent of Medicare beneficiaries have supplemental insurance coverage and would have lower patient liability amounts.

Program Overview

Medicare is a federal health insurance program created in 1965 for people ages 65 and older, regardless of income and health conditions. The program was expanded in 1972 to cover people under age 65 with permanent disabilities. Medicare Part B, also known as the Supplementary Medical Insurance (SMI) program, helps pay for physician, outpatient, some home health, and preventive services.

Part B is financed through a combination of general revenues, premiums paid by beneficiaries, interest and other sources. Premiums are automatically set to cover 25 percent of spending in the aggregate, while general revenues subsidize 73 percent. Higher-income beneficiaries pay a larger share of spending, ranging from 35 percent to 80 percent of Part B costs.

Certain types of drugs including infusible and injectable drugs and biologics administered in physician offices and hospital outpatient departments as well as certain other drugs provided by pharmacies and suppliers (e.g., inhalation drugs and certain oral anticancer, oral antiemetic, and immunosuppressive drugs) are covered by Part B [1]. Providers purchase these Part B drugs and Medicare payments are made directly to these providers.

Measures to Contain Cost through Statutory Payment Changes

Prior to 2005, Medicare payment for a covered drug under the Balanced Budget Act of 1997 (BBA) was based on the average wholesale price (AWP) as published in RED BOOK or similar drug pricing publications used by the pharmaceutical industry¹³. Specifically, for covered drugs available only from a brand source, payment was calculated as 95 percent of the drug's AWP. For covered drugs available from brand and generic sources, payment was the lesser amount of 95 percent of the median AWP for generic sources or 95 percent of the AWP for the brand source.

The AWP-based payment system did little to control prescription drug costs. For a number of reasons, reported AWP exceeded the acquisition cost of the drugs considerably. First, the BBA did not define AWP or establish uniform reporting criteria meaning that it would be difficult to regularly verify reported price data and compare it with actual prices paid [2, 3]. Second, manufacturers were not required to report rebates and other discounts so that the published AWP were substantially higher than the actual acquisition prices available to providers who billed for these drugs. While Medicare paid 95 percent of the AWP, most of these drugs were available to providers for 66 - 87 percent of the AWP, with some drugs available for considerably less [4, 5]. As a result, Medicare paid providers roughly one billion dollars more than acquisition costs annually for Part B drugs. Medicare beneficiaries, who were responsible for a 20 percent copayment, paid hundreds of millions of dollars more annually than if payment rates reflected actual acquisition costs [6, 7].

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA) [8] changed payments for Part B drugs beginning in January 2005. Generally, the MMA tied

¹³ The RED BOOK, maintained by Truven Health Analytics, provides consistent and unbiased Average Wholesale Price (AWP) pricing information for brand name and generic drugs.

payment more closely to health care providers' acquisition costs by paying for a drug's average sales price (ASP) plus a 6 percent add-on (106 percent of ASP). The Secretary was provided discretion for drugs administered in hospital outpatient settings, to determine payment based on average acquisition costs or similarly to how payment is made in a physician's office.¹⁴ The Secretary has opted to use ASP based pricing for most Part B drugs provided in hospital outpatient departments since 2006.

In the first year of the transition from AWP to ASP in 2005, Part B pharmaceutical spending declined 8 percent following a period of rapid increases averaging 25 percent per year from 1997 to 2003 [9]. These data strongly suggested that the change to ASP based pricing was an improvement over the prior system based on AWP payment system.

Under the current ASP acquisition process (as under the AWP before it), Medicare has no price-setting power – payment rates reflect market transaction prices with a short lag. As had been the case under the prior payment mechanism, the two-quarter or 6-month delay in updating payment rates means that when the market price of a drug falls, payment rates exceed prices. Over the period following the Medicare Modernization Act (MMA) as drug prices have generally fallen due to competition by multiple source drugs, reimbursement rates, based on lagged prices, could be greater the prevailing sales prices paid by most providers. In addition, the GAO in a 2016 study concluded that the current ASP methodology could become less suitable over time for drugs with coupon programs because the ASP does not account for coupon discounts to patients. Based on a sample of 18 drugs for which the GAO could obtain coupon discounts data, the GAO found that the ASP exceeded the effective market price by about 0.7 percent in 2013 [16].

Current ASP-Based Payment for Part B drugs

The Centers for Medicare & Medicaid Services (CMS) computes the ASP using quarterly sales price and volume of sales data, which manufacturers are required to report, by the National Drug Code, for each drug covered. By definition, ASP is the volume-weighted average of the sales prices to all purchasers in the U.S.¹⁵ The ASP is net of any price concessions such as volume discounts, prompt pay discounts, and cash discounts; free goods contingent on purchase requirements; chargebacks; and rebates other than those obtained through the Medicaid drug rebate program. Sales that are nominal in amount are exempted from the ASP calculation, as are sales excluded from the determination of "best price" in the Medicaid drug rebate program [12]. Each drug within a Healthcare Common Procedure Coding System (HCPCS) code is reimbursed at the ASP-based payment allowance for the code. Branded drugs and the generic version of the same drug use the same HCPCS. To allow time to submit and process data, healthcare providers receive payments based on drugs' ASP with a two quarter or six month lag.

¹⁴ Under MMA's provisions for payment of hospitals' outpatient department services, the Secretary has the authority to base payment for these drugs on hospitals' average acquisition costs and consider overhead/handling costs in setting payment, or if such acquisition cost data are not available, then the Secretary can also use the same payment as for physicians' offices. In recent years, CMS has chosen the latter option so that most drugs are paid the same rate in the two sites of service. Drugs that are under a cost per day threshold cost (\$95 for CY 2015) are not paid separately in hospital outpatient departments – they are packaged with associated procedures or visits for payment.

¹⁵ CMS computes the volume-weighted ASP based on manufacturers' unweighted data. Not all HCPCS codes have ASP payment limits calculated or published, and CMS only has about 6 weeks to process, verify and clear the data before it has to be released to contractors in pricing files.

Providers are paid 106 percent of ASP, regardless of the acquisition costs they actually incur. Payment does, however, vary based on the classification of drug administered. Payment formulas separate Part B drugs into three categories: single-source small molecule drugs and biologics, multiple-source small molecule drugs, and biosimilars. Single-source small molecule drugs -- without generic substitutes -- and biologics are both reimbursed at 106 of their own ASP. For multiple-source small molecule drugs, all equivalent brand-name and generic products are reimbursed at 106 percent of the weighted average of their ASPs. In other words, each single-source drug has a unique ASP, regardless of the similarities between drugs, allowing two drugs that have comparable effectiveness to have very different payment rates. Both the generic and brand name versions of a multiple-source drug, on the other hand, would have an identical ASP based payment rate.

The complexity of FDA requirements behind approving biosimilar products distinguishes them from other Part B drugs. Biosimilars are biological products that have been shown to have no clinically meaningful differences from an FDA-licensed biological product, known as the reference product. Unlike multiple-source small molecule drugs, however, in order to be considered substitutable with brand names, biosimilars must demonstrate that they are “interchangeable”¹⁶ with their reference product [13]. Biosimilars are defined separately from the reference product for the purposes of Part B payment and will not be grouped for billing and payment with the reference product. Instead, approved biosimilars of the same reference product will be billed under a single code and reimbursed at rate that reflects the weighted average ASP. The 6 percent add-on, however, will be derived from the ASP of the reference product. Thus providers will receive this presumably higher add-on regardless of whether they purchase the brand biologic or a biosimilar biologic product.

Incentives under the current ASP payment system

The current payment system for Part B drugs falls short of providing value-based incentives. On one hand, the method of reimbursing 106 percent of ASP with a six-month lag in updating payment rates does encourage providers to seek lower prices to increase their short run margins on each item, which should slow the rate of growth in payment because the discounts would be reflected six months later. In addition, the six-month lag may discourage manufacturers from raising prices substantially at one time because providers may be less likely to purchase at the new price until it is accurately reflected in the ASP¹⁷.

¹⁶ The Patient Protection and Affordable Care Act (Affordable Care Act), signed into law by President Obama on March 23, 2010, amends the Public Health Service Act (PHS Act) to create an abbreviated licensure pathway for biological products that are demonstrated to be “biosimilar” to or “interchangeable” with an FDA-licensed biological product. This pathway is provided in the part of the law known as the Biologics Price Competition and Innovation Act (BPCI Act). A biosimilar product is a biological product that is approved based on a showing that it is highly similar to an FDA-approved biological product, known as a reference product, and has no clinically meaningful differences in terms of safety and effectiveness from the reference product. An interchangeable biological product is biosimilar to an FDA-approved reference product and meets additional standards for interchangeability. For more information refer to: <http://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/TherapeuticBiologicApplications/Biosimilars/>

¹⁷ In addition, Medicare may over reimburse 340B providers for administering Part B drugs. By participating in the 340B program, certain hospitals and health care providers -- known as “covered entities” --can obtain large discounts on covered outpatient drugs. Importantly, covered entities can purchase any Part B drug, except vaccines, at the

On the other hand, any effect that ASP-based payment has on restraining price increases due to the six-month lag mechanism is likely to be outweighed by other incentives that run counter to providers making choices consistent with high value care. The current payment system does not provide incentives to choose the lowest price drugs available to effectively treat a patient. Physicians can often choose between several similar drugs for treating a patient and there is no incentive to make these choices among the therapeutic options with an eye towards value. The payment rates are based on the purchase prices paid by physicians and hospitals to the manufacturers and a percentage add-on, which means that choosing higher price drugs results in higher dollar payments. In addition, physicians and hospitals may be able to negotiate larger concessions on the higher price drugs, meaning larger short run margins.

There has also been some concern about the effect of the additional 6 percent margin on these choices¹⁸. The fixed 6 percent provides a larger dollar add-on for higher price drugs than for lower price drugs. In other words, if a physician were choosing between clinically equivalent drugs, that physician would receive a \$10,600 payment for a \$10,000 drug and \$10.60 payment for a \$10 drug. Unless overhead costs are proportional to the price of a drug, the larger dollar add-on for the higher price drugs results in increased profit margins for the physicians' office and hospitals (\$600 vs \$0.60 in the example above). Therefore, there is even more potential incentive for choosing the high price drugs as opposed to lower price alternatives of similar effectiveness.

The incentives do differ for multi-source Part B drugs. The brand drug and the generic equivalents are grouped under one billing code and ASP is calculated as a weighted average for the group. Thus, if providers choose this group for treatment, they have the incentive to purchase the lower price alternatives in the group. However, as described above they may have a greater incentive to purchase a higher price clinically equivalent, single source drug.

Medicare Part B Spending and Spending Trends

The following sections describe the Medicare claims files and the Part B drugs paid under the ASP system that were included in the analyses of trends, biologics, top ten drugs in terms of spending, and top therapeutic classes.

Data and Methods

The Medicare claims data used in the analyses of spending and trends in spending include Part B covered drugs administered in physicians' offices and furnished by suppliers (carrier and durable medical equipment (DME) claims files) and covered drugs in hospital outpatient departments

340B discounted price. Additionally, Medicare pays covered entities for certain 340B drugs, such as those used to treat cancer and rheumatoid arthritis. Despite these discounts, however, Medicare pays 106 percent of ASP both to hospitals and health care providers that participate in the 340B Drug Pricing Program and to those who do not. This lack of distinction often causes reimbursement payments for 340B providers to be significantly more than 106 percent of their acquisition costs.

¹⁸ There is no consensus on the exact rationale for the add-on to ASP. It may reflect overhead costs (storage, handling) or a cushion against the 6 month lag before reimbursements reflect transaction prices. It is also not clear how the 6 percent was derived.

(outpatient (OP) claims files) from 2006 to 2014. Analyses start with calendar year 2006 because it is the first year that most hospital outpatient departments began using ASP methodology for payments.

Medicare Part B drugs are identified by the Healthcare Common Procedure Coding System (HCPCS) codes in the claims data. Analyses are restricted to Part B drugs paid under the ASP system. As a result, the analyses exclude vaccines, blood products with P* codes (but include blood clotting with J & Q codes), exclude claims in the durable medical equipment (DME) file with an AWP flag, and exclude enteral and parenteral drugs that have B* codes.

HCPCS that are ESRD¹⁹ drugs or that do not represent drugs were dropped from the analyses. Codes and prices for carrier and DME were obtained from the CMS ASP files, while those for outpatient (OP) come from the CMS Addendum B files. Claim lines with denied payments or Medicare as secondary payer were dropped from the analyses. Medicare payments include Medicare program payments and beneficiary cost sharing, and include the effects of the budget sequestration beginning in 2013, which reduced Medicare spending rates by a fixed 2 percent per year²⁰.

Measures

Biologics and therapeutic classes of Medicare Part B drugs were identified by Acumen based on expert consultants and various sources including the FDA biologics definition and the Medicare Claims Processing Manual [14]. The major therapeutic classes include: intravenous immune globulin, blood clotting, anticoagulant, osteoporosis, infusion or oral cancer, oral anti-nausea, rheumatoid arthritis, immunosuppressive, antigen, clot buster, single antigen intravenous immune globulin, and immune globulin for intramuscular administration. Spending was not evaluated separately for generics because the HCPCS codes used in claims are the same for the branded small molecule drug and the generic version of that drug.

Annual prescription drug costs do not adjust for biomedical inflation.

Overall spending and spending trends

In CY2014, total Medicare expenditures were \$613 billion, of which, \$266 billion was for the total Part B benefit²¹ [15]. The Part B drug benefit in 2014 was \$17.2 billion and it constitutes a relatively small component (6.6 percent) of total Part B benefits. Medicare Part B prescription

¹⁹ ESRD drugs were mostly bundled into the ESRD facility composite rates by 2014.

²⁰ The budget sequestration in 2013 refers to the automatic spending cuts to United States federal government spending in particular categories of outlays that were initially set to begin on January 1, 2013, as an austerity fiscal policy as a result of Budget Control Act of 2011 (BCA), and were postponed by two months by the American Taxpayer Relief Act of 2012 until March 1 when this law went into effect. The nine-year cuts (2013-2021) are split evenly (by dollar amounts, not by percentages) between the defense and non-defense categories. Some major programs like Social Security, Medicaid, federal pensions and veteran's benefits are exempt. By a special provision in the BCA, Medicare spending rates were reduced by a fixed 2 percent per year. That is providers and health insurance plans will be paid 98 cents on the dollar under Medicare for the entire nine-year period 2013-2021. As the sequester applies to federal payment only (80 percent of total payment while beneficiaries still pay the full 20 percent copay), the effective federal payment under ASP+6% is $ASP + (1.06 * (1 - 2\% * 80\%))$ or ASP+4.3%

²¹ Medicare Trustees Report 2015, Table II.B1, p. 11.

drug spending as a percentage of total Part B spending remained relatively modest and stable throughout this period, averaging about 6.2 percent. Nevertheless, Part B drug spending has been growing faster than total Part B benefit spending over the 2006-14 period, and Part B drug spending has accelerated in the last five years. The total Part B drug program payment per enrollee increased from \$250 in 2006 to \$269 in 2009 and to \$349 in 2016

As shown in Table 1, Medicare Part B prescription drug spending increased from \$10.1 billion in 2006 to \$17.2 billion in 2014, implying an average growth rate of 6.9 percent while total Part B benefit spending grew at 5.9 percent annually over the same period [10]. In addition, Part B drug spending grew 4.5 percent annually between 2006 and 2009, and accelerated to 8.4 percent annually between 2009 and 2014.

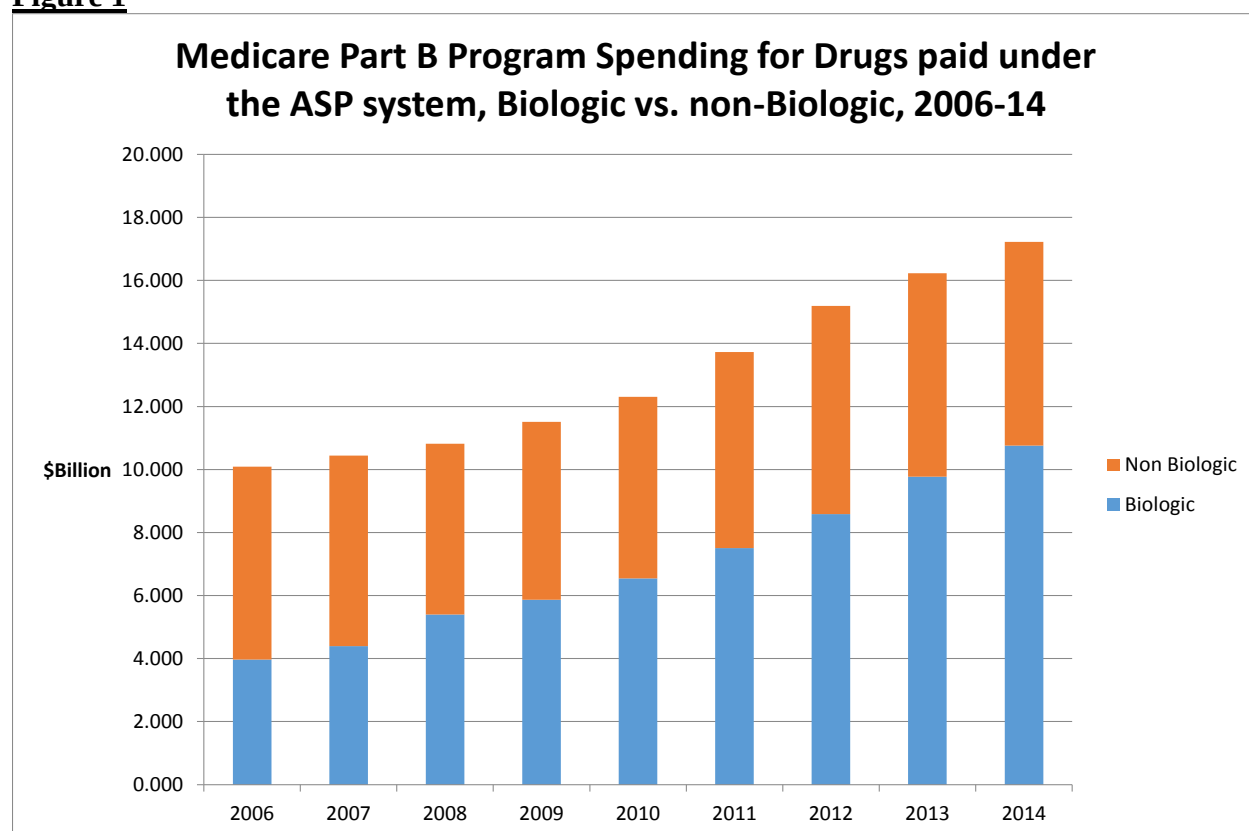
Table 1

Medicare Part B Program Spending for Drug Benefits, 2006-2014									
Year	2006	2007	2008	2009	2010	2011	2012	2013	2014
Total Part B Benefit Spending (\$B)*	165.9	176.4	180.3	202.6	209.7	221.7	236.5	243.8	261.9
Part B Drug Program Spending (\$B) **	10.1	10.4	10.8	11.5	12.3	13.7	15.2	16.2	17.2
Total Enrollees (M) *	40.3	40.9	41.7	42.8	43.8	44.9	46.4	47.9	49.3
Total B Benefit Spending per Enrollee (\$)	4,117	4,313	4,324	4,734	4,788	4,938	5,097	5,090	5,312
B Drug Program Payment per Enrollee (\$)	250	255	259	269	281	306	327	339	349
Part B drugs' share of Part B benefit	6.1%	5.9%	6.0%	5.7%	5.9%	6.2%	6.4%	6.7%	6.6%
* United States Centers for Medicare and Medicaid Services. Medicare Trustees Reports, 2006-2015. June 2016.									
**Analysis of carrier, durable medical, and outpatient claims data 2006-2014 by Acumen for ASPE									

Specialty drugs and biologics

As shown in Figure 1 and Table 2, increases in spending in the Medicare Part B program have been driven by increases in biologics. Spending on biologics between 2006 and 2014 grew by 13.3 percent annually, whereas spending on small molecule drugs grew by 0.7 percent annually during the same period. In 2014, biologics accounted for 63 percent of prescription drug spending.

Figure 1



Similarly, as shown in Table 2, spending per Medicare Part B enrollee increased by 10.4 percent annually for biologics, but declined by 1.8 percent annually for small molecule drugs. When evaluated by user, spending increased by 8.8 percent annually for biologics and 2.4 percent annually for small molecule drugs.

Table 2

Medicare Part B program spending for Drugs Paid under Average Sales Price (ASP), 2006-14										
	2006	2007	2008	2009	2010	2011	2012	2013	2014	Annual increase 2006-14
Medicare Part B Drug Program Payment (\$B)										
Program Pay - All	10.1	10.4	10.8	11.5	12.3	13.7	15.2	16.2	17.2	6.9%
Annual change		3.4%	3.7%	6.4%	6.9%	11.6%	10.7%	6.8%	6.1%	6.9%
Biologic	4.0	4.4	5.4	5.9	6.5	7.5	8.6	9.8	10.8	13.3%
Annual change		10.8%	22.7%	8.7%	11.5%	14.7%	14.3%	13.8%	10.1%	13.3%
Non Biologic	6.1	6.0	5.4	5.6	5.8	6.2	6.6	6.5	6.5	0.7%
Annual change		-1.3%	-10.2%	4.1%	2.2%	7.9%	6.2%	-2.3%	0.0%	0.8%
Medicare Part B Drug Program Payment per Part B enrollee (\$)										
Program Pay (\$)	250	255	259	269	281	306	327	339	349	4.3%
Annual change		1.9%	1.7%	3.6%	4.5%	8.8%	7.1%	3.5%	3.1%	4.3%
Biologic	99	108	129	137	149	167	185	204	218	10.4%
Non Biologic	152	148	130	132	132	139	142	135	131	-1.8%
Medicare Part B Drug Program Payment per user (\$)										
Program Pay (\$)	977	980	1,110	1,142	1,322	1,460	1,564	1,656	1,848	8.3%
Annual change		0.3%	13.3%	2.8%	15.8%	10.4%	7.1%	5.9%	11.6%	8.4%
Biologic	3,490	4,163	4,536	4,759	5,026	5,685	5,984	6,435	6,842	8.8%
Non Biologic	617	585	582	585	655	699	722	703	747	2.4%
Source: Analysis of carrier, durable medical, and outpatient claims data 2006-2014 by Acumen for ASPE										
Data include Part B covered drugs administered in physicians' offices and furnished by suppliers, covered drugs in hospital outpatient departments; and reflect only Part B drugs paid under the average sales price plus 6 percent (ASP).										
The Healthcare Common Procedure Coding System (HCPCS) codes and prices for carrier and DM were obtained from the CMS ASP file, those for OP come from the CMS Addendum B file. Lines with denied payments or Medicare as secondary payer were dropped. Medicare payments include Medicare program payments and beneficiary cost sharing and include the sequester. The analyses started in 2006 when most Part B drugs in Outpatient departments were paid under ASP.										
*United States Centers for Medicare and Medicaid Services. Medicare Trustees Reports, 2006-2015. June 2016.										
https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/ReportsTrustFunds/TrusteesReports.html										

Top therapeutic classes

In 2014, spending in Medicare Part B was dominated by cancer drugs (45 percent). Spending for drugs that treat rheumatoid arthritis (8.7 percent), intravenous immunoglobulin (IVIG) preparations (5.2 percent), and osteoporosis (4.3 percent) was also common. No other therapeutic class represented more than 3 percent of spending. These four classes were also the dominant therapeutic classes for spending in 2006 (Table 4).

Table 3

Medicare Spending levels for Part B Drugs, 2006-2014

By Biologic/non-Biologic, Therapeutic Class, Selected Places of Service

Category	2006		2014	
	Program Payments	Percent of total	Program Payments	Percent of total
All	10,090,556,389.18	100.0%	17,221,426,368.47	100.0%
Biologic	3,972,021,015.30	39.4%	10,759,341,131.75	62.5%
Non Biologic	6,118,535,373.87	60.6%	6,462,085,236.73	37.5%
Anti-Coagulant	6,071,522.60	0.1%	88,331,447.83	0.5%
Antigen	19,905,650.04	0.2%	19,572,146.51	0.1%
Blood Clotting	191,175,761.37	1.9%	434,960,404.87	2.5%
Cancer	4,068,368,256.56	40.3%	7,749,886,317.80	45.0%
Clot Buster	44,391,839.60	0.4%	48,817,354.54	0.3%
IG Intramuscular Admin	2,253,342.09	0.0%	123,142.62	0.0%
Immunosuppressive	319,942,303.38	3.2%	347,223,267.20	2.0%
immune globulin intravenous (IGIV)	217,065,808.81	2.2%	899,689,851.99	5.2%
Oral Anti-Nausea	13,418,384.67	0.1%	956,028.21	0.0%
Oral Cancer	2,338,101.47	0.0%	91,166.12	0.0%
Osteoporosis	277,221,316.86	2.7%	743,845,461.49	4.3%
Rheumatoid Arthritis	584,766,906.50	5.8%	1,498,886,899.59	8.7%
Single Antigen Admin	19,192,057.38	0.2%	11,511,216.58	0.1%
Hospital PLC-Service	2,162,363,774.47	21.4%	6,130,024,105.89	35.6%
Physician Office PLC-Service	6,565,343,638.10	65.1%	9,184,285,176.61	53.3%
ASC PLC-Service	293,513.64	0.0%	8,816,895.78	0.1%

Source: Analysis of carrier, durable medical, and outpatient claims data 2006-2014 by Acumen for ASPE

Data include Part B covered drugs administered in physicians' offices and furnished by suppliers, covered drugs in hospital outpatient departments; and reflect only Part B drugs paid under the average sales price plus 6 percent (ASP).

The Healthcare Common Procedure Coding System (HCPCS) codes and prices for carrier and DM were obtained from the CMS ASP file, those for OP come from the CMS Addendum B file. Lines with denied payments or Medicare as secondary payer were dropped. Total payments include Medicare program payments and beneficiary cost sharing and include the sequester.

The analyses started in 2006 when most Part B drugs in Outpatient departments were paid under ASP.

Spending concentration for top ten drugs

A relatively small number of Part B drugs account for a significant share of the spending. The top 10 drugs in terms of Medicare Part B drug program payment account for 47 percent of the total spending in 2014. Concentrated spending for a relatively small number of drugs has been consistent for the past decade.

In addition, many of the same drugs have been on the top ten list for many years. Table 3 lists the top ten and top twenty drugs by total payments in 2014 and Figure 2 displays the top ten drugs every year between 2006 and 2014. Rituximab (shown in green in Figure 2) has remained in the top two of the top ten list for all nine of the years evaluated for this report. In 2014, Medicare Part B payments were \$1,242.9 million for Rituximab alone, equivalent to \$17,638 per beneficiary and \$4,266 per injection. A beneficiary using Rituximab would incur cost sharing expected to be \$4,430 for that drug alone in 2014. Ranibizumab (shown in yellow in Figure 2)

Prescription Drugs: Innovation, Spending, and Patient Access

entered the top ten list in 2008 and remained high on the top ten list through 2014. Medicare Part B payments for Ranibizumab were \$1,064.8 million in 2014, representing \$7,498 per beneficiary and \$1,575 per service. Beneficiary cost sharing for Ranibizumab is estimated to be \$1,884 in 2014. Other drugs on the top ten list, such as Infliximab, Pemetrexed, Trastuzumab, and Bortezomib, each represented less Medicare Part B spending in 2014, but greater spending per beneficiary (more than \$15,000). Other drugs, such as Darbepoetin were at the top of the list in 2006 and 2007, but dropped below the top ten in 2012. Certain high cost Part B drugs could impose very heavy burden on the beneficiary; a user of Ipilimumab, a drug that is not even on the top 10 list, would be expected to incur over \$18,000 of cost sharing in 2014.

Table 4

CY2014

Top 10 and 20 Part B Drugs by Total Payments

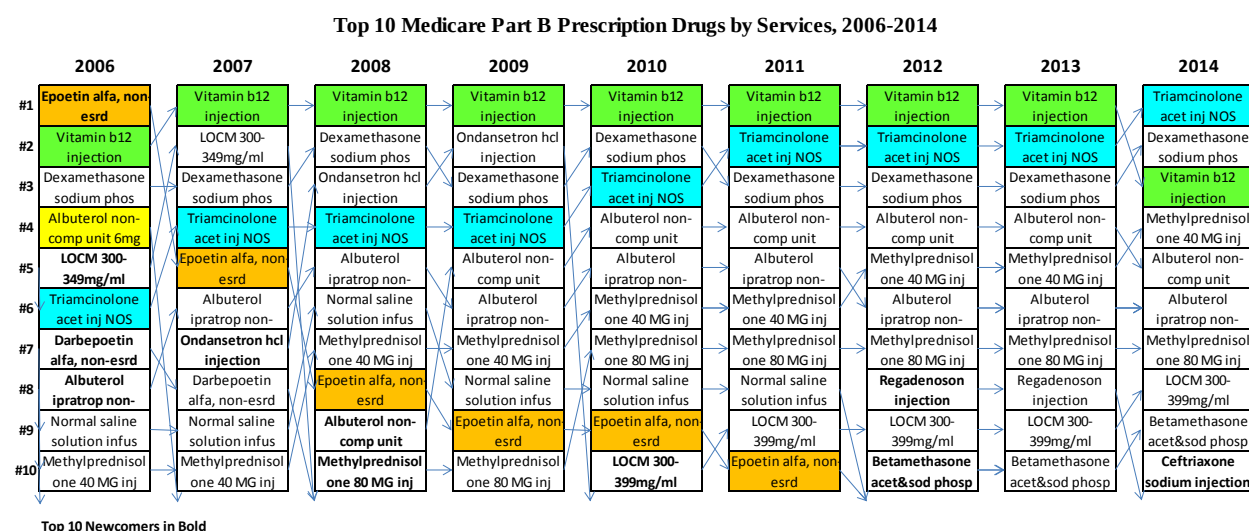
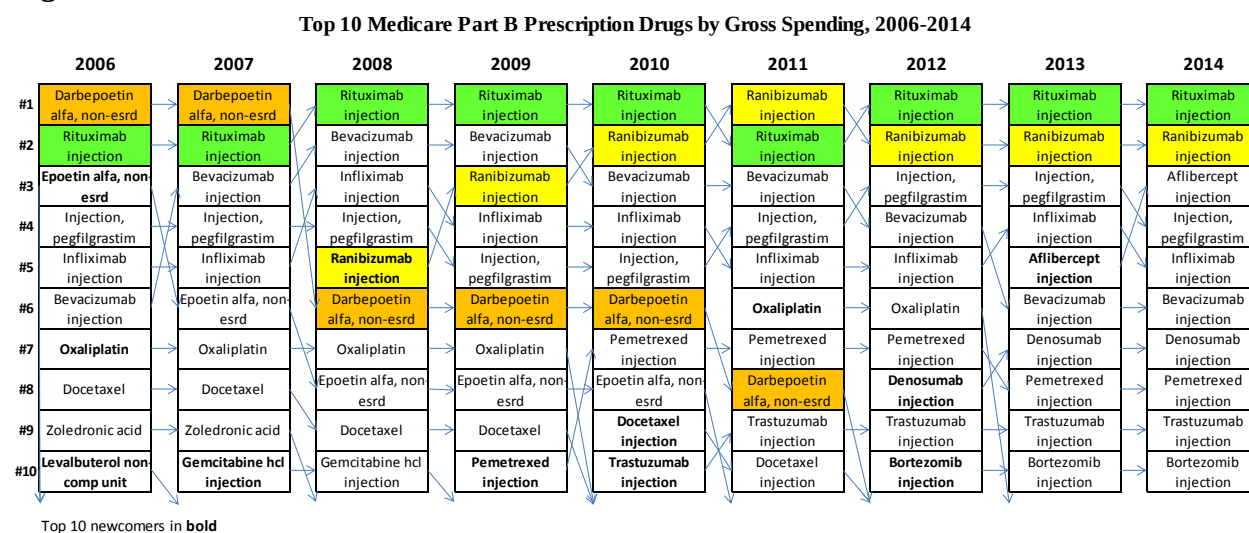
HCPCS code	HCPCS Description	Total Payment (\$Millions)	Medicare Payment (\$Millions)	Number of Beneficiaries (Thousands)	Number of Services (Thousands)	Medicare Spending per Beneficiary (\$)	Medicare Spending per Service (\$)	Estimated Cost Sharing - Total (\$M)	Estimated Cost Sharing - per Beneficiary (\$)
J9310	Rituximab injection	1,550.4	1,242.9	70	291	17,638.4	4,266.3	310.1	4,429.7
J2778	Ranibizumab injection	1,337.8	1,064.8	142	676	7,498.3	1,574.6	267.6	1,884.2
J0178	Aflibercept injection (ophthalmic)	1,302.2	1,036.2	133	627	7,791.6	1,651.4	260.4	1,958.2
J2505	Injection, pegfilgrastim 6mg	1,235.5	974.3	102	373	9,529.6	2,609.6	247.1	2,422.5
J1745	Infliximab injection	1,223.2	965.4	61	360	15,697.9	2,682.0	244.6	4,010.5
J9035	Bevacizumab injection	1,090.7	879.7	217	907	4,054.9	969.6	218.1	1,005.3
J0897	Denosumab injection	798.8	629.4	306	670	2,059.1	939.0	159.8	522.1
J9305	Pemetrexed injection	575.5	463.7	24	107	19,725.6	4,322.9	115.1	4,795.8
J9355	Trastuzumab injection	580.5	463.6	19	198	24,418.9	2,344.3	116.1	6,110.5
J9041	Bortezomib injection	488.8	386.9	21	328	18,545.2	1,179.7	97.8	4,655.2
J2353	Octreotide injection, depot	354.8	281.7	11	90	25,708.8	3,138.0	71.0	6,450.9
J0129	Abatacept injection	351.8	277.7	21	172	13,497.4	1,613.7	70.4	3,350.5
J9033	Bendamustine injection	312.6	252.0	14	88	18,396.2	2,870.3	62.5	4,465.7
J0885	Epoetin alfa, non-esrd	317.6	248.7	98	804	2,538.0	309.2	63.5	648.2
J9228	Ipilimumab injection	271.9	242.1	3	8	82,537.0	29,389.4	54.4	18,126.7
J0881	Darbepoetin alfa, non-esrd	308.6	241.3	66	387	3,654.2	623.6	61.7	935.2
J9264	Paclitaxel protein bound	283.5	225.1	18	143	12,462.9	1,574.1	56.7	3,150.0
J1569	Gammagard liquid injection	268.0	211.6	13	90	16,911.9	2,350.2	53.6	4,123.1
J9055	Cetuximab injection	266.0	211.0	10	94	21,800.2	2,233.9	53.2	5,320.0
J2323	Natalizumab injection	266.1	210.9	7	63	28,177.7	3,373.4	53.2	7,602.9
TOP 10		10,183.4	8,106.9					2,036.7	
All Part B Drug Payments		21,576.0	17,221.4					4,315.2	
Top 10% of payments for ALL Part B drugs*		47.2%	47.1%					47.2%	

Source: Analysis of carrier, durable medical, and outpatient claims data 2006-2014 by Acumen for ASPE

Data include Part B covered drugs administered in physicians' offices and furnished by suppliers, covered drugs in hospital outpatient departments; and reflect only Part B drugs paid under the average sales price plus 6 percent (ASP).

The Healthcare Common Procedure Coding System (HCPCS) codes and prices for carrier and DM were obtained from the CMS ASP file, those for OP come from the CMS Addendum B file. Lines with denied payments or Medicare as secondary payer were dropped. Medicare total payments include Medicare program payments and beneficiary cost sharing and include the sequester. The analyses started in 2006 when most Part B drugs in Outpatient departments were paid under ASP.

Figure 2



Summary

Medicare makes payments directly to physicians and hospital outpatient departments for Part B drugs administered to beneficiaries. In both sites of service, payments are based on ASP plus 6 percent. The payments reflect average transaction prices from 6 months prior.

Between 2006 and 2014, Medicare Part B prescription drug spending increased from \$10.1 billion to \$17.2 billion. The average growth rate in spending was 4.5 percent annually between 2006 and 2009, and then spending grew to 8.4 percent annually between 2009 and 2014. Increases in spending in the Medicare Part B program have been driven by increases in biologics, which grew by 13.3 percent annually between 2006 and 2014. In 2014, biologics accounted for 63 percent of prescription drug spending in Part B.

In 2014, spending in Medicare Part B was dominated by cancer drugs (45 percent) and drugs that treat rheumatoid arthritis (8.7 percent), intravenous immunoglobulin (IVIG) preparations (5.2 percent), and osteoporosis (4.3 percent). These four classes were also the dominant therapeutic classes for spending in 2006. Concentrated spending for a relatively small number of drugs has been consistent for the past decade: the top ten drugs account for nearly half (47 percent) of total Medicare Part B spending in 2014.

The incentives associated with the current ASP plus 6 percent payment system are generally not consistent with the provision of high value care to beneficiaries. The independence of payment from the transaction prices paid by providers may successfully encourage providers to obtain the lowest possible prices for their drugs. For high cost drugs that do not have therapeutic alternatives, this method may have some beneficial effect in slowing growth in Medicare payments.²² However, for drugs where therapeutic alternatives are available, the current system may encourage the use of higher price drugs (higher price clinically equivalent, single source drug) when lower cost drugs of equivalent effectiveness are available.

²² Although this beneficial effect might be counter-balanced by the potential lack of competition.

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CHAPTER 4: MEDICARE PART D

This chapter provides an overview of the Medicare Part D program, including benefit structure, financing, and cost containment measures. Trends in drug costs and utilization, in general and for specialty drugs, generics, and therapeutic classes are presented. In addition, the top 10 drugs by spending are detailed. These results are estimated from quantitative analyses conducted specifically for this report.

For many of the analyses contained in this chapter, measures of drug spending are constructed from Part D claims (PDEs) to include payments to the pharmacy by the Part D plan sponsor and the beneficiaries' out of pocket liability. These measures are referred to as gross drug costs. In some cases we estimate Medicare program spending for Part D which differs from gross drug costs to the extent that rebates and other price considerations affect plan premiums but are not reflected in prices paid at the pharmacy.

Key findings

- Medicare Part D gross drug costs increased from \$61.9 billion in 2007 to \$121.0 billion in 2014. Between 2007 and 2012, annual increases were 7.7 percent. Increases were 16.3 percent between 2012 and 2014.
- Between 2007 and 2014, spending on specialty tier eligible drugs increased from \$6.1 billion to \$35.9 billion. Spending increased faster than did utilization: the average annual growth rates for spending was 29 percent as compared with 15 percent for utilization, implying that price increases are responsible for half the growth in spending.
- Spending on biologics in Medicare Part D for high-cost enrollees grew 91 percent from 2009 to 2012, from \$1.9 billion to \$3.5 billion. In this same time, the number of prescriptions for biologics for high-cost enrollees grew only 32 percent, from 1.1 million to 1.5 million [1].
- Spending in Medicare Part D was highest for antihyperlipidemics, antipsychotic/antimanics, antihypertensives, antiasthmatic and antidiabetics between 2007 and 2014. Antihypertensives and antihyperlipidemics consistently had the highest utilization in all years.
- In Medicare Part D, the top 10 drugs by gross spending accounted for about 20.4 percent of total gross drug cost in 2014. This proportion has been relatively stable since 2007 (21.5 percent), thanks partly to two key opposing forces. On the one hand, generic entry of a blockbuster drug (such as Atorvastatin's entry in late 2011 to compete against Lipitor) decreased the top 10 share of spending. On the other hand, entry of new expensive drugs that made into the top 10 raised the top 10 share of spending. For example, Solvadi entered the market late in 2013 and moved into the top ten list with a relatively small number of claims and users.
- In the Medicare Part D program, generics increased from 55.9 percent of filled prescriptions in 2007 to 78.1 percent in 2014. As a percentage of gross spending, generics increased from 19.3 percent to 23.1 percent over the same period.

- Total drug cost in the catastrophic phase (the highest spending phase in Medicare part D) increased over 20 percent annually on average between 2007 and 2014, implying substantial unit price increases given that the corresponding number of claims in the catastrophic phase grew much less at 7.3 percent annually on average.
-

Program Overview

Medicare Part D is a component of the Medicare program, a federal health insurance program created in 1965 for people ages 65 and older, regardless of income and health conditions. The Medicare program was expanded in 1972 to cover people under age 65 with permanent disabilities. Authorized by the Medicare Modernization Act of 2003 (MMA), Part D was implemented in January 2006 as a voluntary drug benefit for Medicare beneficiaries. Private plans compete for enrollees by providing and managing the drug benefit.²³

Thus, Medicare consists of four parts (A, B, C, and D) covering (A) hospitalizations, skilled nursing facility care, hospice care, and some home health visits; (B) outpatient and physician services including drugs administered in physician offices, and some home health visits; and (D) outpatient prescription drugs. While (C) is the Medicare Advantage (MA) alternative for A and B benefits, most MA plans also cover the Part D benefit (MA-PD). In 2015, total enrollment in Medicare was 55.3 million, of which enrollment in D was 41.8 million [2]²⁴. Each enrollee in either Part A or Part B is also entitled to enroll in a Part D prescription drug plan. Similar to Part B, enrollment in Part D is voluntary and the enrollee pays a monthly premium.²⁵

Under Part D, private plan sponsors submit annual premium bids for providing the benefit. Medicare subsidizes 74.5 percent of the national average premium and provides additional assistance for premiums and out of pocket costs to low income beneficiaries, the low income subsidy (LIS) [3]. In CY2015, total Medicare benefit payment is \$639 billion, of which, \$90 billion (or 14 percent) is for the Part D benefit²⁶ [2].

In CY2016, about 43 million people are expected to enroll in a stand-alone Prescription Drug Plan (PDP) or a Medicare Advantage Prescription Drug Plan (MA-PD plan).²⁷ Enrollees in Part D pay a monthly premium in addition to cost sharing and any deductible for their drugs. Low-income beneficiaries (LIS) pay lower or no premiums, cost sharing, or deductibles.

²³ The Medicare Part D drug benefit is administered through private prescription drug plans, which each separately design and manage benefits and pay claims. Private prescription drug plans use purchasing arrangements and utilization management, including negotiation of prices with manufacturers and pharmacies, formularies, step therapy, quantity limitations, and prior authorization. All formularies must include “all (with specified exceptions)” drugs in the immunosuppressant, antidepressant, antipsychotic, anticonvulsant, antiretroviral, and antineoplastic classes to ensure patient access to these protected classes of drugs. The current exceptions are that the formulary does not have to include all therapeutic equivalents (i.e., generics) and can use safety edits to limit quantities (see 42 CFR 423.120(b)(2)(vi)).

²⁴ See Table II.B.1, p. 10

²⁵ Starting in 2011, higher income enrollees pay higher premiums, as in Part B.

²⁶ Medicare Trustees Report 2016, Table II.B1, p. 10.

²⁷ Medicare Trustees Report 2015, Table IV.B7, p. 145.

Benefit structure

The Part D standard benefit for 2016 includes a \$360 deductible, 25 percent coinsurance for covered drug spending between \$360 and \$3,310, and variable coinsurance for drug spending between \$3,310 and an estimated \$7,515 (a gap in coverage known as the “donut hole”). After \$4,850 in beneficiary true out-of-pocket (or “TrOOP”)²⁸ spending is reached, catastrophic coverage begins and beneficiaries generally are responsible for 5 percent of their drug costs for the remainder of the year, while plans cover 15 percent, and Medicare the other 80 percent of cost. All drug plans must offer a standard benefit plan or an actuarially equivalent benefit plan [5].

The Affordable Care Act (ACA), formally the Patient Protection and Affordable Care Act as amended by the Health Care and Education Reconciliation Act of 2010, reduced beneficiary cost sharing in the coverage gap until the gap is closed in 2020. In 2016, beneficiaries pay 45 percent of the cost for brand name drugs and 58 percent of the cost for generic drugs in the coverage gap. In 2020 and thereafter, beneficiary cost sharing in the coverage gap for brand name and generic drugs will be 25 percent [6].

Table 1

Subsidies Provided by Manufactures and Medicare to Medicare Part D Enrollees for closing the donut hole 2011-2020 and beyond				
		Brand Name		Generic
		Manufacturers	Medicare	Medicare
2010		0%	0%	0%
2011		50%	0%	7%
2012		50%	0%	14%
2013		50%	2.5%	21%
2014		50%	2.5%	28%
2015		50%	5%	35%
2016		50%	5%	42%
2017		50%	10%	49%
2018		50%	15%	56%
2019		50%	20%	63%
2020 and beyond		50%	25%	75%
In 2010, each beneficiary entering the gap receives \$250 rebate check in lieu of subsidies that started in 2011.				

²⁸ Not all drug spending counts toward TrOOP. Actual out-of-pocket spending by the beneficiary counts toward TrOOP, but payments made by other insurers or third parties generally do not count toward TrOOP.

Cost containment efforts

The Medicare drug benefit is administered through private entities²⁹ called prescription drug plans (PDPs) for beneficiaries in fee-for-service (also known as original or traditional) Medicare and through Medicare Advantage prescription drug (MA-PD) plans for beneficiaries enrolled in Medicare managed care. PDPs and MA-PDs perform such functions as: (1) designing and marketing drug benefit plans, (2) negotiating drug prices with manufacturers and pharmacies, (3) building and managing a network of pharmacies, (4) paying claims, (5) enrolling and disenrolling beneficiaries, (6) managing a drug formulary and beneficiary appeals process, and (7) tracking beneficiary drug spending.

The competition among Part D plans is based in part on tying the premium subsidy to the national average of plan bids. Plans with above average costs must then charge additional premiums while those below the average can reduce the beneficiary premium. Thus, plans compete based both on their premiums and on the quality of their benefit package. Within some limitations based on actuarial value and formulary guidance, plans have considerable latitude for varying deductible and copayment structure of the benefit, as well as other drugs to be included in the benefit package. Like most commercial plans, Part D plans use formularies to manage drug costs and utilization. Although CMS has a number of requirements for these formularies, plans can change formularies throughout a year in response to new drugs entering the market, new clinical knowledge, or other market changes. As drug prices increase, plans may respond by decreasing the generosity of their formulary offerings. The resulting impact on beneficiaries can be a reduction of access to drugs and/or an increase in drug costs. For example, to lower their costs, plans can decide to only cover the CMS-required minimum number of drugs per class, or restrict coverage to mostly generic drugs.

As noted in Chapter 7, plans can negotiate prices with manufacturers and implement several benefit management practices to control costs. They can use tiered copayments to provide beneficiaries with financial incentives for choosing higher value alternatives within therapeutic classes—either a generic equivalent to branded drugs or to competing brands that are favored due to the plan negotiating a better price, usually through rebate arrangements. Other benefit management practices, such as step therapy, quantity limitations, and prior approval can also be used to encourage higher value utilization. Plans incorporate a number of utilization management strategies in their formularies to help control access and cost. They can require plan enrollees to obtain prior authorization before covering certain drugs, or set limits on the quantities of drugs that can be filled for each enrollee. A common policy is requiring step therapy, where a drug is covered only after other less expensive treatments have proven to be ineffective for treating an enrollee's health condition. These utilization management strategies are often directly linked to the use of tiers within a formulary. Most Part D plans' formularies organize drugs into separate tiers, with varying levels of cost-sharing which may encourage plan enrollees to choose drugs in certain tiers over others.

²⁹ Employers and unions offering retiree coverage that is at least as generous as Medicare's drug benefit may qualify for retiree drug subsidies (RDS) to help with the cost of providing a drug benefit to their Part D-eligible retirees.

Financing

Part D is financed through general revenues, beneficiary premiums, and state payments for dual eligibles. The transfers from states are referred to as “clawback payments,” and represent a portion of the amounts states could otherwise have been expected to pay for drugs under Medicaid if drug coverage for the dual eligible population had not been transferred to Part D. Part D revenues are credited to a separate Part D account within the SMI trust fund.

In 2016, the base beneficiary premium is \$34.10 [7]; however, beneficiaries pay different premiums depending on the plan they have selected and whether they are entitled to low-income premium subsidies.³⁰ Premiums for the Part D program are required to cover 25.5 percent of standard benefit costs (the sum of the national average monthly bid amount and the estimated catastrophic reinsurance). However, as recipients of the Part D low-income subsidies are not required to pay premiums, premiums covered only about 12.5 percent of Part D program costs in 2015.³¹ Additionally, beginning in 2011 as required by the ACA, higher income Part D enrollees pay higher premiums similar to high-income Part B enrollees.³²[8].

In CY2016, total net spending for Part D is estimated to reach approximately \$102.5 billion, with about \$84.9 billion of that amount paid for by general revenues, \$13.8 billion from beneficiary premiums, and \$9.6 billion from state transfers [2]³³.

Drug Rebates: Program or Net Spending vs. total Gross Drug Cost

Medicare Part D program spending per enrollee (net of rebates)³⁴ has been relatively stable since the program inception in 2006, rising about 2.8 percent annually from 2006 to 2014 [2]³⁵. As the number of enrollees increased about 3.6 percent annually, total spending increased 6.4 percent during the same period.³⁶

³⁰ There is no “hold harmless” provision under Part D similar to that under Part B. Part D premium increases are not affected by Social Security cost-of-living adjustments.

³¹ See Table IV.B10, p. 149 of the Trustees Report 2016

³² The income thresholds are set at the same levels as those under Part B and frozen in the same manner through 2019, as described in Chapter 3.

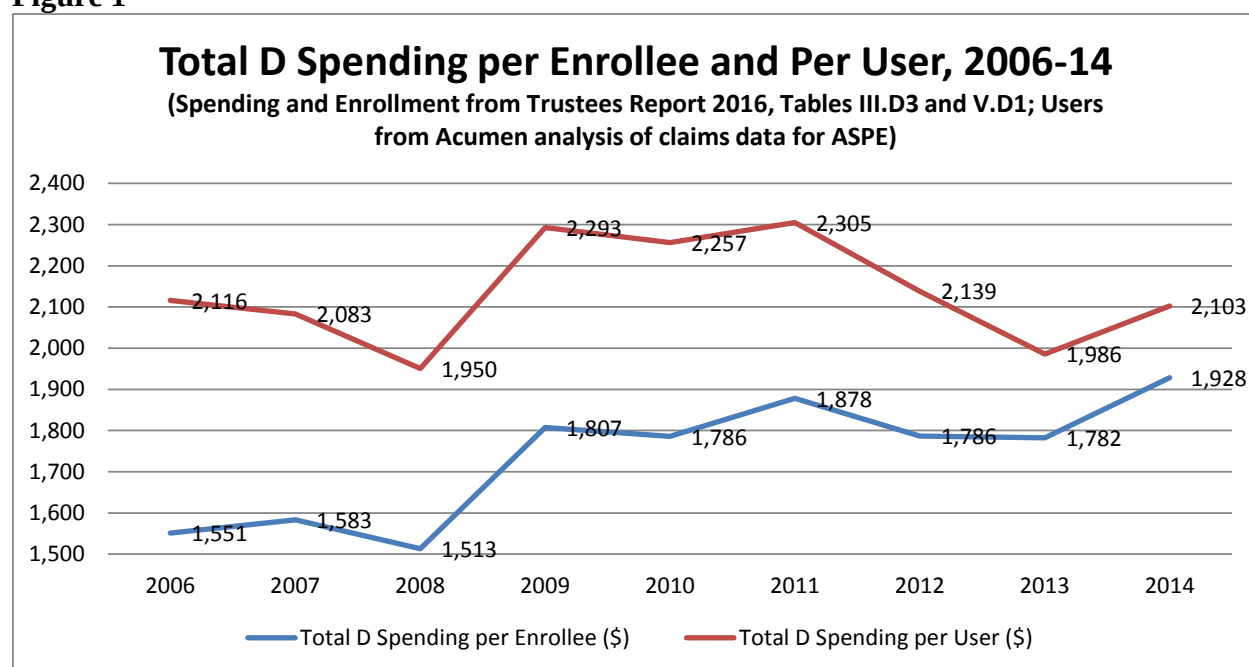
³³ See Table III.D3, p.107

³⁴ Federal spending (Medicare Part D net program spending) is based on a percent of premiums which in turn reflect the rebates plans get.

³⁵ Annual compound growth rate of total program spending per enrollee and enrollment growth computed from United States Centers for Medicare & Medicaid Services. Medicare Trustees Reports, 2016. June 2016. Part D total spending from Table III.D3 (p. 107) and Part D enrollment from Table V.B4 (p. 186).

³⁶ As will be presented below, during 2007 to 2014, total Part D rebates rose 16.5 percent annually, increasing from \$5.9 billion in 2007 to about \$17.3 billion in 2014. The increase in rebates had contributed to the relatively stable program spending per enrollee annual increase of 2.9 percent during 2007-14, while total gross drug costs per enrollee increased 6.1 percent over the same period.

Figure 1



In 2014, total program spending was estimated to be \$78.1 billion, while net benefit spending was \$77.7 billion (Table 2) [2]. The difference reflects factors including plan sponsors' administrative costs.

Table 2

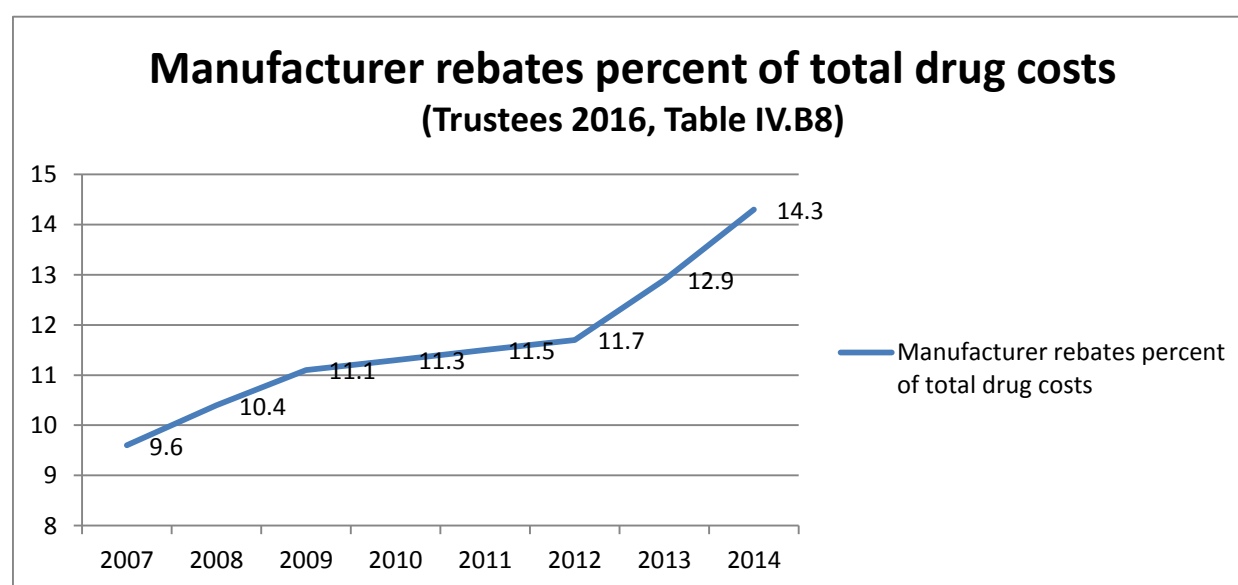
Medicare Part D Total Program Spending and Benefit Spending, 2006-2014										
Year	2006	2007	2008	2009	2010	2011	2012	2013	2014	Annual 2006-14
Part D Total Spending (\$B) *	47.4	49.7	49.3	60.8	62.1	67.1	66.9	69.7	78.1	6.4%
Part D Benefits Spending (\$B) *	47.1	48.8	49.0	60.5	61.7	66.7	66.5	69.3	77.7	6.5%
Total Part D Enrollees (M) *	30.6	31.4	32.6	33.6	34.8	35.7	37.4	39.1	40.5	3.6%
Total D Spending per Enrollee (\$)	1,551	1,583	1,513	1,807	1,786	1,878	1,786	1,782	1,928	2.8%
* United States Centers for Medicare and Medicaid Services. Medicare Trustees Reports, 2016. June 2016.										
https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/ReportsTrustFunds/downloads/tr2016.pdf										
Total spending and benefits spending from Table III.D3; D enrollment from Table V.B4										

Given that the total drug cost obtained from the 2014 claims data was over \$121 billion; this implies that Medicare spending (\$78.1 billion, net of rebates) was about 65 percent of the gross drug cost in 2014.³⁷ This difference reflects cost sharing in addition to rebates.

³⁷ Over the 2006-14 period, Medicare net spending is about 67 percent of the GDC on average (internal communication)

In fact, total rebates reported for Medicare Part D were over \$17 billion, accounting for over 14 percent of total drug cost in 2014. Rebates negotiated by prescription drug plans have been increasing over time, from 9.6 percent of total drug costs in 2007 to 14.3 percent in 2014.³⁸

Rebates for brand named drugs are much higher (17.5 percent of the GDC) than for generics (0.15 percent) in 2014 as a result of the ACA closing the Part D coverage gap, also known as the donut hole.³⁹



Because rebate data are proprietary and not publicly available in detail, the following analyses are based on total gross drug cost (GDC) data that are available on the claims data. The gross drug cost is the total cost of the drug before rebates, and includes administrative costs and cost sharing.⁴⁰

Medicare Part D Spending and Spending Trends

The following sections describe the Medicare claims files that were included in the analyses of trends in overall spending as well as trends in specialty drugs, top ten drugs in terms of spending, top therapeutic classes, and generics. Methods for identifying specialty drugs, therapeutic classes, and generic drugs are also described.

Data and Methods

Medicare Part D prescription drug events (PDE) data were used from 2007 to 2014 to calculate annual total gross drug costs, price, and utilization. Although the Medicare Part D program

³⁸ Trustees Report 2016; Table IV.B8, p. 147.

³⁹ The brand-name and generic shares are based on CMS's analysis of the manufacturer data for the 2014 reconciliation for ASPE's Prescription Drug Report To Congress

⁴⁰ The gross drug cost (GDC) obtained from Medicare claims represents data "available under current law that is not proprietary" as specified in the statutory provision requiring this report.

started in 2006, data in the initial year are not considered reliable for analyses. As a result, 2007 was the first year used for evaluating trends.

The analysis is based on all drugs reported in the PDE data, excluding non-covered claims and compound drugs. Additional information was obtained from the Health Plan Management System (HPMS), Medispan and First Data Bank.⁴¹

The unit of analysis for the study is the National Drug Code (NDC), a unique product identifier. The NDC is a unique 10-digit, 3-segment numeric identifier assigned to each medication identifying the labeler or vendor, product (specific strength, dosage form, and formulation for a particular firm), and trade package (package forms and sizes).

Measures

A drug is categorized in the Specialty Tier if (1) the NDC exceeds the specialty tier monthly cost eligibility threshold in the year, or (2) has the same Drug ID and Brand Name as a drug on any plan's approved specialty tier formulary in the year. The 30 day equivalent cost threshold is \$500 in 2007 and \$600 in years 2008-2014.

Therapeutic classes were identified based on Medispan data. The classes include the Part D protected classes that currently include immunosuppressants, antidepressants, antipsychotics, anticonvulsants, antiretrovirals, and antineoplastics.

Generics and brand name drugs were defined based on the First Data Bank and Medispan data.⁴²

Trends in Gross Drug Costs and Utilization

In 2014, total gross drug cost (GDC) for the Medicare Part D drugs is estimated to be \$121 billion (Table 4).⁴³ This reflects a 17 percent increase from the previous year's \$103.3 billion in GDC.⁴⁴

⁴¹ The Health Plan Management System (HPMS) provides such information as plan type, cost share tier level, and utilization management (quantity limit, prior authorization, step therapy). The First Data Bank (FDB) and Medispan provide such information as generic and brand name, dosage form, strength, and route of administration. In addition, Medispan provides information on drug class and protected class.

⁴² Specifically, a drug is classified as brand name if its FDA marketing category is one of the following: NDA, BLA, NDA Authorized Generic. A drug is identified as generic if FDA marketing category is ANDA.

⁴³ Estimate based on Medicare Part D events (PDE) 2007-14 files by Acumen for ASPE

⁴⁴ MedPAC estimated \$103.6 billion for 2013 (compared to ASPE's \$103.3 billion) in its June 2015 Data Book, Chart 10-22, p. 176.

Table 4

Medicare Part D Prescription Gross Drug Costs (GDC), Users, Days and Scripts: 2007-2014											
										annual change	
	2007	2008	2009	2010	2011	2012	2013	2014	2007-12	2012-14	2007-14
Total Gross Drug Cost (\$B)	\$61.9	\$68.2	\$73.5	\$77.4	\$84.6	\$89.5	\$103.3	\$121.0	7.7%	16.3%	10.1%
Annual change (%)		10.2%	7.8%	5.3%	9.3%	5.8%	15.4%	17.1%			
Users (M)	23.9	25.3	26.5	27.5	29.1	31.3	35.1	37.1	5.6%	9.0%	6.5%
Annual change (%)		5.9%	4.9%	3.8%	5.8%	7.5%	12.2%	5.8%			
Drug Cost Per User (\$)	\$2,594	\$2,699	\$2,773	\$2,813	\$2,908	\$2,862	\$2,944	\$3,258	2.0%	6.7%	3.3%
Annual change (%)		4.1%	2.7%	1.5%	3.4%	-1.6%	2.9%	10.7%			
Scripts (B)	0.96	1.03	1.07	1.10	1.15	1.21	1.37	1.41	4.6%	8.1%	5.6%
Annual change (%)		6.8%	4.0%	3.1%	4.1%	5.2%	13.1%	3.4%			
Drug Cost Per Script (\$)	\$64.3	\$66.4	\$68.8	\$70.3	\$73.8	\$74.1	\$75.7	\$85.7	2.9%	7.5%	4.2%
Annual change (%)		3.2%	3.6%	2.2%	5.0%	0.5%	2.1%	13.3%			
Days (B)	30.93	34.09	36.53	38.51	41.51	45.13	52.56	55.86	7.9%	11.3%	8.8%
Annual change (%)		10.2%	7.2%	5.4%	7.8%	8.7%	16.5%	6.3%			
Drug Cost Per Day (\$)	\$2.00	\$2.00	\$2.01	\$2.01	\$2.04	\$1.98	\$1.97	\$2.17	-0.2%	4.5%	1.1%
Annual change (%)		0.0%	0.6%	-0.1%	1.4%	-2.7%	-0.9%	10.2%			
Source: Analysis of Medicare Part D Events data 2007-2014 by Acumen for HHS/ASPE											

Over the entire 2007-14 period, total gross drug cost increased by 10.1 percent annually, while Medicare benefit spending (net of rebates and cost-sharing) grew at 6.5 percent. The divergence likely reflects growing price concessions over time [9].

Spending growth, however, has been higher recently. During 2007-12, total gross drug cost increased at an annual rate of 7.7 percent, then, jumped to 16.3 percent annually between 2012 and 2014 (Figure 2).

The recent surge in total GDC is driven by both increases utilization (number of users, scripts, and days) (Figure 3) as well as unit cost (per user, per script, and per day). In 2014, however, the unit average price accelerated more rapidly than utilization. The number of users increased by 5.8 percent, but cost per user increased by 10.7 percent.

Figure 2

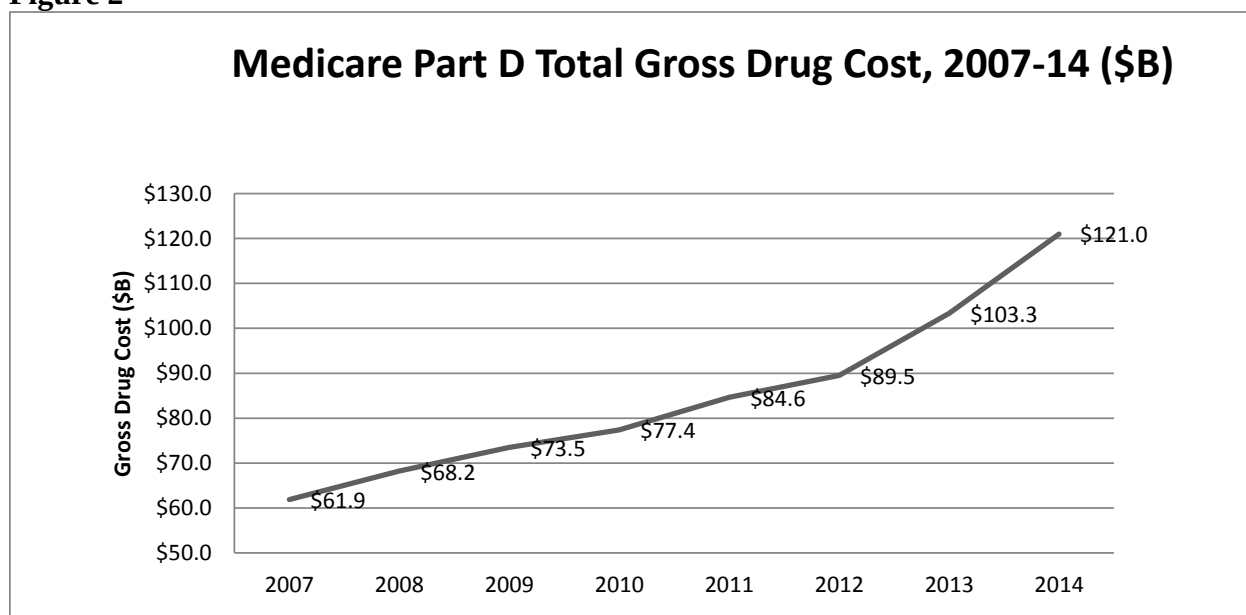
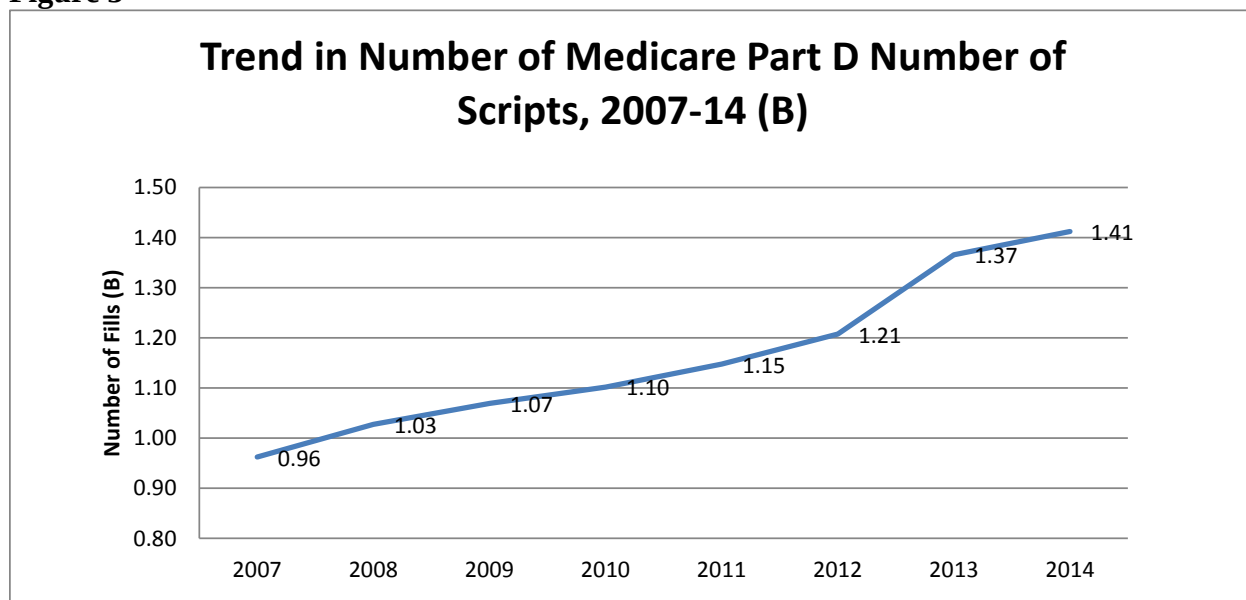


Figure 3



Specialty drugs and biologics

Between 2007 and 2014, spending on specialty tier eligible drugs increased by a factor of 6, from \$6.1 billion to \$35.9 billion (Table 5). The number of filled prescriptions for these specialty drugs, however, only increased by a factor of 2.7 during this time period, from 5.5 million to 15.1 million. Spending increased faster than did utilization: the average annual growth rates for spending was 29 percent as compared with 15.4 percent for number of fills.

Table 5

Medicare Part D Utilization and Total Gross Drug Cost: SPECIALTY Tier Eligible					
		Utilization (M)			Expenditures (\$B)
	Total Fills	Total Days Supply	Unique Users		Total GDC
2007	5.5	143.4	1.0		6.1
2008	5.2	144.7	0.8		7.0
2009	6.4	175.0	1.0		9.0
2010	7.7	216.0	1.1		11.2
2011	8.8	253.0	1.2		14.2
2012	8.9	261.5	1.3		17.2
2013	11.6	343.6	1.7		23.5
2014	15.1	452.2	2.1		35.9
Annual 2007-14	15.4%	17.8%	11.5%		29.0%
Source: Analysis of Medicare Part D events data by Acumen for ASPE					

Biologics are a type of specialty drug. According to a MedPAC analysis of Part D prescription drug event data, spending on biologics in Medicare Part D for high-cost enrollees grew 91 percent from 2009 to 2012, from \$1.9 billion to \$3.5 billion. In this same period, the number of prescriptions for biologics for high-cost enrollees grew only 32 percent, from 1.1 million to 1.5 million [1].

Top therapeutic classes

Between 2007 and 2014, spending was highest for antihyperlipidemics, antipsychotic/antimanics, antihypertensives, antiasthmatic and antidiabetics. These top 5 drug groups accounted for 35 percent of prescription drug spending in 2007, and 34 percent of in 2014.

The two therapeutic classes with the highest utilization consistently between 2007 and 2014 were antihypertensives and anti hyperlipidemics, both of which accounted for approximately 17 percent of prescription drug fills during this time period.

Table 6

Top 10 Drug Group by Gross Drug Cost (GDC) in 2014								
Drug Group (Ranked by 2014 GDC)	2007				2014			
	Claims (M)	Gross Drug Cost (\$B)	GDC per Claim (\$)	GDC per Bene (\$)	Claims (M)	Gross Drug Cost (\$B)	GDC per Claim (\$)	GDC per Bene (\$)
ANTIDIABETICS	58.9	4.0	68	765	80.9	14.1	174	1,635
ANTIASTHMATIC AND BRONCHODILATOR AGENTS	28.5	3.0	105	734	43.5	8.3	190	1,186
ANTIHYPERTENSIVES	75.8	5.9	77	542	115.1	7.6	66	385
ANTIPSYCHOTICS/ANTIMANIC AGENTS	21.9	5.0	228	2,155	28.3	6.3	222	2,085
ANTIHYPERTENSIVES	93.3	3.8	40	317	121.8	4.3	36	223
ULCER DRUGS	43.9	4.0	92	558	72.4	4.4	61	349
ANALGESICS - OPIOID	52.7	2.2	42	244	82.2	4.3	52	304
*PSYCHOTHERAPEUTIC AND NEUROLOGICAL AGENTS - MIS	13.2	2.5	192	1,425	19.9	7.0	354	3,117
ANTICONVULSANTS	25.7	2.8	109	851	58.7	3.8	64	491
ANTIDEPRESSANTS	53.5	2.6	49	397	84.5	3.3	39	297

Source: Analysis of Medicare claims data (carrier, outpatient, and Part D event) by Acumen for ASPE

Top 10 Drugs by Total Spending and by Number of Claims

In both 2013 and 2014, the top 10 drugs by gross spending account for about 20 percent of total gross drug cost. Sovaldi entered the market in the last 2 months of 2013 and quickly took second place of the list in 2014, although with only 109 thousand claims and 33 thousand users (Figures 4 and 5). Costing over \$28 thousand per claim or \$1,000 per day, gross cost for Sovaldi alone is estimated to be \$3.1 billion in 2014. In addition, Medicare beneficiaries using these high cost drugs would face high patient liabilities. Despite the catastrophic coverage built in Part D, a Medicare user of Sovaldi is expected to incur over \$6,500 in out of pocket expenses for that drug.

Lantus, an antidiabetic, drug, tops the list in terms of total gross cost, costing \$3.7 billion. However, with more than 8 million claims and 1.7 million users, a user of Lantus is expected to incur \$676 in liabilities (Figures 4 and 5).

Figure 4

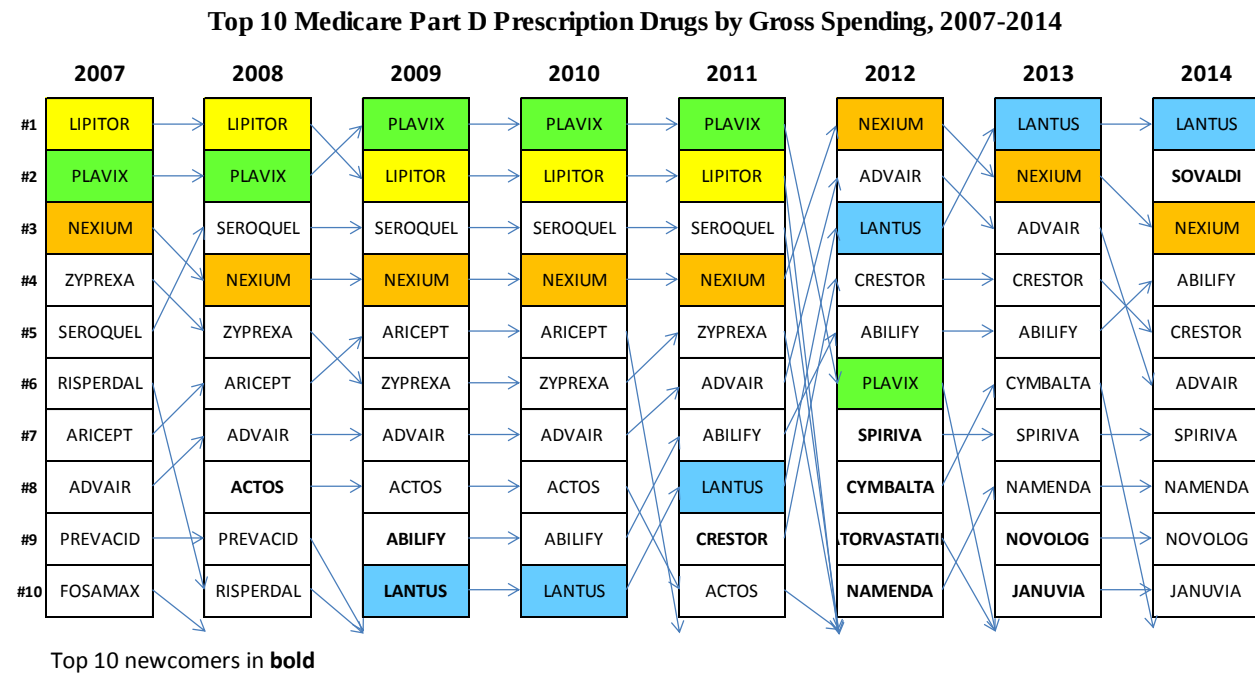
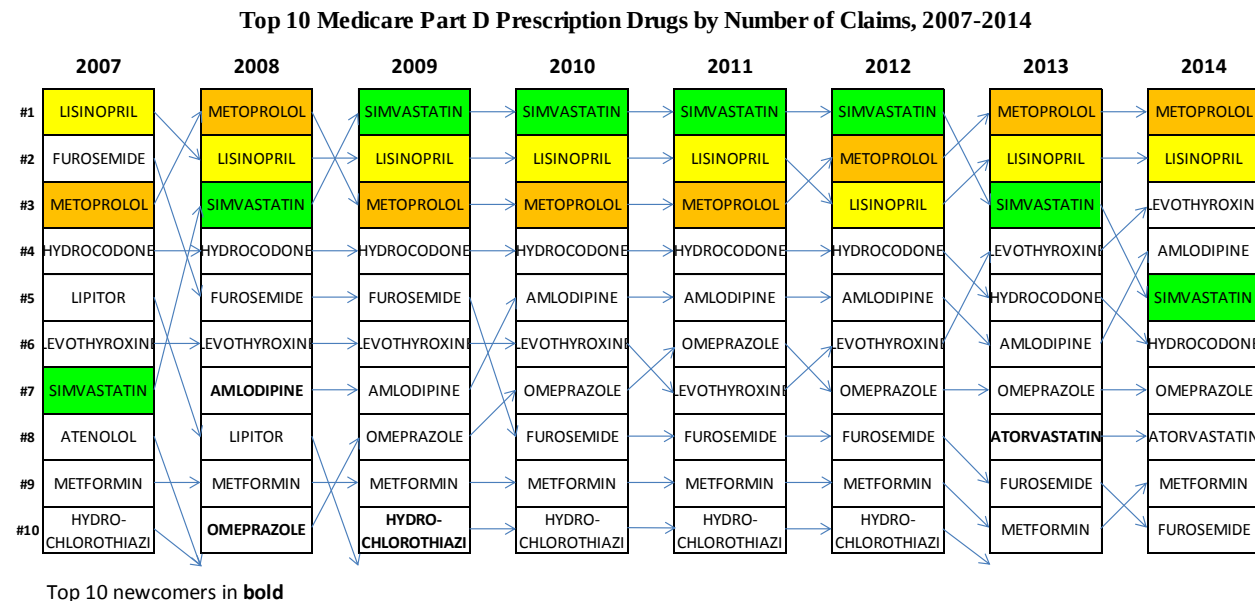


Figure 5



Generics

Generics increased from 55.9 percent of filled prescriptions in 2007 to 78.1 percent in 2014, representing an average annual growth of 4.9 percent. However, annual growth rates in the number of fills of generics declined from 19.7 percent in 2008 to 5.4 percent in 2014. The share of spending allocated to generic drugs has increased from 19.3 percent in 2008 to 23.1 percent in 2014. Spending on generic drugs was higher in 2013, when generic drugs accounted for 24.7 percent of gross spending (Table 6).

Table 7

Medicare Part D GENERIC Dispensing Rate and Spending Share

	Generic Share of Total Fills	Annual growth of Total Fills	Expenditures Share Total GDC
2007	55.9%		19.3%
2008	61.5%	19.7%	21.5%
2009	63.7%	9.8%	21.4%
2010	67.0%	9.1%	21.5%
2011	70.4%	9.7%	22.0%
2012	74.0%	10.8%	23.8%
2013	76.7%	17.1%	24.7%
2014	78.1%	5.4%	23.1%
Annual 2007-14	4.9%		2.6%

Source: Analysis of Medicare Part D events data by Acumen for ASPE

Utilization and Spending in the Catastrophic Phase

Total drug cost in the catastrophic phase (the highest spending phase in Medicare part D) increased over 20 percent annually on average between 2007 and 2014, implying large unit price increases given that the corresponding number of claims in the catastrophic phase grew substantially less at 7.3 percent annually on average (Table 7).

Table 8

Medicare Total Drug Cost, Number of Claims and Beneficiaries in The Catastrophic Phase

Year	Claims: Number (M)	Claims: Annual Change	Enrollees: Number (M)	Enrollees: Annual Change	Total GDC (\$B)	GDC: Annual Change
2007	84.1		2.3		11.0	
2008	91.8	9.1%	2.4	5.3%	13.2	19.5%
2009	90.8	-1.1%	2.4	-2.0%	14.2	8.0%
2010	91.8	1.1%	2.4	0.4%	15.8	10.8%
2011	102.7	11.8%	2.7	11.8%	19.5	23.8%
2012	103.3	0.6%	2.6	-2.3%	21.9	12.2%
2013	113.5	9.9%	2.8	9.6%	27.7	26.1%
2014	137.4	21.1%	3.4	20.3%	39.9	44.2%
Annual compound rate	7.3%		5.9%		20.1%	

Source: Analysis of Medicare Part D Events data 2007-2014 by Acumen for HHS/ASPE

Summary

Medicare Part D began providing oral prescription drug coverage to Medicare beneficiaries in 2006. Medicare Part D gross drug costs, which include payments from Part D plans and beneficiaries, nearly doubled between 2007 (\$61.9 billion) and 2014 (\$121.0 billion). Annual spending increases were 7.7 percent in the first five years of this period (2007-2012), but accelerated to 16.3 percent annually between 2012 and 2014. The sharp increase in 2014 drug spending is not solely due to new high-spending entrants like Sovaldi. Many existing drugs also experienced substantial price rise: the average Part D cost per day for Lantus, the top drug on

both the 2014 and 2013 lists, rose by 31 percent (from \$8.33 in 2013 to \$10.95 per day in 2014). Similarly, the average Part D cost per day for Nexium, 3rd on the 2014 list and 2nd on the 2013 list, rose by 12 percent from \$7.65 in 2013 to \$8.54 per day in 2014.

Between 2007 and 2014, spending on specialty tier eligible drugs increased by a factor of 6, from \$6.1 billion to \$35.9 billion. In 2014, the top ten drugs by gross spending accounted for about 20.4 percent of total gross drug costs in Medicare Part D. This proportion has been relatively stable since 2007 (21.5 percent), thanks partly to two key opposing forces. On the one hand, generic entry of a blockbuster drug (such as Atorvastatin's entry in late 2011 to compete against Lipitor) decreased the top 10 share of spending. On the other hand, entry of new expensive drugs that made into the top 10 raised the top 10 share of spending. For example, Sovaldi entered the market late in 2013 and moved into the top ten list with a relatively small number of claims and users.

Further entry of new expensive drugs (biologics and orphan drugs) into the market may increase the proportion of gross drug costs attributable to the top 10 drugs in the coming years. By therapeutic class, spending in Medicare Part D was highest for antidiabetics and antipsychotics/antimanics from 2007 to 2014. During this time period, generics increased from 18.5 percent to 23.0 percent of gross spending and from 52.8 percent to 77.5 percent of filled prescriptions.

The recent sharp increase in Medicare Part D spending is attributable mainly to price rather than utilization growth, which poses a challenge to the current system to ensure reasonable pricing for new entrant drugs as well as existing drugs with limited competition.

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CHAPTER 5: MEDICAID

This chapter provides an overview of the Medicaid program and approaches used to control spending. It also provides findings from analyses of Medicaid data conducted specifically for this report about prescription drug spending, including trends over time and spending for specialty drugs and biologics, for the top 10 prescription drugs consumed by Medicaid beneficiaries, by therapeutic class, and for generic drugs.

Key Findings

- Medicaid prescription drug spending fell sharply after Medicare Part D assumed costs for dual eligible enrollees in 2006. Between 2006 and 2013, Medicaid prescription drug spending net of rebates rose 15.0 percent, to \$22.0 billion, an increase of about 2 percent per year. In 2014, the combination of new, expensive drugs for hepatitis C and other conditions, price increases in existing drugs, a relatively low number of patent expirations, and increased enrollment due to Medicaid expansion under the Affordable Care Act increased net prescription drug spending 24.3 percent to \$27.3 billion. Spending per enrollee increased 13.5 percent from 2013 to 2014.
 - In the Medicaid program in 2014, biologics accounted for only 3 percent of utilization, but 15.7 percent of gross spending (\$7.3 billion).
 - Psychotherapeutic drugs have consistently been the largest therapeutic class for the Medicaid program. Gastrointestinal drugs are the largest therapeutic class in units dispensed. Led by Sovaldi, gross spending on antivirals rose from \$59.0 million in 2012 to \$1.9 billion in 2014, an increase of 3,092.1 percent.
 - The top 10 small-molecule branded drugs by gross spending accounted for about 17 percent of total gross drug cost in the Medicaid program in 2014. Sovaldi, a recent market entrant, ranked second and Truvada, approved in 2012, ranked fourth.
 - In 2014 in the Medicaid program, generic drugs represented the majority of drugs used, almost 57 percent of units. However, generics represented 18.3 percent of gross spending.
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Program overview

Medicaid, created alongside Medicare in 1965, provides comprehensive health coverage, including prescription drug benefits, to low-income individuals and families. Unlike Medicare or Veterans Health Administration coverage, Medicaid is administered by states in accord with federal statutes and regulations. Financial responsibility for Medicaid is apportioned between the federal government and the states according to the applicable Federal Medical Assistance Percentage (FMAP): the federal government pays from 50 to 100 percent of costs, with variation by state, eligibility group, and services. Although prescription drug coverage is legally an optional rather than a mandatory Medicaid benefit, all states and the District of Columbia have elected to provide this coverage.

About 69 million people were enrolled in Medicaid in 2015 [1]. Most enrollees receive services under some form of managed care [2]. States can carve prescription drugs out of managed care but fewer do so since the Affordable Care Act (ACA) extended Medicaid prescription drug rebates to cover Managed Care Organizations (MCOs) as well as Fee for Service (FFS) utilization. As of August 2016, thirty-one states and the District of Columbia have expanded Medicaid under the ACA to cover nonelderly adults with incomes at or below 138 percent of the Federal Poverty Level. Children and pregnant women in all states are covered up to at least this income level. The program also covers low-income older adults and people with disabilities, who may be eligible for both Medicaid and Medicare.

Approaches to Controlling Costs

About half of the gross cost of Medicaid prescription drugs comes back to the federal government and the states through rebates [3, 4]. Since the Omnibus Budget Reconciliation Act of 1990, manufacturers have been required to provide rebates on prescription drugs as a condition of state Medicaid coverage for their products. For brand drugs, rebate amounts are based on the greater of a percentage of the Average Manufacturer Price (AMP) or the difference between AMP and the “best price” available to other purchasers ⁴⁵. Additional rebates that apply when the cost of a branded drug increases faster than inflation now account for about half of total rebate amounts on these drugs [3]. The Bipartisan Budget Act of 2015 (Public Law 114-74) amended the Social Security Act to provide for the payment of additional inflation-based rebates for generic drugs.

The ACA made several important changes to Medicaid prescription drug rebates. The rebate percentage for single source/innovator multiple source (brand name) drugs was raised from 15.1 percent to 23.1 percent of AMP for most drugs, with lower rates of 17.1 percent for blood clotting factors and drugs approved by the FDA exclusively for pediatric indications. The minimum rebate percentage for non-innovator (generic) drugs was increased from 11.0 to 13.0 percent of AMP. Line extensions, defined as new formulations of single source brand name drugs or innovator multisource drugs in oral solid dosage form, were made subject to an additional penalty that discourages manufacturers from making trivial changes to avoid inflation rebates.

The ACA also required rebates on drugs provided under Medicaid managed care. This change eliminated states’ previous incentives to carve prescription drugs out of managed care arrangements to obtain rebate savings.

States may negotiate supplemental rebates above those required under the statute, and may act individually or in pools with other states to do so. Supplemental rebates are often tied to placement on prescription drug lists (PDLs). As of March 2016, 46 states and the District of Columbia participated in single-state and/or multistate supplemental rebate arrangements with manufacturers [5].

⁴⁵ “Best price” is defined at 42 CFR 447.505. Exclusions from the prices used in this calculation include the prices charged to Medicare Part D Plans and to the Veterans Health Administration.

After three or more products rated therapeutically equivalent become available, both the original innovator drug and its generic equivalents are subject to a Federal Upper Limit (FUL) on aggregate amount the federal government will reimburse state Medicaid programs for these drugs. Under the statute, as amended by the ACA, and following publication of the Covered Outpatient Drug final rule with comment period issued February 1, 2016 (Final Rule), FULs are based on 175 percent of AMP. Before the ACA, FULs were based on 150 percent of the prices published in national drug compendia. Although the percentage adjustment was smaller, it was applied to a much higher base, resulting in higher FULs [6]. The ACA thus reduced federal costs for drugs subject to FULs.

Forty-four states and the District of Columbia have their own Maximum Allowable Cost (MAC) lists for multiple-source drugs [7]. State MAC lists generally include more drugs than are covered by FUL and set lower prices [8].

State Medicaid agencies use many of the same repertoire of drug purchasing arrangements and utilization management tools as private insurers. These approaches are described in more detail in Chapter 7 and include PDLs, prior authorization, drug utilization review [9], and quantity limits. States, however, may not use PDLs or prior authorization requirements to prevent access to drugs approved by the FDA and manufactured by companies participating in the rebate program when they are prescribed for medically accepted indications.

The following sections present summarized data for overall prescription drug spending. Findings for multiple measures of spending and utilization are also presented, including those for trends over time for biologics, for the top 10 prescription drugs, by therapeutic class, and for generic drugs.

Medicaid Spending and Spending Trends

The following sections describe the Medicaid files that were used in the analyses of trends, biologics, top 10 drugs in terms of spending, top therapeutic classes, and generic drugs.

Data and Methods

Gross spending and utilization were developed from the Medicaid State Drug Utilization public-use data available from CMS for years 2003-2014.⁴⁶ CMS provided a proprietary dataset of state-reported rebates for years 2009-2014 under non-disclosure provisions.⁴⁷ Both datasets provide values indexed by individual national drug code (NDC) and year.

⁴⁶ Medicaid State Drug Utilization public-use data available at <https://www.medicaid.gov/Medicaid-CHIP-Program-Information/By-Topics/Benefits/Prescription-Drugs/Medicaid-Drug-Rebate-Program-Data.html>.

⁴⁷ Net price and spending data for 2009 are based on Fee for Service (FFS) utilization only because the rebate data for Managed Care Organizations (MCOs) in that year are unreliable. Net price and spending data for 2010-2014 include both FFS and MCO utilization.

Measures

All measures were summarized at the individual drug level, and then additional information was provided regarding therapeutic class and whether the drug was considered a biologic. Individual NDCs were mapped to therapeutic classes defined by First Databank using a crosswalk constructed for this report. Drugs were identified as biologics using information from the FDA.

Gross and net spending amounts and utilization volumes were summarized for individual brand drugs and at the molecule level for multi-source generics (generics manufactured by more than one company). The definition of a “unit” is determined by the drug’s national drug code (NDC) and can vary widely across drugs in the same therapeutic class or even across different sources of the same drug.

Measures of net spending were developed at the NDC level by subtracting proprietary state-reported rebate amounts from gross spending amounts obtained from the public use files. Rebate data were available from 2009-present and so measures of net spending are limited to those years. Annual prescription drug costs do not adjust for biomedical inflation.

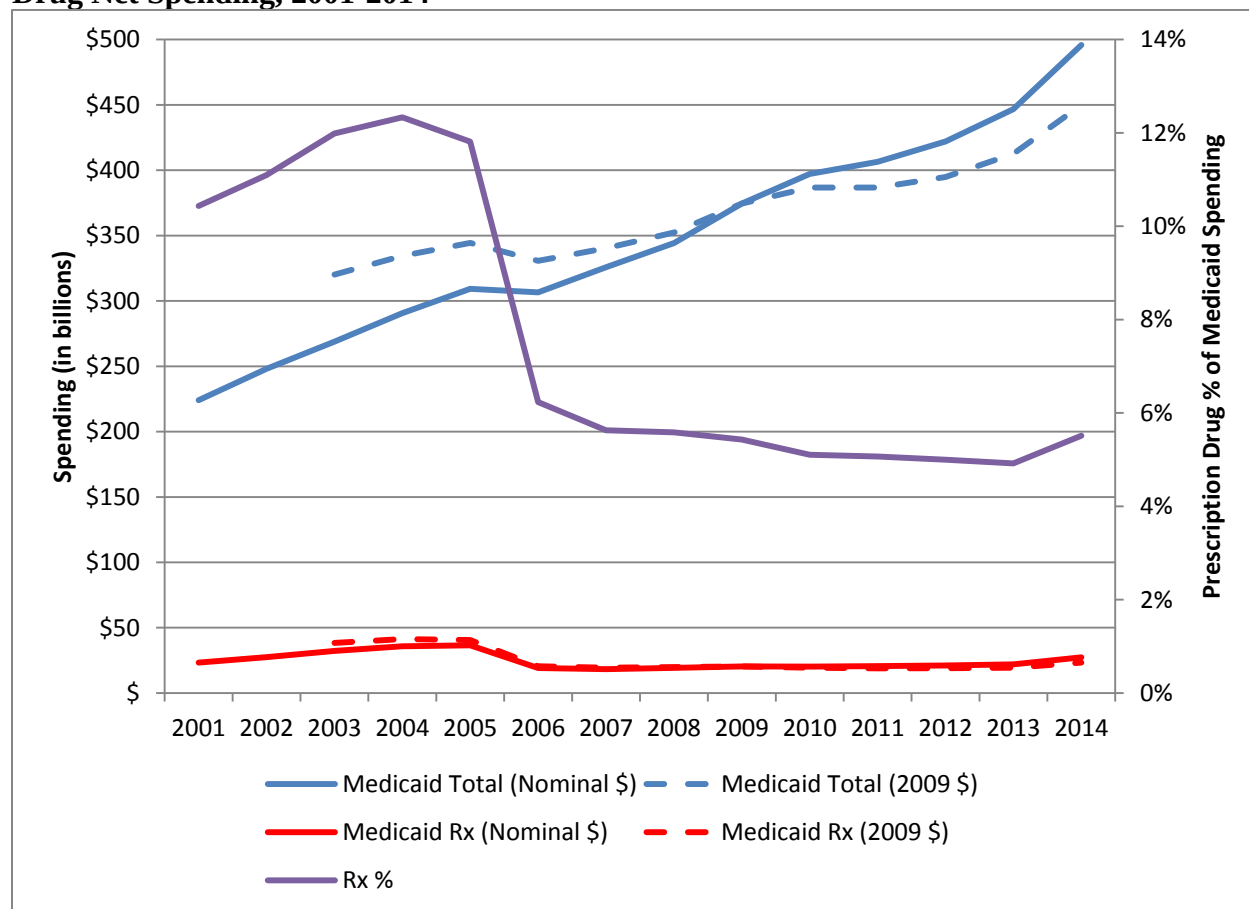
Overall spending and spending trends

Medicaid gross spending on prescription drugs in Calendar Year (CY) 2014 totaled \$46.2 billion. About 47.4 percent, however, came back to the federal government and the states as rebates, resulting in net spending of \$24.3 billion. Figure 1 shows National Health Expenditure Accounts (NHEA) estimates of Medicaid total and prescription drug net spending⁴⁸. The impact of Medicare Part D is particularly evident: between 2005 and 2006, net Medicaid spending on prescription drugs fell by nearly half (47.7 percent), from \$36.5 to \$19.1 billion as prescription drug coverage for dual enrollees shifted from Medicaid to Part D.. The proportion of Medicaid spending that was on prescription drugs also fell, from 11.8 to 6.2 percent. Between 2006 and 2013, Medicaid prescription drug spending rose 15.0 percent, to \$22.0 billion, an increase of about 2 percent per year; this nominal increase, moreover, is equivalent to a 5.5 percent decrease when spending is adjusted to 2009 dollars using the NHEA deflator for prescription drugs.⁴⁹ Throughout this period, prescription drug spending fell as a proportion of all Medicaid spending, from 6.2 percent in 2006 to 4.9 percent in 2013.

⁴⁸ NHEA estimates may be downloaded from <https://www.cms.gov/research-statistics-data-and-systems/statistics-trends-and-reports/nationalhealthexpenddata/nationalhealthaccountshistorical.html>.

⁴⁹ NHIS deflator values are not available for 2001 and 2002.

Figure 1. National Health Expenditure Accounts (NHEA) Medicaid Total and Prescription Drug Net Spending, 2001-2014



In 2014, the combination of new, expensive drugs for hepatitis C and other conditions, price increases in existing drugs, a relatively low number of patent expirations, and increased enrollment due to Medicaid expansion under the ACA increased net prescription drug spending by 24.3 percent to \$27.3 billion, 5.5 percent of total Medicaid spending. The increase in net spending per enrollee between 2013 and 2014 was 13.5 percent (data not shown).

An estimated 25 percent of the increase in Medicaid spending between 2013 and 2014 was due to increased utilization, primarily from increased enrollment, and 75 percent was due to increases in price. Increases in the price of existing brand drugs contributed more than 40 percent of the increase in gross spending in 2014. However two other factors should be noted. First, increases in the price of existing generic drugs contributed 14 percent to the increase in spending in 2014. This pattern is consistent with emerging trends over the past years of increasing generic prices for key drugs. More significantly, 22 percent of the total increase in gross spending was linked to the introduction of just 12 innovator drugs for anticoagulation and treatment of diabetes, hemophilia, hepatitis C, HIV/AIDS and multiple sclerosis.

Biologics

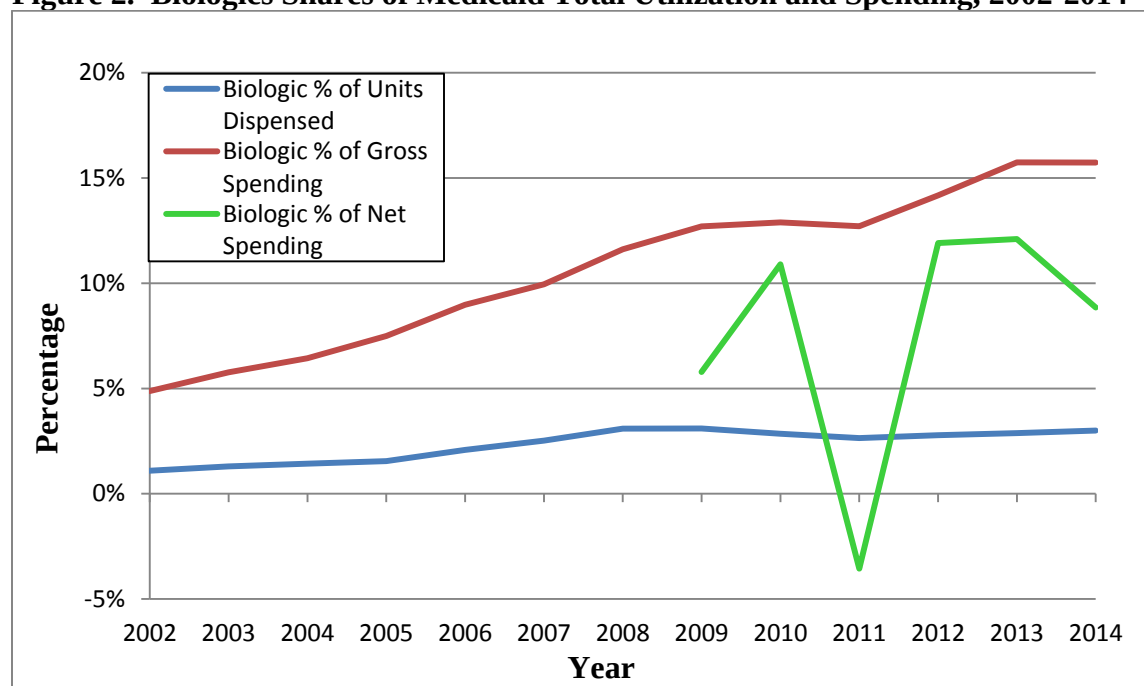
Biologic drugs account for a small share of Medicaid utilization but a larger share of Medicaid spending. In 2014, \$39.0 billion in gross spending (84.3 percent) and \$22.2 billion in net spending (91.2 percent) was on small molecular entities (SMEs); compared with \$7.3 billion gross spending (15.7 percent) and \$2.2 billion net spending (8.8 percent) on biologics (Table 1). SME utilization totaled 35.2 billion units (97.0 percent), compared with 1.1 billion biologic units (3.0 percent).

Table 1. Medicaid Small Molecular Entities (SMEs) and Biologic Spending and Utilization, Calendar Year 2014 (in billions)

Category	SME Total	SME Share	Biologic Total	Biologic Share	Total
Gross Spending	\$39.0	84.3%	\$7.3	15.7%	\$46.2
Net Spending	\$22.2	91.2%	\$2.2	8.8%	\$24.3
Units Dispensed	35.2	97.0%	1.1	3.0%	36.3

Biologics' share of Medicaid utilization nearly tripled between 2002 (1.1 percent) and 2008 (3.1 percent) but has been fairly flat since then (Figure 2). In 2014, biologics made up 3.0 percent of Medicaid units dispensed, compared with 97.0 percent for small molecular entities (SMEs). The biologic share of gross spending was higher and increased more rapidly, from 4.9 percent in 2002 to 15.7 percent in 2014. The biologic share of net spending in 2011 is shown as negative, suggesting that rebates exceeded gross prices. This is possible under Medicaid rebate rules but may also reflect lags in state implementation of ACA changes to those rules that caused rebates to show up in a later year than the utilization to which they were connected.

Figure 2. Biologics Shares of Medicaid Total Utilization and Spending, 2002-2014



Therapeutic class

Table 2 shows spending and utilization by therapeutic class in 2014. Psychotherapeutic drugs, such as Abilify (aripiprazole) and Vyvanse (lisdexamfetamine dimesylate), represent the largest therapeutic class in both gross spending (\$8.8 billion, 19.1 percent of total) and net spending (\$4.2 billion, 17.4 percent of total). The anti-infectives/miscellaneous category, including Truvada (emtricitabine/tenofovir disoproxil fumarate), ranked second in gross (\$4.1 billion, 8.9 percent) and net (\$2.2 billion, 9.1 percent) spending. Utilization was led by gastrointestinal drugs and psychotherapeutic drugs.

**Table 2. Medicaid Spending and Utilization by Therapeutic Class, Calendar Year 2014
(in thousands)**

Therapeutic Class	Gross Spending	Gross Share	Net Spending	Net Share	Units Dispensed	Units Share
Analgesic/Antihistamine Combination	\$12	0.0%	\$12	0.0%	103	0.0%
Analgesics	\$1,826,354	4.0%	\$1,342,448	5.5%	3,120,714	8.6%
Anesthetics	\$147,106	0.3%	\$104,714	0.4%	83,774	0.2%
Antiarthritics	\$1,218,153	2.6%	\$463,426	1.9%	1,492,926	4.1%
Antiasthmatics	\$3,916,072	8.5%	\$1,364,067	5.6%	1,892,579	5.2%
Antibiotics	\$1,747,749	3.8%	\$1,270,829	5.2%	3,058,010	8.4%
Anticoagulants	\$347,794	0.8%	\$255,968	1.1%	94,293	0.3%
Antidotes	\$8,071	0.0%	\$7,203	0.0%	3,672	0.0%
Antifungals	\$296,710	0.6%	\$256,446	1.1%	441,285	1.2%
Antihistamine/Decongestant Combination	\$11,899	0.0%	\$10,380	0.0%	50,793	0.1%
Antihistamines	\$324,467	0.7%	\$242,177	1.0%	1,744,941	4.8%
Antihyperglycemics	\$3,175,484	6.9%	\$605,877	2.5%	892,309	2.5%
Antiinfectives	\$73,655	0.2%	\$63,147	0.3%	133,298	0.4%
Antiinfectives/Miscellaneous	\$4,121,002	8.9%	\$2,202,833	9.1%	316,011	0.9%
Antineoplastics	\$1,802,100	3.9%	\$717,564	3.0%	140,089	0.4%
Anti-obesity Drugs	\$2,273	0.0%	\$784	0.0%	820	0.0%
Antiparkinson Drugs	\$60,105	0.1%	\$47,118	0.2%	155,092	0.4%
Antiplatelet Drugs	\$73,270	0.2%	\$43,180	0.2%	66,511	0.2%
Antivirals	\$1,882,927	4.1%	\$1,400,956	5.8%	11,465	0.0%
Autonomic Drugs	\$1,250,081	2.7%	\$964,293	4.0%	709,630	2.0%
Biologicals	\$186,616	0.4%	\$119,980	0.5%	9,714	0.0%
Blood	\$1,269,234	2.7%	\$759,239	3.1%	852,258	2.3%
Cardiac Drugs	\$178,861	0.4%	\$143,017	0.6%	360,012	1.0%
Cardiovascular	\$2,265,877	4.9%	\$1,716,271	7.1%	1,797,862	5.0%
Central Nervous System Drugs	\$2,817,678	6.1%	\$1,367,194	5.6%	2,659,023	7.3%
Colony Stimulating Factors	\$460,403	1.0%	\$143,742	0.6%	5,656	0.0%
Contraceptives	\$804,591	1.7%	\$465,859	1.9%	306,703	0.8%
Cough/Cold Preparations	\$109,398	0.2%	\$94,142	0.4%	1,062,083	2.9%
Diagnostic	\$19,394	0.0%	\$12,962	0.1%	4,886	0.0%
Diuretics	\$87,468	0.2%	\$79,859	0.3%	402,553	1.1%
Eent Preps	\$563,599	1.2%	\$261,841	1.1%	199,311	0.5%
Elect/Caloric/H2O	\$392,359	0.8%	\$215,508	0.9%	2,211,395	6.1%
Gastrointestinal	\$1,683,477	3.6%	\$898,752	3.7%	4,682,680	12.9%
Herbals	\$14	0.0%	\$13	0.0%	77	0.0%
Hormones	\$1,173,806	2.5%	\$660,203	2.7%	397,764	1.1%

Prescription Drugs: Innovation, Spending, and Patient Access

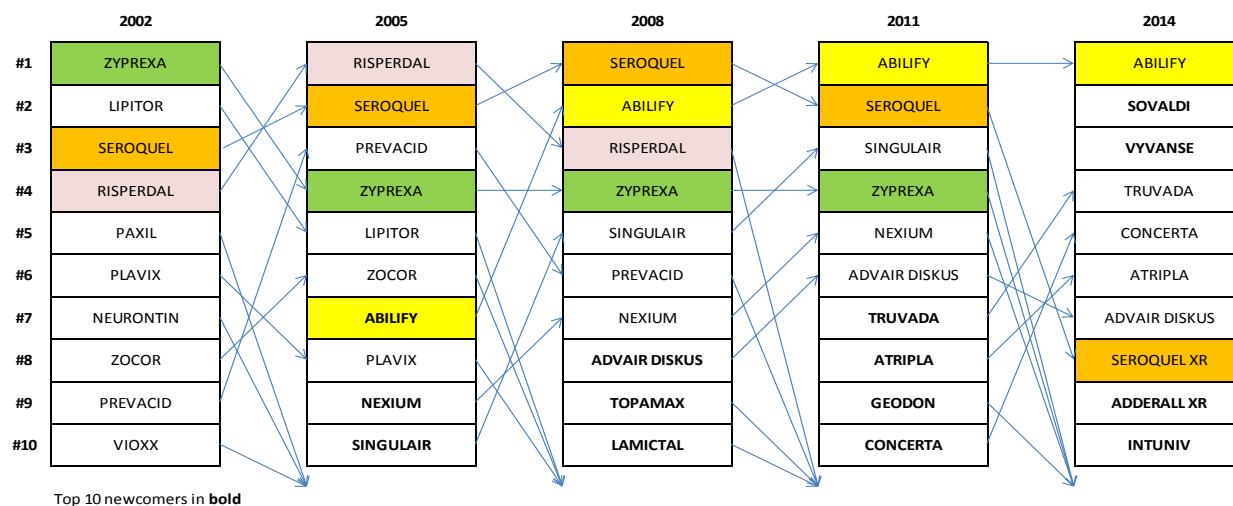
Therapeutic Class	Gross Spending	Gross Share	Net Spending	Net Share	Units Dispensed	Units Share
Immunosuppressant	\$315,967	0.7%	\$129,698	0.5%	106,960	0.3%
Misc. Med. Supplies, Devices, Non-drug	\$1,305	0.0%	\$1,302	0.0%	849	0.0%
Muscle Relaxants	\$140,259	0.3%	\$125,999	0.5%	515,256	1.4%
Prenatal Vitamins	\$59,811	0.1%	\$52,982	0.2%	83,252	0.2%
Psychotherapeutic Drugs	\$8,843,404	19.1%	\$4,221,486	17.4%	3,400,647	9.4%
Sedative/Hypnotics	\$123,386	0.3%	\$61,570	0.3%	200,415	0.6%
Skin Preps	\$778,222	1.7%	\$538,639	2.2%	1,297,726	3.6%
Smoking Deterrents	\$103,748	0.2%	\$42,928	0.2%	48,265	0.1%
Thyroid Preps	\$111,839	0.2%	\$96,737	0.4%	267,287	0.7%
Unclassified Drug Products	\$1,356,116	2.9%	\$651,170	2.7%	775,437	2.1%
Unknown	\$1,440	0.0%	\$751	0.0%	198	0.0%
Vitamins	\$98,711	0.2%	\$76,548	0.3%	223,189	0.6%
TOTAL	\$46,232,266	100.0%	\$24,301,816	100.0%	36,279,772	100.0%

In every year, psychotherapeutic drugs led in gross spending and net spending (when available), and finished second to gastrointestinal drugs in units dispensed. The 19.1 percent share of gross spending on psychotherapeutic drugs in 2014, the smallest share for this category in the entire time series, was nonetheless more than twice the share for the second-highest category, antiinfectives/miscellaneous, which included Truvada. Cardiovascular drugs dropped from 10.1 percent of gross spending and 5.6 percent of utilization in 2005 to 6.0 percent of gross spending and 3.8 percent of utilization in 2006 as dual enrollees moved onto Part D. Led by Sovaldi, gross spending on antivirals rose from \$59.0 million in 2012 to \$1.9 billion in 2014, an increase of 3,092.1 percent.

Top 10 branded small-molecule prescription drugs

The evolving composition of the top 10 Medicaid branded small-molecule prescription drugs by gross spending at three-year intervals, shown in Figure 3, illustrates the continuities and changes in the program between 2002 and 2014. Although none of the top drugs in 2002 was still on the list in 2014, an antipsychotic drug led the list in each of the years shown. The top ten branded small-molecule drugs by gross spending accounted for about 26 percent of total gross drug costs for all drugs (including generics and biologics) in 2002 and 17 percent in 2014.

Figure 3. Top 10 Medicaid Branded Small-Molecule Prescription Drugs by Gross Spending, 2002-2014 (Selected Years)



The inauguration of Medicare oral prescription drug coverage in 2006 affected Medicaid by shifting prescription drug costs for dual enrollees onto Part D. Medicaid spending subsequently shifted away from drugs most utilized by the elderly, including the statin Lipitor (atorvastatin calcium), used to treat high cholesterol, and the blood thinner Plavix (clopidogrel bisulfate). Both of these drugs dropped out of the Medicaid top 10 list between 2005 and 2008, but ranked high on the Medicare Part D list until the later development of their generic equivalents.

Drugs often fall out of the Medicaid top 10 list when generic substitutes become available. Thus, Zyprexa (olanzapine, shown in green), the top drug in 2002, and fourth-ranked in 2005, 2008, and 2011, cycled off the list after FDA approval of a generic in 2011, and Risperdal (risperidone, shown in pink), number one in 2005, dropped off after its generic equivalent was approved in 2008. A generic equivalent to the 2008 leader, Seroquel (quetiapine fumarate, shown in orange), was approved in 2012, but an extended-release version of the drug kept it on the list for 2014. A generic equivalent of the 2011 and 2014 leader, Abilify (ariprazole, shown in yellow), was approved in 2015 and may affect gross spending on this drug going forward.

New brand-name small-molecule drugs most often start in the lower half of the Top 10 list and then move upward as utilization increases and other drugs fall off. But Sovaldi (sofosbuvir), introduced in 2013 as the first of the new hepatitis C drugs, was second-highest in gross spending in 2014. It was followed by Vyvanse (lisdexamfetamine dimesylate), used to treat ADHD, and Truvada (tenofovir/emtricitabine), used for HIV treatment and prevention.

Generic drugs

The majority of Medicaid spending is on brand-name drugs, but most utilization is of generics, which are not available for all medications but are usually much less expensive than branded versions when they are available. As shown in Table 3, gross spending of \$37.8 billion on brand-name drugs represented 81.7 percent of the total, compared with \$8.4 billion (18.3 percent) for generics. Because brand-name drugs are subject to larger rebates, the branded drug

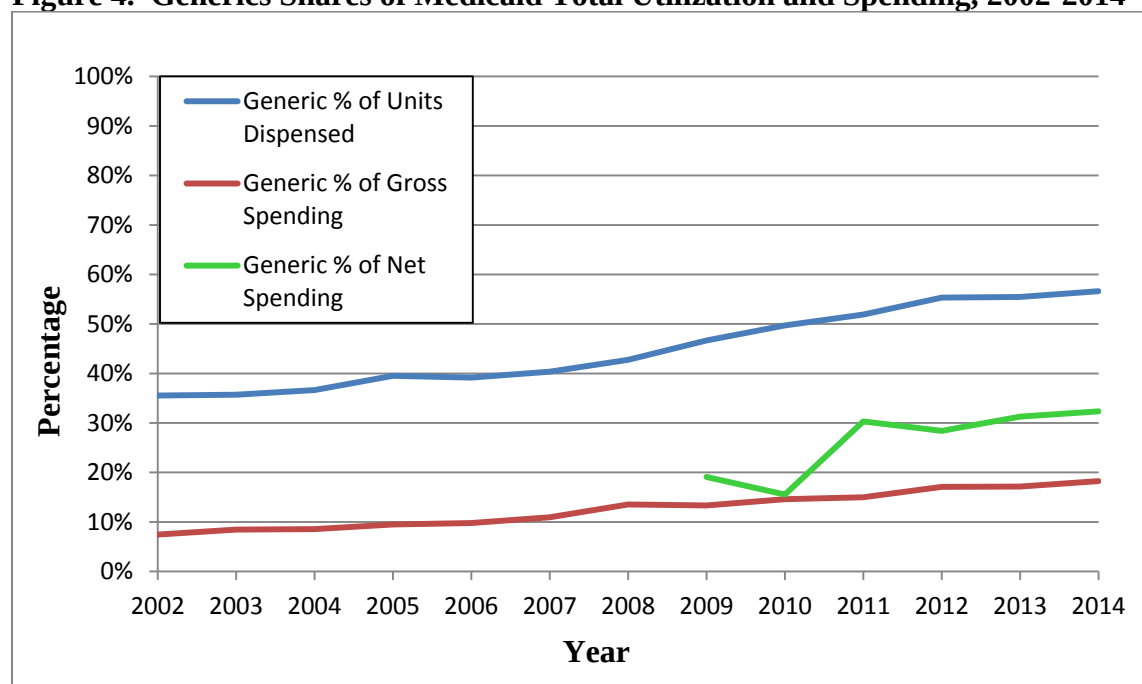
share of net spending was lower, with \$16.4 billion net spending on brands (67.6 percent) compared with \$7.9 billion spending on generics (32.4 percent). Utilization of brand-name drugs totaled 15.7 billion units (43.4 percent), compared with 20.5 billion units of generics (56.6 percent).

Table 3. Medicaid Brand and Generic Spending and Utilization, Calendar Year 2014
(in billions)

Category	Brand Total	Brand Share	Generic Total	Generic Share	Total
Gross Spending	\$37.8	81.7%	\$8.4	18.3%	\$46.2
Net Spending	\$16.4	67.6%	\$7.9	32.4%	\$24.3
Units Dispensed	15.7	43.4%	20.5	56.6%	36.3

Medicaid utilization of generic drugs has steadily increased, from 35.5 percent of all units dispensed in 2002 to 56.6 percent in 2014 (Figure 4). The generic share of gross spending has also risen, but remains below the generic share of utilization. Generics are subject to proportionally smaller rebates than brand drugs: in 2014, for example, rebates represented 6.8 percent of gross spending on generics, compared with 56.5 percent of gross spending on brands. The generic share of net spending is between the generic share of utilization and the generic share of gross spending.⁵⁰

Figure 4. Generics Shares of Medicaid Total Utilization and Spending, 2002-2014



Tables 4-6 show gross and net spending and utilization for the top 10 small molecule brand drugs, top 10 small molecule generic, and top 10 biologic prescription drugs. As Figure 3 shows,

⁵⁰ Rebate data, and thus net spending, are not available for 2002-2008.

the identity of the top drugs changed over the period shown. These charts are generally consistent with the aggregate trends noted above. Utilization and spending increased more rapidly for the top generic drugs than for the top branded drugs, while the growth in utilization of and spending on the top biologic drugs slowed in recent years. In 2014, the top 10 small molecule brand drugs accounted for 13.1 percent of all units dispensed. The top generic drugs had larger shares of utilization than brands, with the top 10 accounting for 17.8 percent of all units dispensed. The top 10 biologics had 2.2 percent of units dispensed, but their shares of total gross and net spending were higher.

Table 4. Top 10 Small Molecule by Brand

Top 10 Small Molecule by Brand	By Gross Medicaid Payments		By Net Medicaid Payments		By Medicaid Units
	Gross	Net	Gross	Net	Total
2002	\$1,664,881,476	--	--	--	3,425,378,059
2003	\$2,143,099,796	--	--	--	4,235,665,788
2004	\$3,651,508,345	--	--	--	4,411,657,813
2005	\$4,632,868,573	--	--	--	4,449,495,905
2006	\$3,264,729,507	--	--	--	3,269,109,215
2007	\$4,067,265,168	--	--	--	2,974,603,589
2008	\$5,164,570,939	--	--	--	3,149,343,453
2009	\$5,278,081,667	\$3,383,040,538	\$5,212,941,139	\$3,448,537,357	3,092,512,236
2010	\$6,718,767,961	\$6,126,451,318	\$6,718,767,961	\$6,126,451,318	4,107,375,494
2011	\$7,538,901,436	\$3,048,887,245	\$7,289,951,183	\$3,320,397,815	4,680,057,293
2012	\$6,523,711,564	\$3,122,632,811	\$6,395,267,922	\$3,193,230,730	4,228,845,647
2013	\$6,269,414,748	\$2,457,852,855	\$5,553,327,043	\$2,571,613,004	4,299,176,827
2014	\$7,972,781,452	\$3,642,058,631	\$7,589,236,704	\$3,962,424,295	4,763,262,020

Table 5. Top 10 Small Molecule by Generic

Top 10 Small Molecule by Generic	By Gross Medicaid Payments		By Net Medicaid Payments		By Medicaid Units
	Gross	Net	Gross	Net	Total
2002	\$161,772,850	--	--	--	3,218,971,723
2003	\$198,807,130	--	--	--	3,673,582,527
2004	\$294,458,498	--	--	--	3,883,161,351
2005	\$ 512,553,337	--	--	--	4,270,359,081
2006	\$411,800,042	--	--	--	2,955,186,442
2007	\$548,776,150	--	--	--	2,888,193,688
2008	\$910,117,279	--	--	--	3,152,233,732
2009	\$938,189,889	\$906,196,206	\$938,189,889	\$906,196,206	3,620,218,078
2010	\$1,250,002,311	\$1,249,415,576	\$1,250,002,311	\$1,249,415,576	5,035,178,506
2011	\$1,416,794,071	\$1,292,023,568	\$1,416,794,071	\$1,292,023,568	5,815,977,417
2012	\$1,556,580,290	\$1,460,170,515	\$1,556,580,290	\$1,460,170,515	6,061,461,278
2013	\$1,410,950,311	\$1,340,422,429	\$1,408,980,560	\$1,341,359,533	6,036,499,925
2014	\$2,552,156,911	\$2,462,711,713	\$2,552,156,911	\$2,462,711,713	6,471,810,265

Table 6. Top 10 Biologics

Top 10 Biologics	By Gross Medicaid Payments		By Net Medicaid Payments		By Medicaid Units
	Gross	Net	Gross	Net	Total
2002	\$ 217,387,325	--	--	--	248,119,312
2003	\$319,451,376	--	--	--	339,404,271
2004	\$571,122,392	--	--	--	374,695,798
2005	\$917,523,160	--	--	--	419,977,448
2006	\$741,167,775	--	--	--	374,896,005
2007	\$972,725,787	--	--	--	374,335,789
2008	\$1,315,977,559	--	--	--	458,556,240
2009	\$1,510,601,016	(\$105,363,698)	\$1,299,944,129	\$1,011,664,645	477,559,022
2010	\$1,901,317,933	\$1,543,441,067	\$1,828,089,536	\$1,737,577,701	548,031,153
2011	\$2,128,982,941	(\$2,082,776,058)	\$1,839,604,532	\$1,037,971,602	580,950,455
2012	\$2,420,292,202	\$1,056,289,311	\$2,155,366,439	\$1,205,316,506	642,888,412
2013	\$2,857,808,641	\$932,928,039	\$2,145,839,628	\$1,144,154,763	708,264,918
2014	\$3,750,245,190	\$854,209,167	\$2,867,277,672	\$1,190,907,206	784,845,331

Summary

Medicaid prescription drug spending fell sharply after Medicare Part D assumed costs for dual eligible enrollees in 2006. Between 2006 and 2013, however, Medicaid prescription drug spending rose 15.0 percent, to \$22.0 billion. In 2014, prescription drug spending increased 24.3 percent to \$27.3 billion. Biologics accounted for only 3 percent of utilization, but 15.7 percent of gross Medicare spending (\$7.3 billion) in 2014. In contrast, generic drugs represented the majority of drugs used in 2014, almost 57 percent of units, but accounted for only 18.3 percent of gross spending. The top 10 drugs by gross spending accounted for about 17 percent of total gross drug cost. Psychotherapeutic drugs have consistently been the largest therapeutic class in spending, and gastrointestinal drugs the largest therapeutic class in units dispensed, for the Medicaid program. Led by Sovaldi, gross spending on antivirals rose from \$59.0 million in 2012 to \$1.9 billion in 2014, an increase of 3,092.1 percent in Medicaid spending.

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CHAPTER 6: VETERANS HEALTH ADMINISTRATION

This chapter provides an overview of the Veterans Health Administration (VHA). Data and analyses related to prescription drug spending conducted specifically for this report are presented. Trends in prescription drug spending address the top ten drugs, therapeutic class, and generics.

Key Findings:

- VHA total spending on prescription drugs rose from \$3.2 billion in 2007 to \$3.6 billion in 2014, with an average annual growth rate of 1.5 percent. Growth in the last year of that period, however, was 11.7 percent.
 - In 2014, VHA drug spending was highest for antimicrobials, which include the hepatitis C drugs, and central nervous system medications. Those two categories accounted for about 36 percent of 2014 drug spending. Spending in 2007 was highest for central nervous system medications and cardiovascular medications, which accounted for 41 percent of 2007 drug spending. The hepatitis C drugs were largely responsible for the antimicrobial medications moving into the highest cost drug class for VHA in 2014.
 - The top ten drugs by total cost in the VHA accounted for 27 percent of all prescription drug costs in 2014, a figure similar to that in 2007.
 - In 2014 in VHA, generic drugs represented approximately 84 percent of the outpatient 30-day equivalent prescription fills and 25 percent of the drug spending.
-

Program Overview

The Veterans Health Administration (VHA) is the largest integrated health care system in the United States. Operated by the Department of Veterans Affairs (VA), VHA provides medical benefits and services to veterans who meet certain eligibility criteria. VHA health care services are generally available to all honorably discharged veterans who are enrolled in VA's health care system, subject to a priority enrollment system based on various criteria. The VHA is distinct from the Department of Defense health care system.

Approaches to Controlling Costs

Unlike other federal health programs such as Medicare or Medicaid which provide insurance coverage and financing for privately-delivered medical care services, the VHA is a direct medical service provider. Also distinguishing the VHA system from other federal health insurance programs is its ability to establish an evidence-based national formulary for preferred drugs (for example, those drugs deemed to provide the greatest value for the price). Drugs are added to the national formulary based on safety, efficacy, and cost (in that order), and when differences in safety and efficacy between drugs are smaller, cost considerations become more

important. The national formulary allows the VHA to purchase prescription drugs at some of the lowest prices available to any buyer in the U.S., based on its ability to drive utilization to the most cost effective drugs. Another factor helping the VHA obtain price concessions is that its negotiated prices are exempted from the “best-price” provision in the Medicaid program, which makes it easier for manufacturers to offer steep discounts without owing a greater rebate to Medicaid. Private purchasers such as HMOs, for example, are not exempted from the best-price provision, with the result that manufacturers effectively would pay a financial penalty if they offered an HMO a price discount.

VHA Prescription Drug Spending and Spending Trends

Overall spending and spending trends

The VHA spent \$3.6 billion on prescription drugs in fiscal year 2014, compared to \$3.2 billion in fiscal year 2007, for an annual growth rate of 1.4 percent—a notably modest rate of increase compared to that seen in other programs. During 2007 and 2008, total drug spending actually fell, while the total number of prescriptions continued a steady increase. Most recently, from 2013-2014, total spending growth rose to 11.7 percent, presumably in part because of the availability of costly new drug therapies. (See Figures 1 and 2.)

Figure 1

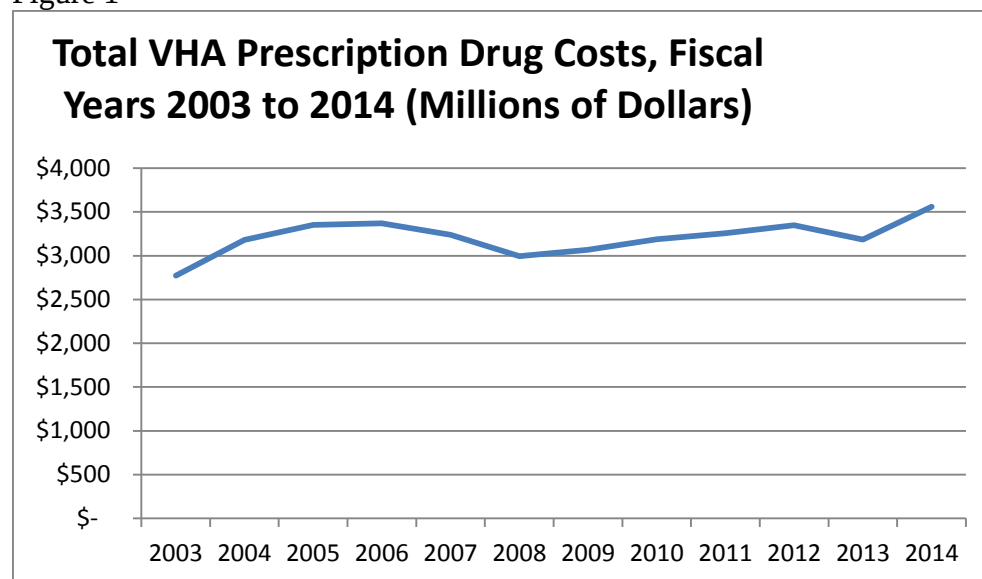
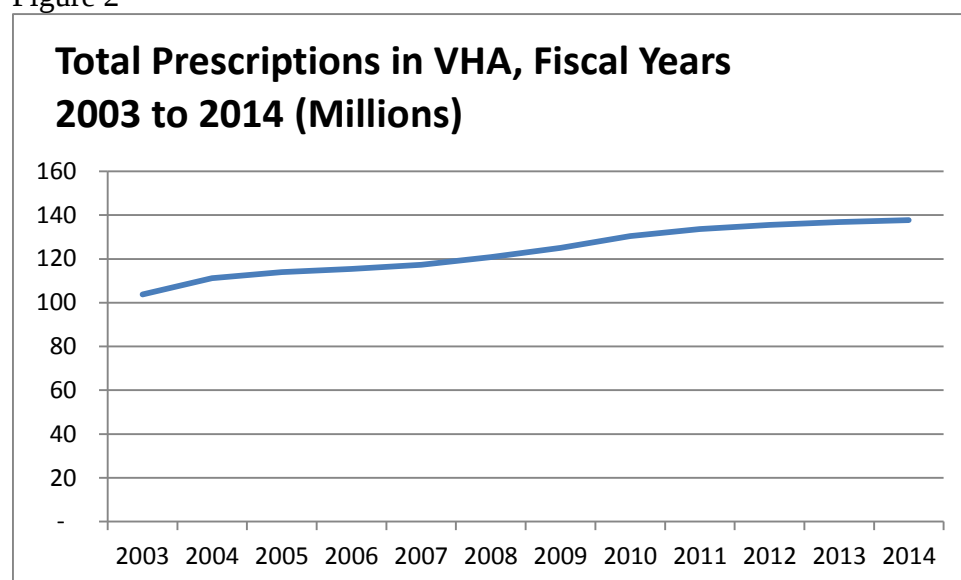


Figure 2



Top Drug Therapy Groups in the VHA

Table 1 shows the five highest-cost drug therapy groups in the VHA for 2007 and 2014. One notable change during this period is that antimicrobials, which were not in the top five groups in 2007, emerged as the highest-cost group in 2014. Between 2007 and 2014, VHA prescription costs for the antiviral drug class, which includes hepatitis C drugs, has increased from \$159.2 million to \$600.3 million and the percentage spent on antivirals has increased from 5 percent to 17 percent of VHA total spending on prescription drugs

Table 1

VHA Prescription Drug Spending by Therapy Group:						
Top Five Groups by Total Cost, Fiscal Years 2007 and 2014 in Millions						
		FY2007			FY2014	
		Cost	% of Total		Cost	% of Total
1	CENTRAL NERVOUS SYSTEM MEDICATIONS	\$777.4	24%		ANTIMICROBIALS	\$654.6 18%
2	CARDIOVASCULAR MEDICATIONS	\$544.8	17%		CENTRAL NERVOUS SYSTEM MEDICATIONS	\$639.2 18%
3	BLOOD PRODUCTS/ MODIFIERS/ VOLUME EXPANDERS	\$303.7	9%		HORMONES/ SYNTHETICS/ MODIFIERS	\$355.0 10%
4	HORMONES/ SYNTHETICS/ MODIFIERS	\$303.6	9%		ANTINEOPLASTICS	\$290.9 8%
5	GASTROINTESTINAL MEDICATIONS	\$234.2	7%		CARDIOVASCULAR MEDICATIONS	\$273.7 8%

Top Ten VHA Drugs by Cost

Figure 3 shows the top ten highest-cost drugs in the VHA system from 2007 to 2014. As is the case in the corresponding charts representing other programs, the rankings for various years differ as new drugs displace others near the top of the list. One difference in this chart representing the VHA compared to the one representing Medicare Part D is that Lipitor (Atorvastatin) does not appear as a top drug in 2007 or 2008; this is attributable to the national formulary used by the VHA system, where drugs are preferred based on safety, efficacy, and cost (in that order). For example, generic simvastatin was VA's preferred high-potency statin in 2007 and 2008. If Lipitor (Atorvastatin) was used in place of generic simvastatin during those two years, based on the volume of tablets and the average price per tablet, VA would have spent an additional \$1.2 billion.

Figure 3 Top Ten VHA Prescription Drugs by Total Cost, Fiscal Years 2007 - 2014

FY2007	FY2008	FY2009	FY2010	FY2011	FY2012	FY2013	FY2014
CLOPIDOGREL	CLOPIDOGREL	CLOPIDOGREL	CLOPIDOGREL	CLOPIDOGREL	INSULIN	INSULIN	SOFOSBUVIR
OMEPRAZOLE	OLANZAPINE	INSULIN	INSULIN	INSULIN	CLOPIDOGREL	ARIPIRAZOLE	INSULIN
SIMVASTATIN	RISPERIDONE	OLANZAPINE	OLANZAPINE	OLANZAPINE	ARIPIRAZOLE	EMTRICITABINE/ TENOFVIR	ARIPIRAZOLE
OLANZAPINE	INSULIN	ALBUTEROL/ IPRATROPIUM	ALBUTEROL/ IPRATROPIUM	ARIPIRAZOLE	ALBUTEROL/ IPRATROPIUM	ADALIMUMAB	EFAVIRENZ/ EMTRICITABINE/
RISPERIDONE	VENLAFAXINE	QUETIAPINE	QUETIAPINE	ALBUTEROL/ IPRATROPIUM	QUETIAPINE	IMATINIB	ADALIMUMAB
VENLAFAXINE	OMEPRAZOLE	VENLAFAXINE	ARIPIRAZOLE	QUETIAPINE	RICITABINE/TEN OFOVIR	ETANERCEPT	IMATINIB
ROSIGLITAZONE	GLUCOSE TEST	DONEPEZIL	VENLAFAXINE	PIOGLITAZONE	OLANZAPINE	ALBUTEROL/ IPRATROPIUM	TIOTROPIUM
FELODIPINE	QUETIAPINE	GLUCOSE TEST	PIOGLITAZONE	GLUCOSE TEST	ADALIMUMAB	TIOTROPIUM	ETANERCEPT
INSULIN	ALBUTEROL/ IPRATROPIUM	ARIPIRAZOLE	GLUCOSE TEST	EMTRICITABINE/ TENOFVIR	ROSUVASTATIN	GLUCOSE TEST	EMTRICITABINE/ TENOFVIR
GLUCOSE TEST	DONEPEZIL	OMEPRAZOLE	DONEPEZIL	ADALIMUMAB	IMATINIB	EMTRICITABINE/ TENOFVIR	SIMEPREVIR

Generics

In 2014 in VHA, generic drugs represented approximately 84 percent of the outpatient 30-day equivalent prescription fills and 25 percent of the drug cost.

Summary

The VHA provides direct medical services to veterans who meet eligibility criteria. Spending on prescription drugs by the VHA grew by a modest annual average rate of 1.5 percent between 2007 and 2014, increasing from \$3.2 billion to \$3.6 billion. The top ten drugs accounted for slightly more than a quarter of all prescription drug costs in 2007 and 2014. The top two therapeutic drug classes accounted for 41 percent of prescription drug spending in 2007 (central nervous system medications and cardiovascular medications) and 36 percent of spending in 2014 (central nervous system medications and antimicrobials). The hepatitis C drugs were largely

responsible for the antimicrobial medications moving into the highest cost drug class for VHA in 2014. Spending on this drug therapy group increased from \$159.2 million in 2007 (5 percent of total spending) to \$600.3 million in 2014 (17 percent of total spending). Generic drugs accounted for 25 percent of spending in 2014, but represented 84 percent of the outpatient 30-day equivalent prescriptions fills.

CHAPTER 7: PRESCRIPTION DRUG PURCHASING ARRANGEMENTS, UTILIZATION MANAGEMENT, AND VALUE-BASED APPROACHES

This chapter provides an overview of prescription drug purchasing arrangements, utilization management and review, and value-based approaches to promote value and control cost. It describes their current use by government insurance programs, including Medicare Part B, Medicare Part D, Medicaid, and the Veterans Health Administration (VHA).

Key Findings

- Use of negotiation with manufacturers and pharmacies, rebates, preferred drug lists or formularies with tiers, prior authorization, step therapy, prescription quantity limits, value-based purchasing and payment, and performance-based risk-sharing or outcomes-based arrangements by Medicare Part B, Medicare Part D, Medicaid, and the VHA vary.
- Medicare Part B does not currently use any of the purchasing arrangements, utilization management strategies, or value-based approaches available in the private sector.
- The Medicare Part D drug benefit is administered through private prescription drug plans, which each separately design and manage benefits and pay claims. Private prescription drug plans use purchasing arrangements and utilization management, including negotiation of prices with manufacturers and pharmacies, rebates, formularies, step therapy, quantity limitations, and prior authorization. All formularies must include “all (with specified exceptions)”⁵¹ drugs in the immunosuppressant, antidepressant, antipsychotic, anticonvulsant, antiretroviral, and antineoplastic classes to ensure patient access to these protected classes of drugs.
- The Medicaid program uses purchasing rebates, preferred drug lists, prior authorization, and drug utilization review to help ensure value in purchasing prescription drugs and managing their use. The Medicaid program cannot deny access to drugs approved by the FDA and manufactured by companies participating in the rebate program when they are prescribed for medically accepted indications.
- The VHA ensures cost-effective use of drugs through statutory discounts, direct negotiation with manufacturers that may include volume discounts and rebates, an evidence-based national formulary process, generic drug use, national criteria-for-use documents with a blend of clinical criteria, step therapy, and quantity limits, and outcomes-based risk-sharing agreements. The VHA influences prescribers to use cost-

⁵¹ The current exceptions are that the formulary does not have to include all therapeutic equivalents (i.e., generics) and can use safety edits to limit quantities (see 42 CFR 423.120(b)(2)(vi)).

effective drugs through the use of national monitoring of cost-saving opportunities and academic detailing.

- Some strategies to control costs may restrict access and increase patient cost-sharing for prescription drugs. However, data about utilization management and formulary strategies and patient access to prescription medications are not collected systematically across programs. As a result, the effects of these approaches cannot be compared by program.
-

Overview of prescription drug purchasing arrangements, utilization management, and value-based approaches

A number of prescription drug purchasing arrangements, utilization management strategies, and value-based approaches are used by commercial health insurers and other large purchasers, such as pharmaceutical benefits managers (PBMs), as well as some government programs, to promote value and control the cost of prescription medications. Purchasing arrangements reduce the price of prescription drugs that commercial and government insurers and PBMs pay to manufacturers. Utilization management strategies are used by insurers and PBMs to encourage use of drugs based on safety, appropriateness, and cost. These strategies frequently provide incentives to patients for specific medications through lower cost-sharing for similar drugs that are less expensive (e.g., generics). Value-based approaches structure benefits and patient cost-sharing to incentivize providers to prescribe and patients to use drugs with the highest value, frequently measured in terms of effectiveness, rather than cost alone. Value-based approaches can also be used by health insurance programs and PBMs in prescription drug purchasing from manufacturers.

Purchasing arrangements

Purchasing arrangements to reduce the price of prescription drugs include direct negotiation and rebates. Direct negotiation is typically a volume discount approach that leverages high volume of specific prescription drugs or groups of prescription drugs to negotiate discounts from manufacturers. Growth-based discounts are also used to reduce prices for purchasers who meet criteria for volume growth over a specified time period. Rebates are a negotiated price discounting strategy where drug manufacturers return a portion of the purchase price to the purchaser. Rebates may have volume or market-share requirements, or requirements for formulary inclusion or provider, pharmacist, and patient incentives to promote the selection and use of specific prescription drugs.

Utilization management and review strategies

Utilization management and review strategies include prior authorization, step therapy, quantity limits, and formularies. Prior authorization refers to the process used to determine whether an insurer will provide coverage of a specific medication prior to its receipt based on a determination about both safety and cost. Prior authorization is most commonly used for the prescribing of brand-name drugs where generics are available, for expensive medications, or when prescribing deviates from standard use.

Step therapy is a sequential process whereby a patient must first have an unsuccessful result from a medication preferred by the insurance provider, typically either lower cost or considered more effective or safer, before a different, more expensive option will be approved for coverage. Step therapy is more commonly applied to prescription drugs with multiple options within a therapeutic class and less commonly applied to specialty drugs, where few substitutes exist. Quantity limits may also be applied to the number of different and/or branded prescriptions a patient can fill in a specified period (e.g., month).

A formulary or preferred drug list is a list of drugs that are covered by a PBM or an insurer. Formularies are continuously updated. New drugs approved by the Food and Drug Administration (FDA) are reviewed by a PBM or health plan's pharmacy and therapeutics committee, which typically focusses on safety, efficacy, and cost. Some drugs that have close substitutes may be excluded from a formulary. Covered drugs are generally assigned to different tiers of the formulary based on type of the drug (i.e., generic, branded small molecule drug, biologic or specialty drug), and by level of patient cost-sharing (via co-payments, a fixed flat fee, or coinsurance, calculated as a percentage of the prescription drug list price). For example, in the first tier of a five-tiered formulary, a preferred generic drug copayment might be \$2. In the second tier, a non-preferred generic drug copayment might be \$6; in the third tier, a preferred brand copayment, \$40; and in the fourth tier, a non-preferred brand copayment, \$90 or a coinsurance rate of 25 percent of the drug list price. In the fifth tier, a specialty drug coinsurance rate of 20 percent-33 percent of the retail price of the drug is common [1]. Quantity limits can also be applied to specialty drugs to address safety and minimize waste and cost. Quantity limits of 15-day or 30-day supply for specialty drugs for a fixed number of prescriptions within a specified time period are common. The percentage of plans and insurers using formularies with five tiers and using coinsurance for the non-preferred brand tier has increased in recent years [1].

Value-based purchasing and patient- and provider-incentive approaches

Value-based purchasing approaches structure purchasing of prescription drugs from manufacturers based on clinical effectiveness, and include indication-based pricing and performance-based risk-sharing or outcomes-based agreements. Indication-based pricing is a drug purchasing strategy where the payment for a drug varies based on its effectiveness for different clinical indications [2]. For example, the drug erlotinib can be used to treat both metastatic non-small cell lung cancer and pancreatic cancer, but gains in median survival are much higher for lung cancer patients than for pancreatic cancer patients [2]. Currently, the purchase price of erlotinib is the same whether it is used to treat lung or pancreatic cancer. Under an indication-based pricing approach, the price paid to the manufacturer for erlotinib would be higher for treating lung cancer than for treating pancreatic cancer because erlotinib is more effective in treating lung cancer [2].

Performance-based risk-sharing or outcomes-based agreements refer to agreements between payers and manufacturers to link payment to the drug performance and the health outcomes of treated patients. That is, if patient outcomes are poorer than the agreed-upon outcome target, the manufacturer provides price adjustments, rebates, or refunds to the payer. These arrangements require significant data infrastructure to identify eligible patients and to measure and monitor relevant patient outcomes [3, 4]. They may also require information about patient adherence to

treatment and other aspects of care. Performance-based risk-sharing arrangements are more frequently used in Europe, Canada, and Australia, than in the United States [3], although commercial insurers and PBMs are increasingly implementing these arrangements.

Plan design benefits and patient cost-sharing can also be structured to incentivize providers to prescribe and patients to use drugs with the highest value, measured in terms of effectiveness, rather than cost alone. For example, high-value medications might be available to patients with no cost-sharing, even if they are expensive. Lower-value medications might have higher cost-sharing, even if they are relatively inexpensive. A systematic review of value-based approaches found that they are associated with increased adherence to drug use as prescribed, reductions in health care utilization, and lower out-of-pocket spending on prescription drugs, but no overall cost savings to insurers or patients [5].

Reference pricing uses a single reimbursement rate for a group of therapeutically similar drugs based on a benchmark (e.g., lowest-priced drug or most clinically effective drug in the group of drugs) [6-10]. Drugs can be grouped by active substance, pharmacologic class, or therapeutic class. The grouping of a branded drug and all corresponding generics, when available, is an example of grouping by active substance. If the price of a specific drug within a group is higher than the reference price for that group, the patient is responsible for paying the difference, providing incentives for using lower-cost drugs with comparable therapeutic effects. In situations where the provider purchases medication and bills the insurer, the provider would only be reimbursed for the reference price for any of a group of medications, resulting in incentives for providers to prescribe lower-cost drugs with comparable therapeutic effects. Reference pricing has been successfully used in many European countries for many years [6-10].

Use of purchasing arrangements, utilization management strategies, and value-based approaches by program

As shown in Table 1, commercial health plans and PBMs use a variety of purchasing arrangements and utilization management strategies. Value-based purchasing and insurance design are also increasingly used in the private sector. In the following section, details about the use of arrangements and utilization management strategies are described by program, including Medicare Part B, Medicare Part D, Medicaid, and the VHA.

Table 1. Prescription Drug Purchasing Arrangements, Utilization Management, and Value-Based Approaches in the United States

		Commercial Health Plans and Pharmaceutical Benefits Managers	Veterans Health Administration	Private Prescription Drug Plans on Behalf of Medicare Part D	Medicare Part B	State Medicaid Programs and Managed Care Plans
Purchasing Arrangements	Direct negotiation/volume discounts	X	X	X		X
	Rebates	X	X	X ¹		X ²
Utilization Management and Review	Formulary design	X	X	X		X
	Utilization and management review	X	X	X		X
	Prior authorization	X	X	X		X
	Step therapy	X	X	X		X
	Quantity limits	X	X	X		X
Value-based Approaches	Indication-based pricing	X				
	Value-based purchasing	X				
	Performance-based risk-sharing/Outcomes based arrangements	X				
	Reference pricing ³				⁴	
	Value-based patient cost-sharing	X	X			

¹ Rebates are negotiated by commercial insurers and pharmacy benefits managers (PBMs) for retail pharmacy drugs; and by providers for drugs administered in offices or hospital outpatient settings. CMS is prohibited from interfering in drug price negotiations between prescription drug plans and manufacturers. However, the program and its beneficiaries benefit from rebates negotiated by the private entities through lower payments and premiums. For example, Medicare Part B reimburses providers using the Average Sales Price (ASP). The ASP is calculated from sales price data sent by manufacturers to the Centers for Medicaid & Medicare Services (CMS); these are required to be net of all rebates and other price concessions. ² About half of Medicaid gross spending on prescription drugs is returned to the federal government and the states in the form of manufacturer rebates. ³ Commercial insurers do not formally use reference pricing. They do, however, frequently employ tiered cost-sharing arrangements which can have incentives similar to reference pricing for patients and providers in situations where generics or other therapeutic alternatives are available in different cost-sharing tiers. ⁴ Branded drugs and their generic counterparts are grouped together when calculating the ASP. When generics are available, the ASP for that active substance is a form of reference pricing. However, active substances within pharmacologic class are not grouped together in calculating a broader ASP for the pharmacologic class, as is done in other countries. In addition, pharmacologic classes are not grouped within therapeutic class.

Medicare Part B

Medicare Part B does not use any of the purchasing arrangements available in the private sector. However, Medicare Part B payments use the Average Sales Price (ASP), which is calculated from sales price data sent by manufacturers to the Centers for Medicaid & Medicare Services (CMS) and required to be net of all rebates and other price concessions. Thus, Part B payments reflect the broader use of rebates by other insurers and programs. It does not employ any of the utilization review and management approaches that are available in the private sector. While the grouping of branded drugs and their generic counterparts (when available) when calculating ASP is a form of reference pricing, Medicare Part B does not use any of the other value-based approaches for purchasing drugs or incentivizing providers and patients to select and use the most effective and least expensive alternatives.

Medicare Part D

The Medicare Part D oral drug benefit is administered through private prescription drug plans, which each separately design and manage benefits and pay claims. These private plans use purchasing arrangements and utilization management, including negotiation of prices with manufacturers and pharmacies, arrangements for rebates, formularies, prior authorization, step therapy, and quantity limits. Because the Medicare Part D program is administered through private plans, the Medicare Program sets general policies regarding the use of purchasing tools, utilization management, and value-based approaches. However, all formularies of the private plans must include “all (with specified exceptions)”⁵² drugs in the immunosuppressant, antidepressant, antipsychotic, anticonvulsant, antiretroviral, and antineoplastic classes to ensure patient access to these six protected classes of drugs.

Medicaid

The Medicaid program cannot deny access to drugs approved by the FDA and manufactured by companies participating in the rebate program when prescribed for medically accepted indications. State Medicaid agencies use purchasing arrangements with manufacturers and a number of utilization review strategies for prescription drugs to help ensure value in their use. About half of the gross cost of Medicaid prescription drugs comes back to the federal government and the states through rebates [11{[MACPAC], 2016 #237}. States may negotiate supplemental rebates and may act individually or in pools with other states. Supplemental rebates are often tied to drug placement on preferred drug lists. As of March 2016, 46 states and the District of Columbia participated in single-state and/or multistate supplemental rebate arrangements with manufacturers [12]. In addition, the Medicaid program has a “best price” provision, which requires makers of innovator (brand-name) drugs to provide either a rebate or the lowest price that a manufacturer has negotiated with other payers, whichever results in lower prices net of rebates.

⁵² The current exceptions are that the formulary does not have to include all therapeutic equivalents (i.e., generics) and can use safety edits to limit quantities (see 42 CFR 423.120(b)(2)(vi)).

State Medicaid agencies use the same repertoire of utilization management tools as private insurers. Forty-five states and the District of Columbia had prescription drug lists as of October 2014 [13]. Prior authorization may be required and all states employ drug utilization review [14]. Many states limit either the overall number of prescriptions or the number of branded prescriptions that can be filled per month (quantity limits) per enrollee. The most restrictive state, Texas, allows enrollees no more than three prescription fills per month, with some exceptions [15].

CMS is interested in exploring value-based purchasing arrangements in the Medicaid program. In November 2015, CMS sent letters to four manufacturers of hepatitis C drugs requesting information about the value-based purchasing arrangements they had developed with other payers for these and other drugs, and whether state Medicaid agencies could participate in such arrangements.

Veterans Health Administration

The VHA purchases prescription drugs directly through a pharmaceutical prime vendor and receives a significant statutory discount on covered drugs - drugs with a new drug application (NDA) or biologics license application (BLA). Purchasing arrangements used include direct negotiation with manufacturers, volume discounts, and rebates. In addition to statutory and negotiated discounts, VHA uses a wide variety of utilization management strategies. The VHA provides coverage for all FDA-approved drugs based on medical necessity. When Veterans require a medication not listed on the VHA Formulary, VHA has an established prior authorization process for providers to prescribe the medications. Additional utilization strategies include the preference for generic drug options when available, as well as national criteria-for-use documents that are evidence-based and emphasize safety and efficacy with a blend of clinical criteria, step therapy, and quantity limits. The VHA also uses value-based approaches that include outcomes-based risk-sharing agreements. Finally, the VHA influences prescribers to use cost-effective drugs through the use of national monitoring of cost-saving opportunities and the use of academic detailing.

Purchasing arrangements, utilization management, and value-based strategies and patient access to prescription drugs

As described above, government programs vary in their use of prescription drug purchasing arrangements, utilization management strategies, and value-based approaches. Purchasing arrangements with manufacturers and negotiation for rebates can potentially lower patient cost-sharing and improve access to prescription drugs if any savings are reflected in lower available prices to patients. Relevant data are not publicly available and little research has explored these relationships, within or between commercial insurers, PBMs, and government programs. Utilization management strategies, such as prior authorization, step therapy, quantity limits, and formularies can restrict or limit patient access to specific prescription medications. In addition, many specialty drugs do not have lower cost alternative treatments, and patients may be faced with the highest levels of cost-sharing for these medications, potentially leading to cost-related barriers to access. This issue is receiving increasing attention in the popular press and the research literature [16] Under value-based benefit designs, even expensive high-value

medications might be available to patients with no cost-sharing, potentially improving access. Thus, utilization management strategies, formularies, and value-based benefit design could have important effects on patient access to prescription medication. However, relevant data are not collected systematically and the effects of these approaches on patient access to prescription medication cannot be evaluated across programs.

Accumulating evidence suggests that patients with higher levels of cost-sharing are more likely to delay or forgo prescription medications for acute and chronic illness or not take medication as prescribed [17-19], jeopardizing any potential benefits of treatment. Cost-related medication non-adherence is also associated with higher rates of potentially avoidable hospitalizations and poorer patient outcomes [20-22]. With increasing costs of prescription drugs [23] and increasing patient cost-sharing [24], this is an active area of research and increasing concern. Access to prescription medications and its association with patient satisfaction with care and health outcomes for each of the government programs is evaluated in greater detail in Chapters 8, 9, and 10, respectively.

Summary

A number of purchasing arrangements, utilization management strategies, and value-based approaches are used by commercial insurers and some government programs. Variation in the use of strategies to promote value in prescription drugs purchasing, prescribing, and use by patients across government programs reflect differences in program structure and statutory requirements. With increasing cost of new drugs, price increases in existing drugs, and greater utilization, understanding the effects of these approaches on access to prescription drugs and patient outcomes is an active area of research, especially with ongoing development and implementation of value-based approaches.

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CHAPTER 8: PATIENT ACCESS TO PRESCRIPTION MEDICATIONS

This chapter provides an overview of the published literature on patient access to prescription medication in the United States and patient factors associated with access to care, including age, health insurance, comorbidity, and medical need. It describes patient cost-sharing under four government programs: Medicare Part B, Medicare Part D, Medicaid, and the Veterans Health Administration (VHA). Findings from quantitative analyses of National Health Interview Survey (NHIS) data conducted specifically for this report are presented by program. Finally, an overview of published literature describing the time interval between patient attempts to fill a prescription and receipt of prescription drugs is presented.

Key Findings

- In the United States, individuals with limited access to prescription medications are more likely to have emergency room visits, preventable hospitalizations, and poorer health outcomes than similar individuals who are able to access and take medications as prescribed.
- One of the strongest predictors of access to prescription drugs is health insurance. The uninsured are most likely to report skipping medication doses, taking less medication or delaying filling prescription drug medications due to cost. Among individuals with health insurance, those with higher cost sharing and out-of-pocket costs are more likely to delay initiation of prescription drugs and have poorer adherence to taking prescription drugs as prescribed.
- Insurers are increasingly shifting prescription drug costs to patients through higher deductibles, copayments and coinsurance rates, raising concerns about medical financial hardship and patient access to prescription medications.
- Access to prescription drugs varies substantially by age in the United States. Two times as many younger adults ages 18-64 years than elderly adults (ages 65 years and older) report skipping doses, taking less medication, or delaying filling prescriptions because of cost in the past 12 months (9.7 percent vs. 4.5 percent) despite their lower prevalence of chronic conditions and medical need.
- Among adults ages 18-64 years, the prevalence of not taking drugs as prescribed because of cost was 9.7 percent in the United States overall and varied by insurance program: Medicare Part B (23.1 percent), Part D (23.3 percent), Medicaid (11.5 percent), VHA (5.8 percent), private insurance (6.7 percent), and uninsured (17.6 percent). Adjusting for patient characteristics, such as comorbidity, that vary across programs, significantly impacted estimates of prescription drug access. Adjusted estimates of not taking drugs as prescribed because of cost became closer to those of the general population: Medicare Part B (11.0 percent), Medicare Part D (10.3 percent), Medicaid (6.0 percent), VHA (4.1 percent), and private insurance (7.8 percent). Problems with access to prescription drugs remained elevated for the uninsured (16.4 percent).
- Among adults aged 65 years and older, prevalence of not taking drugs as prescribed because of cost was 4.7 percent in the United States overall and varied little across

insurance program: Medicare Part B (4.8 percent), Medicare Part D (5.4 percent), Medicaid (6.1 percent) and VHA (2.9 percent). Adjusting for patient characteristics had little impact for those with Medicare Part B, Part D, or VHA coverage, although not taking prescription medication because of cost declined for Medicaid enrollees from 6.1 percent to 3.2 percent following adjustment.

- The percentage of individuals not taking drugs as prescribed because of cost declined between 2011 and 2014, from 12.5 percent to 7.0 percent of individuals ages 18-64 and from 5.7 percent to 4.4 percent of individuals ages 65 years and older. Among Medicare Part D beneficiaries ages 65 years and older, the percentage not taking prescription drugs because of cost declined from 6.9 percent to 5.2 percent between 2011 and 2014.
- Patient assistance programs, individual drug couponing, and savings card programs are increasingly common as a means to reduce patient out-of-pocket cost and increase access to prescription drugs. However, the federal anti-kickback statute prohibits Medicare, Medicaid, and VHA beneficiaries from participating in programs sponsored by pharmaceutical companies or using coupons or savings card programs that apply only to a specific drug.

Overview of access to health care

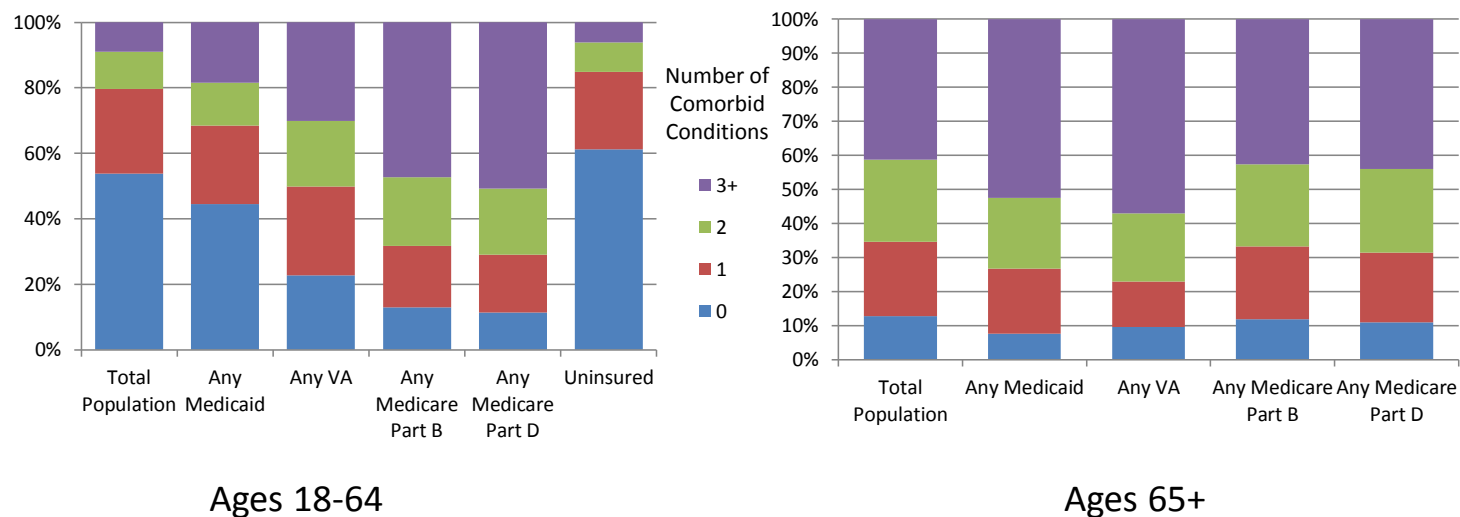
In the landmark Institute of Medicine Report, *Access to Healthcare in America* [1], access to health care was defined as “the timely use of personal health services to achieve the best possible health outcomes”. This definition encompasses both the ability of individuals to obtain needed medical services and the potential for those medical services to improve health. The research literature has consistently shown that individuals in the United States who have access to health care have lower levels of unmet health needs [1], fewer potentially avoidable hospital admissions [1, 2] and lower rates of preventable deaths [1].

Patient characteristics, including minority race/ethnicity, living in poverty, limited educational attainment, and other measures of lower socioeconomic status are associated with less access to health care [3]. Historically, having and maintaining health insurance coverage have been a strong patient-level predictor of having a usual source of care [4], receipt of recommended preventive services [4-6] and diagnosis and effective treatment of chronic conditions [4, 7]. The uninsured are at greater risk of poor health outcomes [4, 8] and mortality than their counterparts with health insurance, even after considering other risk factors [4, 9].

Patient age is another important predictor of access to medical care, in part, because the elderly have higher levels of chronic conditions and poorer health [3] and thus more health care needs on average than younger populations [10]. As shown in Figure 1, approximately 40 percent of individuals ages 65 and older have three or more reported medical conditions, whereas less than 10 percent of individuals ages 18-64 years have three or more medical conditions. Conversely, 54 percent of individuals ages 18-64 do not have any chronic conditions, compared to only 13 percent of individuals ages 65 years and older. As might be expected with higher levels of chronic conditions, the prevalence of prescription drug use is highest among those aged 65 and older – 90 percent take at least one prescription drug and 39 percent take five or more prescription drugs [11].

Figure 1

Characteristics of US Population by Age and Type of Health Insurance: Comorbidity



NOTE: Insurance categories are not mutually exclusive

Estimates are from NHIS 2011-2014

Comorbid condition count includes: arthritis, asthma, cancer, diabetes, emphysema, coronary heart disease, hypertension, stroke, angina pectoris, and heart attack

Age can also reflect differences in insurance coverage between younger and older populations (i.e., 18-64 years and 65+ years). Although the absolute number of uninsured has declined substantially since the passage of the Affordable Care Act with the introduction of Marketplace coverage and Medicaid expansions [12], the vast majority of the remaining uninsured are younger than 65 years. About 94 percent of the U.S. population ages 65 years and older receives health insurance through the Medicare program, whereas health insurance for the younger working age population is predominantly employment-based private insurance [13]. Individuals under the age of 65 years are eligible for Medicare only because of permanent disabilities. Medicaid eligibility for both age groups is based on having income below federal poverty levels, although the threshold for eligibility can vary by state for the younger age group. Basic eligibility for coverage through the VHA is based on completed active military service, but other factors influence enrollment, including medical need and status-based criteria.

These differences in eligibility criteria for government health insurance programs are reflected in the levels of comorbidity and health care needs within each program, especially for the adult population ages 18-64 years. As shown in Figure 1, nearly 50 percent of younger Medicare beneficiaries ages 18-64 years report three or more conditions compared to about 10 percent in the United States population ages 18-64 years overall. Prevalence of three or more chronic conditions in the population with Medicaid (18 percent) and VHA (30 percent) coverage is also higher than the overall population in this age group. Notably, the population without health insurance is less likely to have multiple chronic conditions than the overall population.

In the overall United States population ages 65 years and older, 41 percent of individuals report three or more chronic conditions; 24 percent report two conditions; 22 percent report a single condition and 13 percent do not report any chronic conditions. The prevalence of 3 or more chronic conditions was higher for those with Medicaid (57 percent) and VHA coverage (53 percent), and closer to the overall population ages 65 years and older for those with Medicare Part B or Medicare Part D. All of these differences in population characteristics affect medical need and utilization, especially with respect to prescription medication. Because prescription drugs can be used to prevent and effectively treat both acute and chronic disease, access to prescription drugs plays a key role in improving health.

Access to prescription drugs

The most common measures of access to prescription drugs are related to skipping prescription drug doses, taking less medicine, or delaying filling a prescription due to cost [14-17]. These measures are collectively referred to as not taking prescription medication as prescribed due to cost [14, 18] or cost-related medication non-adherence [14, 15]. Low income, poor health status, and high levels of comorbidity are patient-level factors associated with problems with access to prescription drugs [14]. Lack of insurance coverage is one of the most consistent barriers of access to prescription drugs [14, 18]. However, even among individuals with prescription drug insurance coverage, those taking expensive medications or with higher cost-sharing and out-of-pocket costs are more likely to have problems with access to prescription drugs [14, 19]. Even if individual prescriptions are not particularly expensive, the cumulative out-of-pocket costs for multiple prescriptions may present a barrier to access.

Several recent trends have increased financial barriers to access in taking prescription drugs as prescribed. The list price for some categories of prescription drugs has increased dramatically, particularly for conditions such as cancer, rheumatoid arthritis, hepatitis C, multiple sclerosis, and macular degeneration. Many of these drugs are referred to as “specialty” drugs, and have prices of more than \$100,000 per year. In addition, insurers are increasingly shifting prescription drug costs to patients through higher deductibles, copayments, and coinsurance rates [20, 21]. Since 2010, health plan deductibles have risen by as much as 66 percent [20]. Patient out-of-pocket costs have risen by as much as 46 percent [21] over the past decade. For some conditions such as cancer, these drugs are increasingly used in combination (i.e., more than one specialty drug) along with other supportive agents [22]. As a result, medical financial hardship, also known as “financial toxicity,” is increasingly documented in the U.S. [23], especially in relation to prescription drug use.

Cost-related problems with access to prescription drugs can also have important implications for receipt of other care and patient outcomes. Studies have documented that individuals with problems with access to prescription drugs have higher risk of conditions such as myocardial infarction and stroke [24]. These individuals are also more likely to have emergency room visits [16] and ambulatory care sensitive or preventable hospitalizations compared to their counterparts without access problems [24, 25]. Chapters 9 and 10 of this report evaluate the relationships between access to prescription drugs and patient satisfaction with health care and health outcomes, respectively. Recognizing the importance of access to prescription drugs, the Healthy People 2020 national objectives for improving the health of all Americans includes the measure of reducing the proportion of the U.S population who report delaying or forgoing prescription drug medication [26]. Increasing the proportion of individuals with prescription drug insurance is a Healthy People 2020 developmental measure for evaluating progress in improving access to care [26].

Patient out-of-pocket payments for prescription drugs

Health insurance typically covers a portion of the price of prescription drugs and patients are responsible for the remainder. Patient out-of-pocket payments for an individual prescription drug consist of copayments or coinsurance after any deductible has been met. Copayments are a

fixed amount for a prescription drug and coinsurance is the patient's share of the retail price of a covered prescription drug. Medicare Part D and some private insurance plans also have monthly premiums in addition to any deductibles and copayments and coinsurance for prescription drugs.

Many insurers use formularies or prescription drug lists, which are lists of covered prescriptions. Formularies can have multiple tiers with different levels of cost-sharing using either copayments or coinsurance. For example, in the first tier, a preferred generic drug copayment might be \$2; in the second tier, a non-preferred generic drug copayment might be \$6; in the third tier, a preferred brand copayment, \$40; and in the fourth tier, a non-preferred brand copayment, \$90 or a coinsurance payment of 25 percent. In the fifth tier, a specialty drug coinsurance rate of 20-33 percent of the retail price of the drug is common [20]. The percentage of plans and insurers using formularies with five tiers and using coinsurance for the non-preferred brand tier has increased in recent years [20]. In addition, plans are increasingly using higher coinsurance rates for the specialty tier [20]. Patients taking specialty drugs with list price of more than \$100,000 annually would face \$20,000-\$33,000 a year in cost sharing.

A summary of prescription drug coverage and patient out-of-pocket costs for Medicare Part B, Medicare Part D, Medicaid, and the VHA is described below.

Patient cost-sharing by program

As noted in Chapter 3, *Medicare Part B* generally covers prescription drugs administered by injection or infusion in physicians' offices or hospital outpatient departments. Some oral medications are also covered under Part B. Patients pay 20 percent coinsurance of the Medicare approved amount (average sales price (ASP) + 6 percent) for covered drugs. Medicare Part B does not have a cap on patient out-of-pocket payments, but many beneficiaries purchase supplemental insurance or have secondary insurance through their former employers.

Medicare Part D began covering oral prescription drugs in January 2006 as described in Chapter 4. Enrollees in Part D pay a monthly premium in addition to any deductible and prescription specific cost-sharing for their drugs. Low-income beneficiaries (including those dually eligible for Medicaid) pay lower or no premiums, cost sharing, or deductibles. A key piece of patient cost-sharing for Medicare Part D is the "donut hole", or the Part D coverage gap. Prior to the passage of the ACA in 2010, the standard Part D benefit included a deductible, 25 percent coinsurance for drug spending up to a spending threshold, at which point a temporary coverage gap required paying 100 percent coinsurance for any drug spending until the catastrophic coverage threshold was reached. After beneficiaries exceeded the catastrophic threshold, coinsurance was generally 5 percent of drug costs for the remainder of the year. The ACA enacted changes to the Medicare drug benefit, most notably reducing beneficiary cost-sharing in the coverage gap. After spending is high enough for catastrophic coverage to begin, coinsurance remains at 5 percent coinsurance for the calendar year.

In 2015, more than 80 percent of Medicare Part D enrollees were in plans with formularies with 5 tiers and more than 57 percent had specialty drug tier coinsurance rates of 33 percent [20]. Medicare Part D has no annual limit or cap on out-of-pocket spending.

Medicaid covers oral and implanted, infused, inhaled, injected and instilled prescription drugs as described in Chapter 5. Although pharmacy coverage is an optional benefit under federal

Medicaid law, all states and the District of Columbia currently provide coverage for outpatient prescription drugs to all categorically eligible individuals and most other enrollees within their respective Medicaid programs. Starting January 1, 2006, Medicaid enrollees who also receive Medicare benefits no longer receive their pharmacy benefits under their state Medicaid agency, except for drugs not covered under Medicare Part D. Each state has the option to cover these drugs for their Medicaid beneficiaries who also have Medicare coverage.

Cost-sharing is generally limited to \$4 for preferred drugs and \$8 for non-preferred drugs. States can charge enrollees with family incomes above 150 percent of the Federal Poverty Level up to 20 percent of the cost of a nonpreferred drug, but this option has been difficult to implement because pharmacies typically do not have information on family income (subject to inflation adjustments) [27]. States are not required to charge the full cost-sharing amounts permitted, and as of 2012, nine states imposed no patient copayments at all [28].

Chapter 6 describes VHA insurance coverage of both oral and infusion prescription drugs. The VHA has an established formulary consisting of medications that demonstrate high quality along with good value. The formulary is sorted by drug class, and drugs are listed by generic name as available. VHA beneficiaries can also enroll in Medicare Part D or other prescription drug coverage programs to supplement their VHA prescription coverage. The VHA does not stratify drugs into tiers, but maintains a formulary of preferred drugs covered at no cost for treatment of service-related conditions or for patients who fit certain other criteria. Copayments, when applicable, are set at fixed rates. There is no yearly out-of-pocket cap on patient cost-sharing.

The uninsured are responsible for the entire price of prescription drugs. Patient assistance programs, individual drug couponing, pharmacy-specific purchasing agreements, and savings card programs are increasingly common as a means to reduce patient out-of-pocket cost and increase access to prescription drugs [29-31], although data about their use are not systematically collected. While these patient assistance programs and brand-name drug couponing programs might appear to be beneficial for the uninsured, many programs require patients to have private insurance to be eligible to participate. In addition, due to the federal anti-kickback statute, Medicare, Medicaid, and VHA beneficiaries are not eligible to participate in programs sponsored by pharmaceutical companies or use coupons or savings card programs that apply only to a specific drug. Thus, the potential benefits of these programs in improving access to prescription medication are limited in scope. In addition, few data are available to evaluate their use.

Analyses of Access to Prescription Drugs by Government Program

The analyses conducted for this report used multiple years of publicly available nationally representative data that serve as the source for monitoring population health in Healthy People 2020. Health insurance coverage was measured with validated measures. Access to prescription drugs was measured consistently across programs using standard metrics, and the same methods and measures were used to evaluate prescription drug access across the four government insurance programs (Medicare Part B, Medicare Part D, Medicaid and the VHA).

Data sources

Data from the National Health Interview Survey (NHIS), an annual in-person household survey of health, health insurance, and access to care conducted in the U.S. civilian, non-institutionalized population, were used in the analyses of access to prescription medication. The NHIS was created by the National Health Survey Act of 1956 with the goal of providing “statistical information on the amount, distribution, and effects of illness in the United States and the services rendered for or because of such conditions” [32]. The NHIS serves as the primary source of information on the health of the U.S. population and is currently used to monitor progress towards achieving many national health objectives put forth by Healthy People [33]. The NHIS has a very high response rate and sample weights reflect complex survey design and probability of non-response. Multiple years of the NHIS were pooled to ensure sufficient numbers of individuals with insurance coverage from each of the program to produce stable estimates for this report.

Measures

The NHIS is an in-person household survey, which allows the interviewer to query the respondent directly about their answers to the survey. In the case of questions about health insurance coverage, interviewers also examine health insurance cards. This approach maximizes the accuracy of reporting about every respondent’s health insurance coverage. For these analyses, individuals were classified as having any Medicaid, any Medicare Part B, any Medicare Part D, any VHA, any private insurance, or being uninsured. Because some individuals have more than one source of health insurance coverage, these categories were not mutually exclusive.

For the purposes of this report, measures of access to prescription drug coverage used standard definitions. In the NHIS, respondents were asked the following questions: “During the past 12 months, were any of the following true for you? 1) You skipped medication doses to save money; 2) You took less medicine to save money; and 3) You delayed filling a prescription to save money.” A dichotomous summary measure reflecting endorsement of any of these three behaviors was created as an indicator of either taking or not taking the medication as prescribed because of cost. This measure has also been used to report cost-related non-adherence [18]. Another NHIS question asked if there had ever been a time that the respondent could not afford prescription medication in the past 12 months. These two measures of prescription drug access were evaluated separately. Findings related to inability to afford prescription drugs in the past year are reported in the Appendix for Chapters 8-10.

Statistical methods

Estimates of access to prescription medications are reported for the total U.S. population and separately for each of the four government health insurance programs. All analyses were stratified by age group (18-64, 65+) to reflect differences in the prevalence of comorbidity and eligibility for health insurance. Within each program, estimates of access to prescription drugs are presented with and without adjustment for patient factors that vary across health insurance programs and that are also associated with problems with access to prescription drugs. For example, Medicare beneficiaries younger than 65 years are eligible for coverage because of their disability and will differ substantially in terms of medical need from their counterparts with other

types of insurance coverage. To address these differences in patient characteristics across programs, estimates were adjusted for the effects of individual characteristics (age, gender, race/ethnicity, educational attainment, marital status, family income, number of chronic conditions, and geographic region) and survey year with multivariable logistic regression models. For ease of interpretation, predicted margins estimated from the odds ratios from the logistic regressions are reported, which are interpreted as adjusted population percentages [34].

Access to Prescription Drugs by Government Program

Among adults ages 18-64 years in the United States, 9.7 percent reported skipping doses, taking less medication, or delaying filling prescription drug medications because of cost in the past 12 months (Figure 2). Estimates of not taking drugs as prescribed because of cost were slightly higher for individuals with Medicaid coverage (11.5 percent) and lower for individuals with coverage through the VHA (5.8 percent) or with private insurance (6.7 percent). For Medicare beneficiaries ages 18-64 years with Part B or Part D coverage, problems with access to prescription medications were substantially higher (23 percent). Because insurance coverage is categorized based on status at the time of interview and prescription drug access is measured using a twelve-month lookback period, some individuals may be reporting access problems from before they had their current coverage. Approximately 18 percent of the uninsured reported problems with access to prescription drugs.

When estimates are adjusted for individual level characteristics that differ between Medicare, Medicaid, VHA, and uninsured populations and the United States population as a whole, such as number of chronic conditions, problems with access to prescription drugs are less discrepant between programs, ranging from about 4 percent for individuals with VHA coverage to about 11 percent for individuals with Medicare Part B or Part D (Figure 2). For individuals without any health insurance, 16.4 percent reported not taking prescription drugs as prescribed.

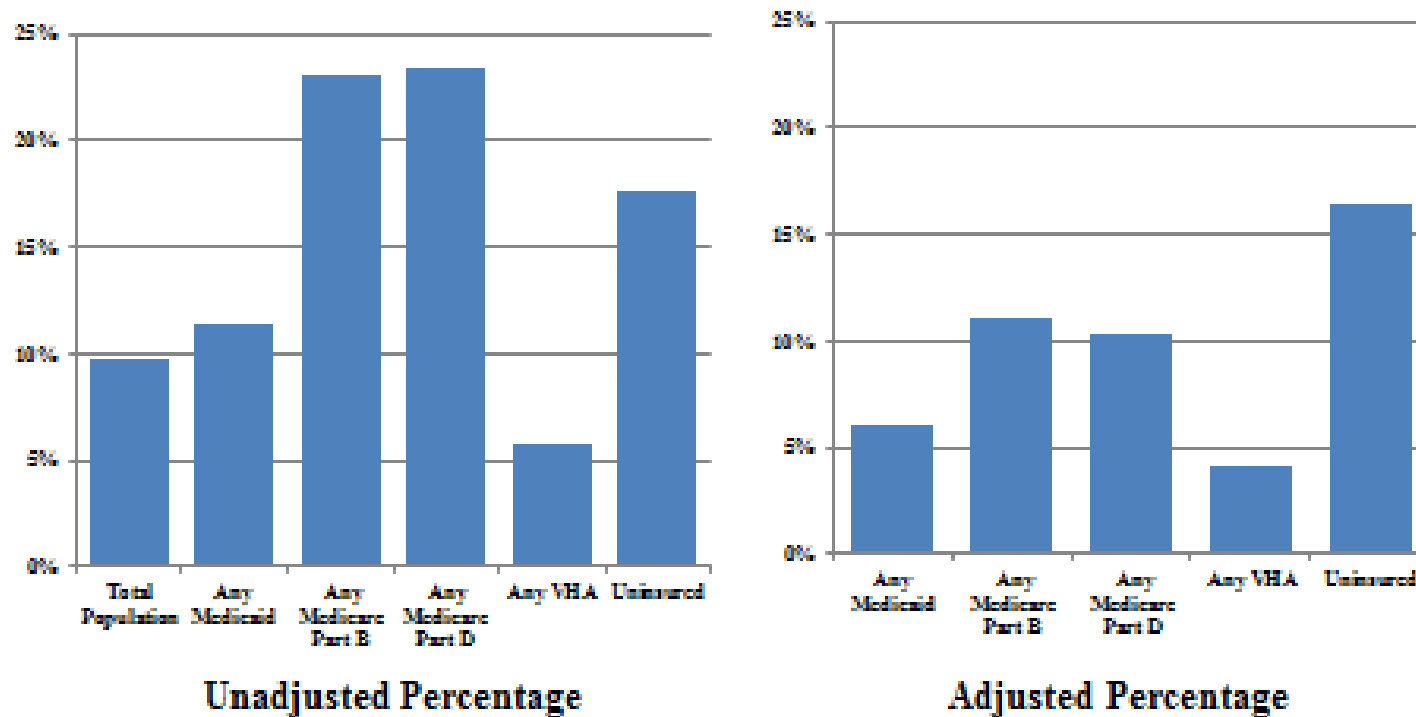
Among individuals aged 65 years and older in the United States, 4.7 percent reported skipping doses, taking less medication, or delaying filling prescription drug medications because of cost in the past 12 months (Figure 3). Problems with access to prescription drugs measured by not taking medication as prescribed because of cost or not being able to afford prescriptions varied little for individuals with Medicare Part B (4.8 percent), Medicare Part D (5.4 percent), and VHA coverage (2.9 percent). Adjusting for individual level characteristics that vary between the programs and the U.S. population had little impact for those with Medicare Part B, Part D, or VHA coverage (Figure 3). For individuals with Medicaid coverage, not taking prescription medication because of cost declined from 6.1 percent to 3.2 percent following adjustment.

Findings for the other measure of prescription drug access, not being able to afford prescription medication in the past 12 months, were virtually identical in both age groups.

Despite lower prevalence of chronic medical conditions described in Figure 1, younger adults ages 18-64 years, were twice as likely as those 65 years and older to report not taking prescription drugs as prescribed due to cost. Similarly, the uninsured, who have the lowest prevalence of chronic conditions of any of the population groups evaluated, had the highest level of not taking prescription drugs as prescribed due to cost even after adjustment for individual characteristics.

Figure 2

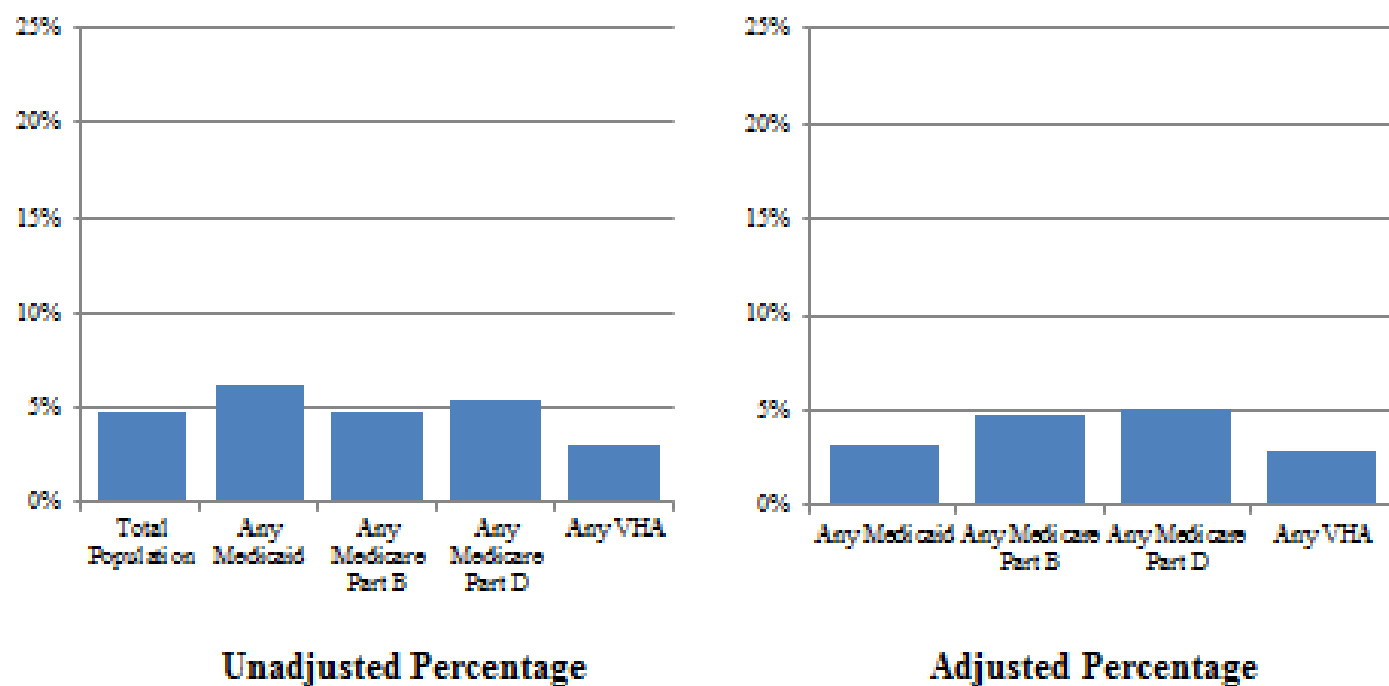
Problems with Access to Prescription Medication Past 12 Months by Program, Ages 18-64 Years



NOTE: Estimates are from National Health Interview Survey (NHIS) data 2011-2014.
Insurance categories are not mutually exclusive.

Figure 3

Problems with Access to Prescription Medication Past 12 Months by Program, Ages ≥ 65 Years



NOTE: Estimates are from National Health Interview Survey (NHIS) data 2011-2014.
Insurance categories are not mutually exclusive.

Trends in access to prescription drugs

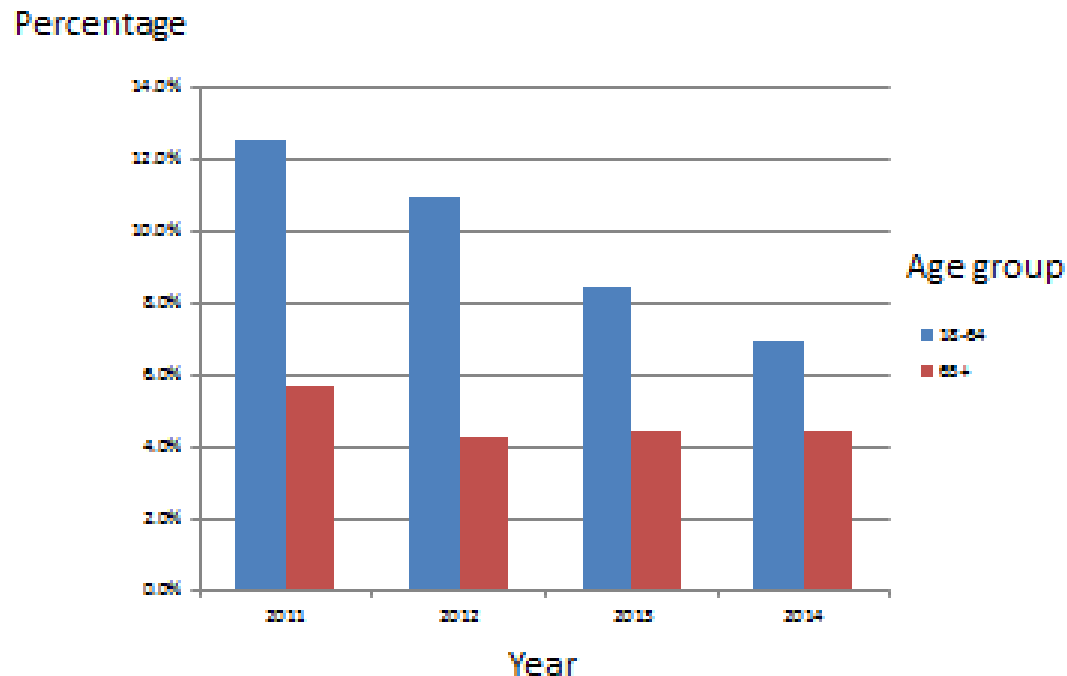
Starting in 2010 with the passage of the Affordable Care Act, the number of individuals in the U.S. who were uninsured declined with the introduction of Marketplace coverage and Medicaid expansions [12]. Other provisions of the ACA related to individuals of all ages, including elimination of pre-existing condition restrictions and premiums based on health conditions and requirements for private health insurance plans to cover recommended preventive services without cost-sharing, may also reduce patient out-of-pocket costs. Another provision of the ACA is leading to the gradual closing of the Medicare Part D coverage gap, or donut hole. Additional research shows that between 2013 and 2014, uninsured individuals who gained private insurance filled 28 percent more prescriptions and had 29 percent lower out-of-pocket spending per prescription. Uninsured individuals who gained Medicaid coverage filled 79 percent more prescriptions and had 58 percent lower out-of-pocket spending per prescription [35]. Between 2003 and 2014, median monthly out-of-pocket spending for privately insured users of non-specialty drugs has declined, even though patient out-of-pocket spending for specialty drugs has increased [21]. Because a relatively small proportion of individuals use specialty drugs, these findings are consistent with overall improvements in patient access to prescription drugs, despite increasing prescription drug spending.

As shown in Figure 4, the percentage of individuals experiencing problems with access to prescription drugs also declined between 2011 and 2014. Approximately 13 percent of individuals ages 18-64 years reported skipping medication doses, taking less medicine, or delaying filling prescriptions because of cost in 2011 compared to 7 percent in 2014. For individuals ages 65 years and older, about 6 percent reported problems with access to prescription drugs in 2011 compared to about 4 percent in 2014. Findings were similar when the sample was restricted to the elderly Medicare Part D beneficiaries – the percentage not taking prescription drugs because of cost declined from 6.9 percent to 5.2 percent. Trends in smaller percentage of individuals reporting limited access to prescription drugs were statistically significant.

Trends in not being able to afford prescription medication in the past 12 months were virtually identical in both age groups.

Figure 4

Percentage of Individuals Skipping Prescription Drug Doses, Taking Less Medicine or Delaying Filling Prescriptions in past 12 months Because of Cost



NOTE: Estimates are from National Health Interview Survey (NHIS) 2011-2014

P-value for trends: $p < 0.001$ ages 18-64 and $p = 0.003$ for 65+

Time interval between patient attempts to fill a prescription and receipt of prescription drugs

As noted in the Institute of Medicine definition of access to medical care [1], timeliness is also a component of access. In the case of retail prescription drugs, the time interval between when a patient attempts to fill a prescription drug and actual receipt of the drug may also be considered a component of access to care. However, data about the time interval between attempting to fill a prescription and its receipt by the patient or the underlying reasons for any delays in receipt of prescriptions are not systematically collected across insurance programs and numerous factors complicate attempts to estimate these data.

Patient forgetfulness, for example, may play an important role in longer times between attempts to fill a prescription and the actual receipt of a drug. Further, any evaluation of the time interval is complicated by the increased prevalence of electronic prescriptions, or e-prescriptions, a service by which physicians electronically record the prescription and automatically send it to the pharmacy to be filled. In addition to differences between the start time of e-prescriptions and paper prescriptions that patients take to the pharmacy, *all* prescriptions written at a doctor's office would be considered in timing of e-prescriptions, whereas many paper prescriptions are never recorded by the pharmacy because the patient never submitted them to begin the process. Mail-order pharmacies are increasingly common as well, further complicating any comparisons of time it takes to receive prescription medication. In the few studies that have been conducted of traditional prescriptions where patients receive a written prescription, submit it to the pharmacy, and then receive it, wait time at the pharmacy averages 15-30 minutes [36-38].

A related measure to the time interval for receipt of prescriptions is the rate at which prescriptions are submitted to the pharmacy, but never picked up, referred to as "prescription abandonment" [39]. In the limited research that has been conducted, both e-prescriptions and traditional paper prescriptions for drugs with higher out-of-pocket costs [39] [40] or those that are not listed on a formulary [41, 42] were more likely to be abandoned. Branded drugs were also more likely to be abandoned than generics [39]. Some categories of drugs, such as opiates, blood pressure medications, statins and oral diabetic drugs were at low risk for abandonment (i.e., <2 percent) compared to insulin and asthma medications (2.2-3.5 percent). Medications for cough and cold were at the highest risk for abandonment (3.6 percent) [39]. Patient characteristics associated with abandonment are low income [40] and younger age [39, 40]. Forgetfulness may also play a role in drug abandonment. Several studies have evaluated insurance coverage and prescription drug abandonment, but these provide limited information about differences by insurance program because they were conducted within a single organization (e.g., pharmaceutical benefits manager) and did not always consider key patient features that vary by insurance program (e.g., age, level of comorbidity, income level).

Summary

As described in this chapter, access to prescription drugs is a key component of health care delivery in the United States. Individuals with limited access to prescription medications are more likely to have preventable hospitalizations and poorer health outcomes than similar individuals who are able to access and take prescription medications as prescribed. One of the strongest predictors of access to prescription drugs is health insurance coverage. The uninsured

are most likely to report skipping medication doses, taking less medication or delaying filling prescription drug medications due to cost. However, even among individuals with health insurance, those with higher cost sharing and out-of-pocket costs are more likely to delay initiating prescription drugs and discontinue taking them as prescribed, making the structure of prescription drug coverage a key feature in improving access.

The quantitative analyses conducted specifically for this report used multiple years of nationally representative data and measures of prescription drug access used in Healthy People 2020 to monitor population health. Importantly, the same methods and measures were used to evaluate prescription drug access across government insurance programs, including Medicare Part B, Medicare Part D, Medicaid and the VHA, and findings could thus be compared with the U.S. population overall. In evaluating access to prescription drugs across insurance programs, skipping doses, taking less medication, or delaying filling prescription drugs was found to vary substantially by age. Although adults ages 18-64 years have lower prevalence of chronic conditions and medical need than adults ages 65 years and older, more than twice as many younger adults reported problems with access in the past 12 months. Not taking drugs as prescribed because of cost was also shown to vary by insurance program, especially in the younger group, reflecting different levels of medical need across program. Adjusting for patient characteristics that vary across programs had an important effect on findings about prescription drug access: estimates for Medicare Part B, Medicare Part D, Medicaid and VHA became closer to those for the U.S. population overall. Problems with access to prescription medication remained highest for the uninsured, however, even after adjustment for other patient characteristics.

Important trends over time in improved access to prescription drugs were observed in these analyses as well. Despite increasing spending on prescription drugs, the percentage of individuals not taking drugs as prescribed declined between 2011 and 2014 in both age groups, with larger declines for adults ages 18-64 years than for older adults. These changes likely reflect the Affordable Care Act's expansions in health insurance coverage that includes prescription drugs, and the closing of Medicare Part D coverage gap under the Affordable Care Act.

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CHAPTER 9: SATISFACTION WITH HEALTH CARE

This chapter presents information on satisfaction with health care in the United States and separately for individuals with coverage under four government programs: Medicare Part A, Medicare Part B, Medicaid, and the Veterans Health Administration (VHA). Information from the published literature is provided about patient satisfaction with care and its associations with prescription drug coverage as well as findings from analyses of the National Health Interview Survey (NHIS) data conducted specifically for this report.

Key Findings

Adults in the United States report high levels of satisfaction with health care received in the past 12 months.

- Fewer than 10 percent of adults ages 18-64 reported dissatisfaction with health care. More than 80 percent of adults in this age group who have any Medicare Part B or Part D, any Medicaid, or any VHA coverage reported being satisfied with health care they received. A much lower percentage of the uninsured (44.0 percent) reported being satisfied with health care, but this was due mainly to the fact that nearly half (46.8 percent) of uninsured adults ages 18-64 reported no health care in the past 12 months rather than substantially higher levels of dissatisfaction (9.2 percent). After adjustment for sociodemographic factors, the percentages of adults expressing satisfaction with health care increased slightly for most groups.
 - Satisfaction varied little by insurance program for the population ages 65 years and older, with at least 89 percent of those with any Medicare Part B or Part D, any Medicaid, or any VHA coverage reporting being satisfied with health care. Adjustment for sociodemographic factors that vary across programs had very little effect on these percentages.
 - Satisfaction with health care was highest for individuals with access to prescription drugs and lowest for those who reported not taking medication as prescribed due to cost. This finding was consistent across age groups and by insurance program.
-

Overview of Satisfaction with Health Care

In 2001, the Institute of Medicine issued a report titled “Crossing the Quality Chasm” that called for fundamental changes to the health care delivery system in the United States. It noted that patient needs would best be met by high-quality care that was patient-centered, safe, effective, timely, efficient, and equitable [1]. Patient-centered care is defined as “care that is respectful of and responsive to individual patient preferences, needs, and values and ensuring that patient values guide all clinical decisions” [1]. Patient satisfaction has been used as an indicator of patient-centered care and is also recognized as an important outcome in and of itself [2].

Patient satisfaction^{aaa} is a subjective evaluation of health care services and providers [7]. It has been defined as the gap between patient expectations and experiences [8] and highlighted as an important indicator of quality of care [9]. While there is widespread agreement that patient satisfaction is a multi-dimensional construct [3, 10, 11], there is little agreement about the best way to measure it. Attempts have been made to note how the term “satisfaction” was operationalized or defined in each of the studies cited in this report.

Results of studies exploring the association between patient-reported satisfaction and objective measures of quality of care have been mixed. One study reported that hospitals with the highest patient satisfaction scores (measured as the percentage of patients who reported they would recommend the hospital to family and friends) had the highest scores on a composite measure of surgical quality that took into account length of stay, surgical care processes, 30-day readmission rate, and perioperative mortality [12]. However, other studies exploring specific utilization and mortality outcomes have yielded different conclusions. For example, one study found that hospitals with higher overall patient satisfaction scores (based on hospital ratings and likelihood of recommendation) had lower 30-day readmission rates for acute myocardial infarction, heart failure, and pneumonia among patients ages 18 and older [13], but another using the same definition of patient satisfaction failed to find an association of patient satisfaction scores with 30-day hospital readmission among patients ages 65 and older who had undergone inpatient surgery [14]. Whereas this latter study did find a negative association between patient satisfaction and odds of death 30 days post-operation [14], a cohort study of more than 50,000 adults showed higher mortality among patients with the highest patient satisfaction scores (measured as quality of physician communication and a subjective rating of quality of care (worst to best)) [15]. Differences in the associations between quality of care and patient satisfaction previously reported are likely due, at least in part, to inconsistent definitions of patient satisfaction.

Patient satisfaction with health care is not monitored at the national level. Perhaps accordingly, with minor exception [16], very few studies have explored differences in patient satisfaction with medical care across health insurance programs. Nevertheless, some information can be gleaned from national opinion polls. In response to a 2014 Gallup poll, 66 percent of U.S. adults indicated that they were satisfied with how the health care system was working for them. However, there were large differences by whether or not individuals had health insurance: 70

^{aaa} Over the past few decades, several concerns have been raised regarding the construct of patient satisfaction. In the early 1990s, researchers argued that reports of patient satisfaction reflected not only the care received but also the preferences and expectations of the patient [3]. Thus, different levels of patient satisfaction may reflect not only differences in care but also differences in patient perspectives [4,5]. Others have pointed to high levels and limited variability in satisfaction reported by patients [6] and hypothesized that expressions of satisfaction may be influenced by multiple motivations leading to biased responses [3]. The Institute of Medicine [1] notes that the evaluation of patient satisfaction is difficult as there are no established criteria for determining an acceptable level of satisfaction. Without such a standard, patient satisfaction ratings and reports cannot provide information about the degree of necessary or possible improvement [1].

percent of respondents with, but only 37 percent of respondents without, health insurance were satisfied with their health care [17].

Overview of Access to Prescription Medication and Satisfaction with Care

In recognition of the public health importance of access to prescription medicines, Healthy People 2020 has set a goal of reducing by the percentage of people who are unable to obtain or who experience delays in obtaining necessary prescription medicines by 10 percent [18]. As noted in Chapter 8, 9.7 percent of the adult population ages 18-64 and 4.7 percent of adults ages 65 and older in the U.S. do not take medication as prescribed due to cost. Much of the research dedicated to prescription medications focuses on health care spending and patient behavioral responses to pricing changes [19, 20] rather than satisfaction with care.

The limited research exploring associations between prescription medication access and patient satisfaction is mixed. One study of 1,200 adults ages 40 and older found 68 percent of patients with at least one insurance-related issue related to filling a prescription reported decreased patient satisfaction as measured by getting upset with the insurance company, doctor, or pharmacist [21]. A much larger national survey of nearly 52,000 adults over an eight-year period found higher patient satisfaction (defined in terms of physician communication and perceived quality of care) to be associated with increased spending on prescription drugs the following year [15].

Since 2006, Medicare Part D has provided prescription drug coverage to Medicare beneficiaries. Enrollment in this program has grown considerably: whereas the percentage of eligible Medicare beneficiaries who were enrolled in Part D grew from 53 percent in 2006 to nearly 72 percent in 2015 [22]. A 2012 survey of Medicare beneficiaries in Northern California found 47 percent were very or extremely satisfied with Medicare Part D. However, among those with a prescription drug plan, fewer than half (40.3 percent) rated the plan as very good or excellent [23].

Analyses of Satisfaction with Health Care by Health Insurance Program

Data source

The National Health Interview Survey (NHIS) was selected as the data source to conduct analyses of health outcomes by health insurance program. The NHIS is an annual nationally-representative household interview survey of the US civilian non-institutionalized population [24]. It was created by the National Health Survey Act of 1956 with the goal of providing “statistical information on the amount, distribution, and effects of illness in the United States and the services rendered for or because of such conditions” [24]. The NHIS serves as the primary source of information on the health of the US population and is currently used to monitor progress towards the achievement of many national health objectives put forth by Healthy People [25]. Two years of the NHIS data were pooled to ensure sufficient numbers of individuals with insurance coverage from each of the programs to produce stable estimates.

Measures

Because the NHIS is an in-person household survey, interviewers can query respondents directly about their answers to the survey questions. Interviewers also examine health insurance cards to maximize the accuracy of reporting about health insurance coverage. Individuals were classified as having any Medicaid, any Medicare Part B, any Medicare Part D, or any VHA. Some individuals have more than one source of health insurance coverage, and as a result, these categories are not mutually exclusive.

Satisfaction with health care was added to the NHIS in 2013. It is assessed with the following question: “In general, how satisfied are you with the health care you received in the past 12 months?” Response options include “very satisfied”, “somewhat satisfied”, “somewhat dissatisfied”, “very dissatisfied”, and “haven’t had health care in the past 12 months”. Responses were then categorized as satisfied, dissatisfied and no health care in past 12 months.

As in other analyses for this report in Chapters 8 and 10, limited access to prescription medication was measured as: 1) skipped medication doses, took less medicine, or delayed filling a prescription due to cost in past 12 months (collectively referred to as not taking medication as prescribed due to cost) and 2) inability to afford prescription medication in the past 12 months. Because results for these two indicators of access to prescription medication were very similar, only results for not taking medication as prescribed due to cost are presented.

Statistical methods

Data from the 2013-2014 NHIS were used to assess patient satisfaction with health care and access to prescription medications. Estimates of satisfaction with care are reported for the total U.S. population and separately for each of the four government health insurance programs. Estimates are also reported for uninsured adults ages 18-64. Within each insurance program, estimates of satisfaction with care are also presented for individuals with and without problems with access to prescription drugs due to cost. All analyses were stratified by age group (18-64, 65+) to reflect differences in the prevalence of chronic conditions and age-related eligibility for the Medicare Program. Estimates of satisfaction were also adjusted for the effects of individual characteristics (age, gender, race/ethnicity, educational attainment, marital status, family income, number of chronic conditions, and region) related to health that vary across insurance programs with multivariable logistic regression models. For ease of interpretation, predicted margins, which are interpreted as adjusted population percentages [26], are reported.

NHIS sampling weights were used in all analyses to incorporate complex survey design and provide nationally representative estimates. All analytic files were created using SAS 9.3. Multivariable logistic regressions were performed using STATA 13.1.

Satisfaction with health care by health insurance program

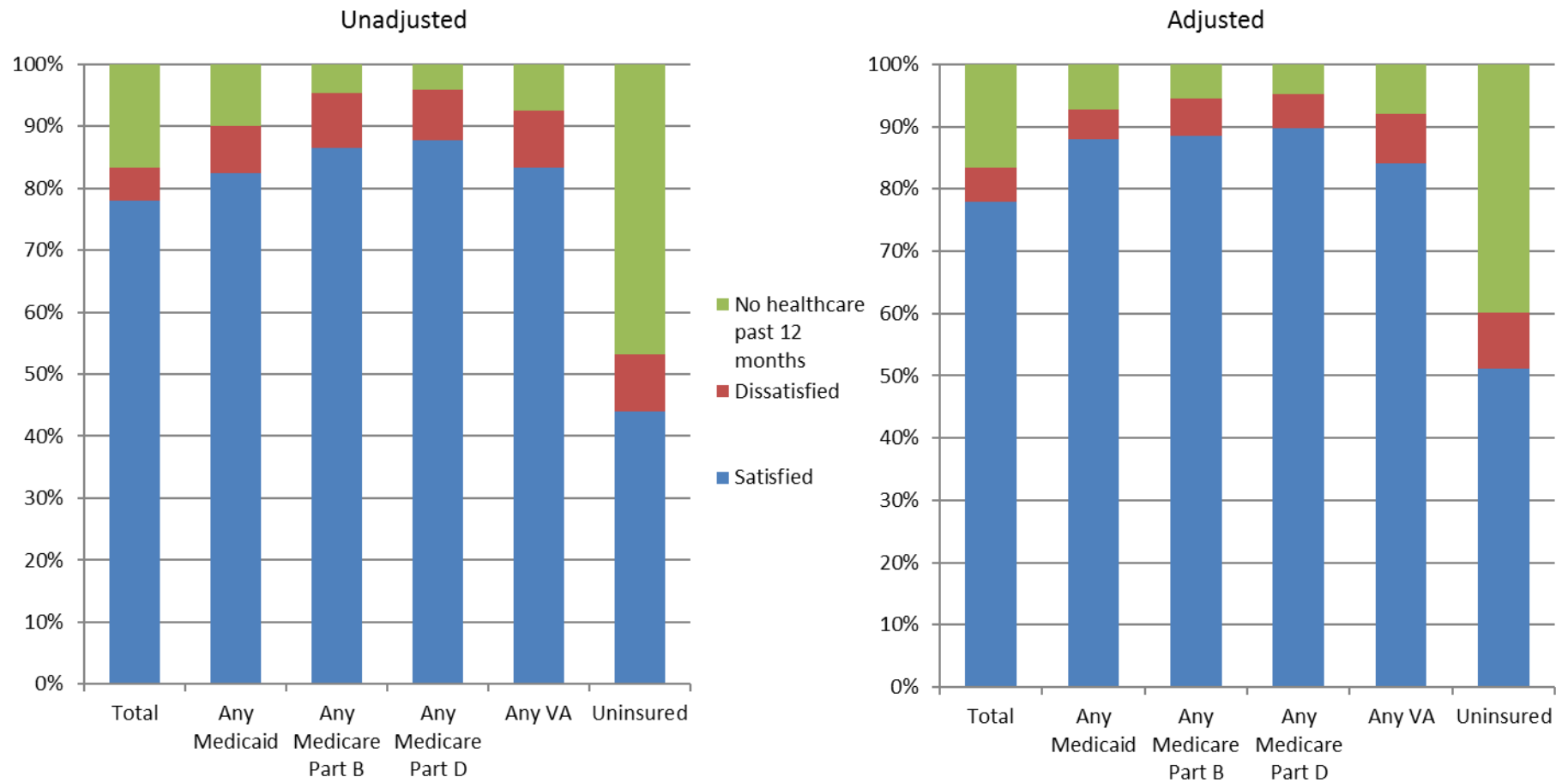
Nearly 80 percent of adults ages 18 to 64 in the total U.S. population reported being satisfied with the health care they received in the past year (Figure 1). Only a small percentage (5.4 percent) reported being dissatisfied with the health care they received and the remainder (16.6 percent) did not receive any health care in the past year. When evaluated by program, more than 80 percent of adults with insurance coverage through Medicaid, Medicare Part B, Medicare Part D, and the VHA report being satisfied with their health care whereas less than 45 percent of the

uninsured reported being satisfied with the health care they received. Notably, more than 45 percent of the uninsured did not receive health care in the past 12 months, and thus could not evaluate satisfaction with health care.

Levels of dissatisfaction with health care were relatively low across programs, ranging from 7.6 percent for those with Medicaid to 9.2 percent for those with VHA coverage. An even higher percentage of adults ages 65 and older in the U.S. population indicated that they were satisfied with the health care they have received in the past year (89 percent or more) (Figure 2). Only 3-5 percent of older individuals with health insurance through Medicaid, Medicare Part B or Part D, or the VHA indicated dissatisfaction with their care in the past year.

After adjusting for patient factors that differ across programs to make the groups covered through different insurance programs more comparable, the percentages of adults ages 18-64 without insurance or with coverage through Medicaid, Medicare Part B or D, and VHA reporting that they were satisfied with the health care received in the past year were higher than in the unadjusted models. Increases were greatest for the uninsured (+7.0 percent) and those with coverage through Medicaid (+5.5 percent) and lowest for those with coverage through the VHA (+0.7 percent). Patterns for adults ages 65 and older did not change appreciably between the unadjusted and adjusted models.

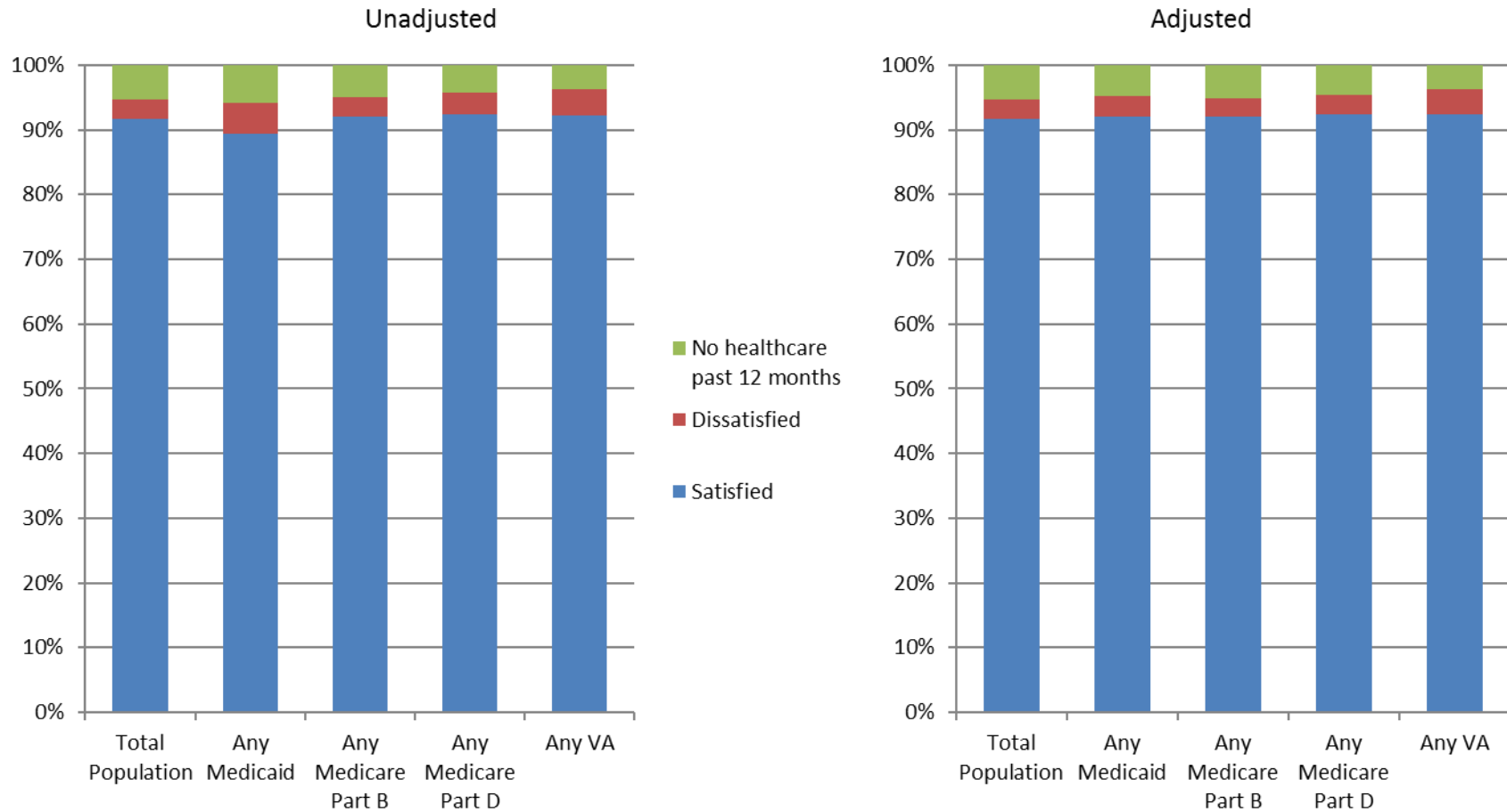
Figure 1. Satisfaction with Health Care by Program, Ages 18-64 Years



Source: NHIS 2013-2014

Note: Insurance categories are not mutually exclusive.

Figure 2. Satisfaction with Health Care by Program, Ages 65 Years and Older



Source: NHIS 2013-2014

Note: Insurance categories are not mutually exclusive.

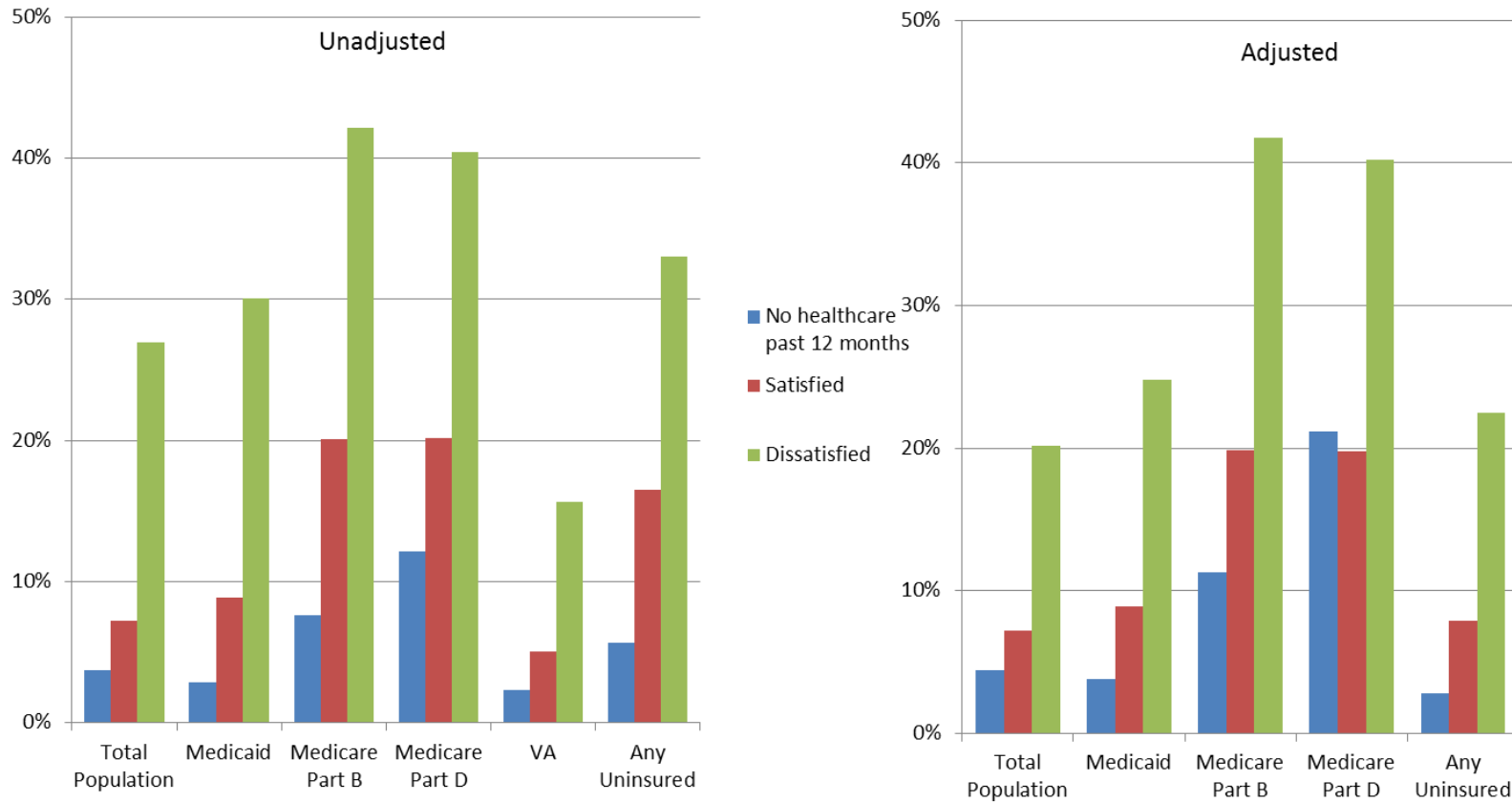
Prescription medication access and satisfaction with health care by program

Higher percentages of adults who were dissatisfied with the health care they received in the past year reported not taking medication as prescribed due to cost than adults reporting that they were satisfied. This pattern held across both age groups, all insurance programs, and the unadjusted and adjusted models. There are some notable differences, however. The percentage of adults ages 18-64 in the total U.S. population who expressed dissatisfaction with their care and reported not taking medication as prescribed due to cost (Figure 3) was approximately twice as high as the percentage of adults ages 65 and older in the total U.S. population who fit these criteria (Figure 4) in the unadjusted and adjusted models alike. Because these data are cross-sectional, it is not possible to determine the causality between access and dissatisfaction. Longitudinal data are required to determine whether patients with limited access to prescriptions drugs later report dissatisfaction or patients who are dissatisfied with care then are less likely to take medication as prescribed due to cost.

Adjusting for patient factors resulted in lower percentages of adults of all ages who were dissatisfied with their care reporting not taking medication as prescribed due to cost across nearly all programs; the only exceptions were adults ages 18-64 with coverage through Medicare Part B or D for whom no differences were observed. Adjusted estimates for individuals covered through the VHA are not provided due to limited sample sizes.

Associations between satisfaction with care and inability to afford prescription medication in the past 12 months among adults ages 18-64 and 65 and older were not qualitatively different from the patterns described above.

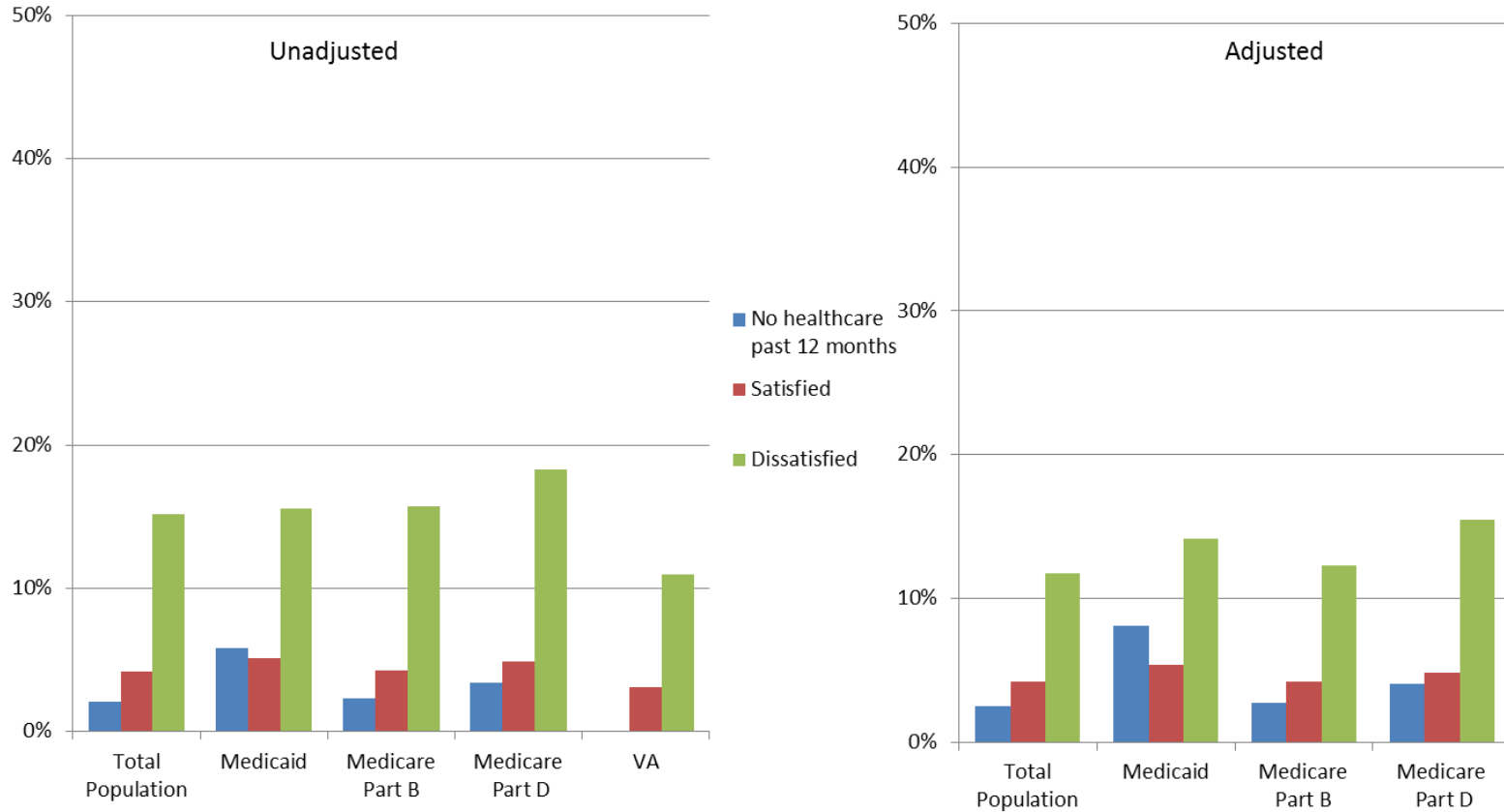
Figure 3. Satisfaction with Health Care and Not Taking Medication as Prescribed Due to Cost by Program, Ages 18-64 Years



Source: NHIS 2013-2014

Note: Insurance categories are not mutually exclusive.

Figure 4. Satisfaction with Health Care and Not Taking Medication as Prescribed Due to Cost by Program, Ages 65 Years and Older



Source: NHIS 2013-2014

Note: Insurance categories are not mutually exclusive.

Summary

Patient satisfaction is a subjective evaluation of health care services and providers and is recognized as an important indicator of quality of care. Research on the link between satisfaction with care and access to prescription medication is limited. There is some evidence to suggest that satisfaction with care is negatively associated with problems filling a prescription and positively associated with spending on prescription drugs.

In the United States, adults express high levels of satisfaction with health care. Results from analyses of National Health Interview Survey data indicate that 77.9 percent of adults ages 18-64 years, and more than 90 percent of those ages 65 years and older, report being satisfied with the health care they have received in the past 12 months. Satisfaction was lowest among uninsured adults ages 18-64 years, with only 44.0 percent reporting satisfaction with health care in the past 12 months. This large difference in satisfaction between the insured and uninsured in this age group was due mainly to lack of health care utilization in the past 12 months among the uninsured, rather than higher levels of dissatisfaction with care received.

Patient satisfaction with care was associated with limitations in prescription drug access. Higher percentages of adults who were dissatisfied with the health care they received in the past year also reported not taking medication as prescribed due to cost as compared to those adults reporting that they were satisfied with their care or had not had any health care in the past 12 months. These patterns held across for adults ages 18-64 and 65 and older, all insurance programs, and in unadjusted and adjusted models.

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CHAPTER 10: PATIENT HEALTH OUTCOMES

This chapter presents information on patient health outcomes in the United States and for individuals with coverage under Medicare Part B, Medicare Part D, Medicaid, and the Veterans Health Administration (VHA) programs. Common measures of health outcomes are described, and a summary of the published research literature is followed with findings from analyses of National Health Interview Survey (NHIS) data conducted specifically for this report.

Key Findings

- Almost 90 percent of adults ages 18-64 years reported good, very good, or excellent health, with the following variation in good-to-excellent self-reported health by insurance program: Medicare Part B (37.5 percent) or Part D (36.0 percent), Medicaid (69.5 percent), VHA (67.8 percent), and uninsured (86.9 percent). After adjustment for individual characteristics that vary by program, self-reported health was more similar across programs, with at least 70 percent reporting good, very good, or excellent health: Medicare Part B (70.0 percent) or Part D (71.3 percent), Medicaid (82.6 percent), VHA (82.4 percent), and uninsured (88.3 percent).
 - Overall, 78.1 percent of adults ages 65 years and older reported good, very good or excellent health, with the following variation by insurance program: Medicare Part B (77.4 percent) or Part D (76.0 percent), Medicaid (50.7 percent), and VHA (69.1 percent). After adjustment for characteristics that vary across programs, distributions of self-reported health are more similar across insurance programs, with at least two-thirds (66.5 percent) reporting good, very good, or excellent health.
 - Individuals who reported their health was very good or excellent were more likely to report access to prescription drugs than individuals who reported their health was fair or poor. This finding was consistent across age groups and by insurance program.
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Overview of Health Outcomes

The health and well-being of a population is commonly measured in terms of life expectancy and death rates [1]. In 2014, life expectancy in the United States was 78.8 years, with a difference of about five years between men (76.4 years) and women (81.2 years) [2]. The age-adjusted death rate in 2014 was 724.6 deaths per 100,000 people, the lowest ever recorded [3]. Death rates were highest for Black males (1,060.3 per 100,000) and lowest for Hispanic females (437.5 per 100,000) [3]. Heart disease, cancer, and chronic lower respiratory diseases were the three leading causes of death in 2014 [3].

Life-expectancy and death rates are long term outcomes and may be more tenuous measures of health in relation to health insurance at a specific point in time [4]. As a result, intermediate health outcomes are commonly used to measure health. Self-reported health is a subjective global evaluation of one's own health status [5] that captures burden associated with acute and

chronic health conditions [6]. Self-reported health has been shown to predict mortality across numerous populations in the United States [5, 7-11] and in multiple countries [12-17] and has been widely used in sociological, medical, and epidemiological research [18]. It has been recognized by the Institute of Medicine as an important indicator of population health [19] and is included as a national health objective measure in Healthy People 2020 [20].

Self-reported health is usually measured with a single item question that asks individuals to rate their health on a five-point scale (excellent, very good, good, fair, poor) [18]. Nearly two-thirds (62.3 percent) of the U.S. population ages 18 and older reported excellent or very good health and another 25.8 percent reported good health in the 2014 National Health Interview Survey [21]. However, these estimates may conceal differences in self-reported health for individuals with different health insurance coverage [22-24].

Several recent studies examined the effects of expanded access to health insurance through the Affordable Care Act (ACA) or new eligibility qualifications on self-reported health. Data from a national telephone survey showed that by the end of the second Open Enrollment period for Marketplace coverage in 2015, the percentage of adults ages 18-64 reporting fair or poor health decreased by 3.4 percent relative to the pre-ACA trend [25]. There were no differences in self-reported health among adults in states that did and did not expand Medicaid, a finding that was echoed in a recently published similar study [26]. Several other studies have not shown improvements in self-reported health with insurance coverage through Medicare [27] or other coverage options through health care reform [28, 29]. One hypothesis that has been offered to explain these null effects is that ratings of self-reported health may be negatively impacted by increased contact with health care professionals and objective information obtained about one's actual health status resulting from increased access [26]. Additional research is needed to understand how, when, and for whom insurance coverage is associated with self-reported health.

Overview of Access to Prescription Medication and Health Outcomes

In recognition of the public health importance of access to prescription medicines, Healthy People 2020 has set a goal of reducing the percentage of people who are unable to obtain, or who experience delays in obtaining necessary prescription medicines [30]. As noted in Chapter 8, 9.7 percent of the adult population in the U.S. ages 18-64 years and 4.7 percent of those ages 65 years and older, respectively, reported not taking prescription drugs as recommended due to cost.

Much of the research about access to prescription medications focuses on health care spending and patient behavioral responses to pricing changes [31, 32] rather than health outcomes. A recent review of 23 studies of prescription drug insurance coverage and patient health outcomes concluded that with broader prescription drug insurance, other health care service use was decreased and improvements in a variety of health outcomes were observed [32]. The review included only two studies that examined self-reported health as an outcome, only one of which showed decreases in self-reported health to be associated with cost-related poor adherence among Medicare beneficiaries [35]. The second study in the review showed no improvements in self-reported health with drug prescription coverage [33], nor did recent study that examined the impact of Medicare Part D coverage on self-reported health among the community-dwelling Medicare population [34].

A likely mechanism through which prescription drug insurance coverage could potentially influence health outcomes is via improved access and adherence to medication regimens [35, 36]. Higher medication costs due to lack of prescription drug insurance or high cost-sharing have been consistently shown to reduce treatment initiation, adherence, and continuation among chronically ill patients [37, 38], the middle-aged and elderly [36], and individuals with diabetes [39]. Since 2006, Medicare Part D has provided drug insurance to Medicare beneficiaries [40]. An examination of changes in cost-related prescription nonadherence in 2006 vs 2005 estimated an 8-percentage point decrease among newly insured beneficiaries with Part D coverage [41]. A recent analysis of data from the 2000-2010 Health and Retirement Surveys showed similar changes [42]. Nevertheless, Medicare Part D beneficiaries may still experience difficulties paying for needed medications [43].

Health outcomes and prescription drug access among individuals in different insurance programs are rarely compared, in part because many studies use separate health insurance claims data to evaluate patterns of care. By definition, studies using Medicare claims data can only evaluate treatment patterns in Medicare beneficiaries, and cannot provide information about individuals with other insurance coverage or services not covered by the Medicare Program. One of the few studies to examine differences in prescription medication use by health insurance program was conducted in 766 adults ages 21 and older (95.6 percent male) with a diabetes diagnosis receiving care in one of five health care systems (three VHA, one university-based, one county-based system). Underuse of medications due to cost was lowest among patients with coverage through the VHA (9 percent), followed by patients with coverage through private insurance (18 percent), Medicare (25 percent), Medicaid (31 percent), or no coverage (40 percent) [39].

Analyses of Outcomes by Health Insurance Program

Data source

The National Health Interview Survey (NHIS) was selected as the data source to conduct analyses of health outcomes by health insurance program. The NHIS is an annual nationally-representative household interview survey of the US civilian non-institutionalized population [44]. It was created by the National Health Survey Act of 1956 with the goal of providing “statistical information on the amount, distribution, and effects of illness in the United States and the services rendered for or because of such conditions” [44]. The NHIS serves as the primary source of information on the health of the US population and is currently used to monitor progress towards the achievement of many national health objectives put forth by Healthy People [45]. Multiple years of the NHIS were pooled to ensure sufficient numbers of individuals with insurance coverage from each of the programs to produce stable estimates.

Measures of health insurance coverage

Because the NHIS is an in-person household survey, interviewers can query respondents directly about their answers to the survey questions. In addition, interviewers also examine health insurance cards to maximize the accuracy of reporting about health insurance coverage. Individuals were classified as having any Medicaid, any Medicare Part B, any Medicare Part D, or any VHA. Because some individuals had more than one source of health insurance coverage,

these categories were not mutually exclusive. Individuals without any health insurance coverage were classified as uninsured.

Measures of self-rated health and access to prescription medication

Self-reported health has been assessed in the NHIS since 1972 with the following question: “Would you say your health in general is excellent, very good, good, fair, or poor?” This question was rigorously evaluated through cognitive interviews and psychometric testing [46, 47] to be considered for inclusion as a Healthy People 2020 objective.

As in the analyses described in Chapters 8 and 9, limited access to prescription medication was measured as: 1) skipped medication doses, took less medicine, or delayed filling a prescription due to cost in past 12 months (collectively referred to as not taking medication as prescribed due to cost) and 2) inability to afford prescription medication in the 12 months. Again, because results for these two indicators of access to prescription medication were very similar, we only present results for not taking medication as prescribed due to cost.

Statistical methods

As in the analysis of patient satisfaction with care described in Chapter 9, estimates of self-reported health are reported for the total U.S. population and separately for each of the four government health insurance programs. Within each insurance program, estimates of self-reported health are also presented for individuals with and without problems with access to prescription drugs due to cost. All analyses were stratified by age group (18-64, 65+) to reflect differences in the prevalence of chronic conditions and age-related eligibility for the Medicare Program. Estimates of self-reported health were also adjusted for the effects of individual characteristics (age, gender, marital status, race/ethnicity, geographic region, number of chronic conditions, and income) related to health that vary across insurance programs with multivariable logistic regression models. For ease of interpretation, predicted margins are reported, which are interpreted as adjusted population percentages [48].

NHIS sampling weights were used in all analyses to incorporate complex survey design and provide nationally representative estimates. All analytic files were created using SAS 9.3. Multivariable logistic regressions were performed using STATA 13.1.

Self-reported health by health insurance program

Approximately 9 out of 10 (88.9 percent) adults ages 18-64 in the U.S. reported excellent or very good health (Figure 1). However, nearly two-thirds of individuals in this age group with Medicare Part B (62.5 percent) or Part D (64.0 percent) reported fair or poor health. To qualify for Medicare before the age of 65, individuals must have a documented disability. Nearly equal percentages (29-40 percent) of individuals with any health insurance through Medicaid or the VHA reported excellent/very good, good, or fair/poor health. Self-reported health among the uninsured was similar to that observed among the total population, with fewer reporting excellent/very good health (56.0 percent) and slightly more reporting good (30.9 percent) and fair/poor health (13.1 percent).

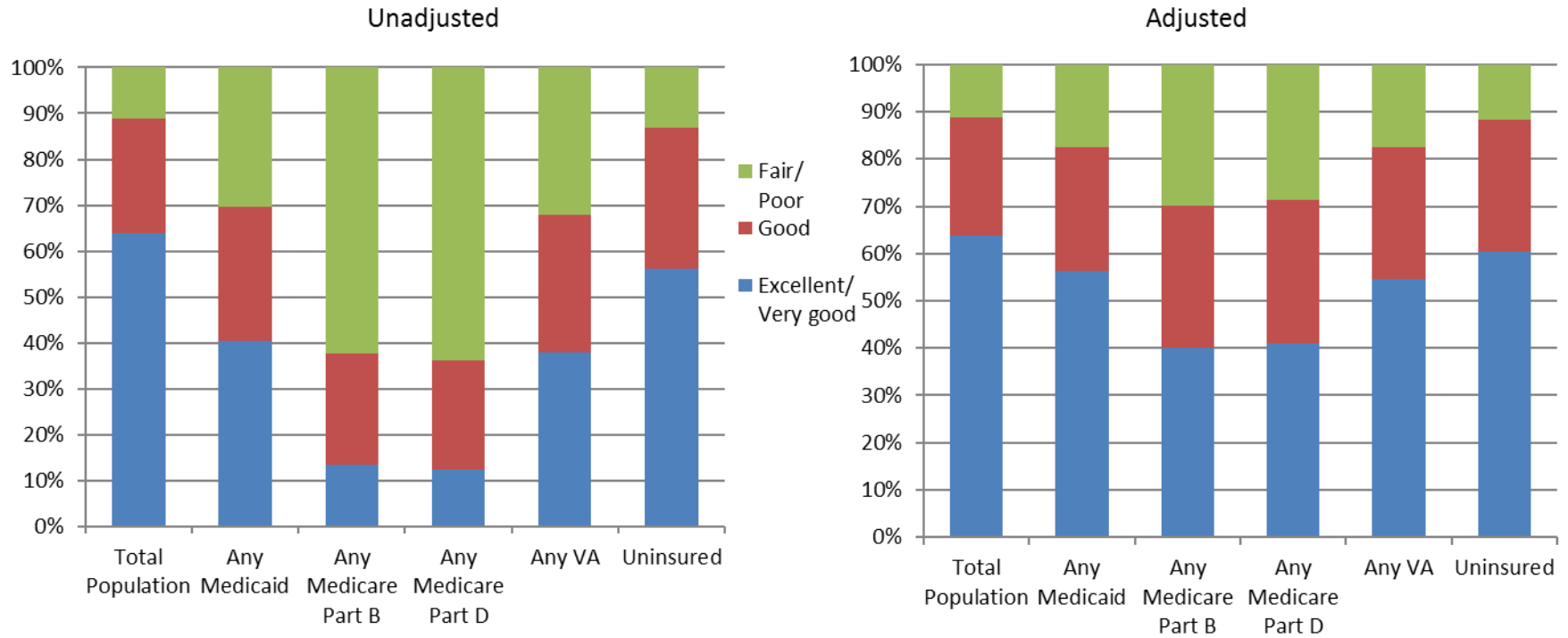
Approximately 8 out of 10 (78.1 percent) adults ages 65 and older in the US population reported good to excellent health, which is comparable to the percentage observed among younger adults

(Figure 2). However, more adults ages 18-64 (63.9 percent) reported excellent/very good health than did adults ages 65 and older (45.0 percent). A similar percentage of individuals ages 65 and older with health insurance through Medicare Part B (44.4 percent) and Part D (43.0 percent) reported excellent/very good health. Approximately half (49.3 percent) of adults older than 65 with Medicaid coverage reported fair/poor health. Self-reported health was more evenly split among individuals accessing care through the VHA.

After adjusting for sociodemographic factors that vary across government insurance programs, distributions of self-reported health among adults ages 18-64 looked quite similar across insurance programs (Figure 1). Large majorities of adults ages 18-64 with coverage through Medicare Part B (70.0 percent) or Part D (71.3 percent), Medicaid (82.6 percent), and the VHA (82.4 percent) as well as the uninsured (88.3 percent) reported good, very good, or excellent health. After adjustment, the percentage of adults ages 18-64 reporting excellent/very good health increased, and the percentage reporting fair/poor health decreased, for all groups.

Adjusting for sociodemographic characteristics did not greatly change estimates of self-reported health for adults ages 65 and older (Figure 2). After adjustment, at least two-thirds of adults ages 65 and older across insurance programs reported good, very good or excellent health (Medicaid: 66.5 percent; Medicare Part B: 77.8 percent; Medicare Part D: 77.5 percent; any VHA: 72.0 percent).

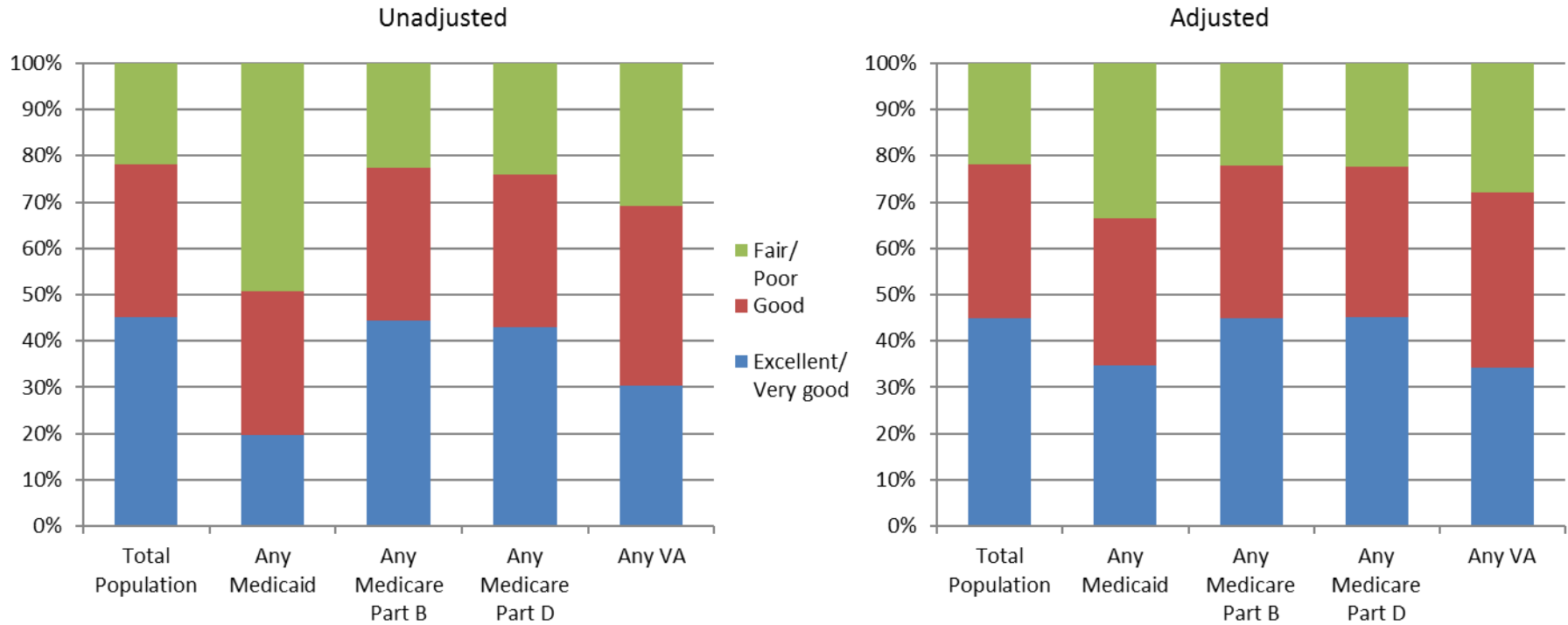
Figure 1. Self-Reported Health by Program, Ages 18-64 Years



Source: NHIS 2011 -2014

Note: Insurance categories are not mutually exclusive.

Figure 2. Self-Reported Health by Program, Ages 65 Years and Older



Source: NHIS 2011 -2014

Note: Insurance categories are not mutually exclusive.

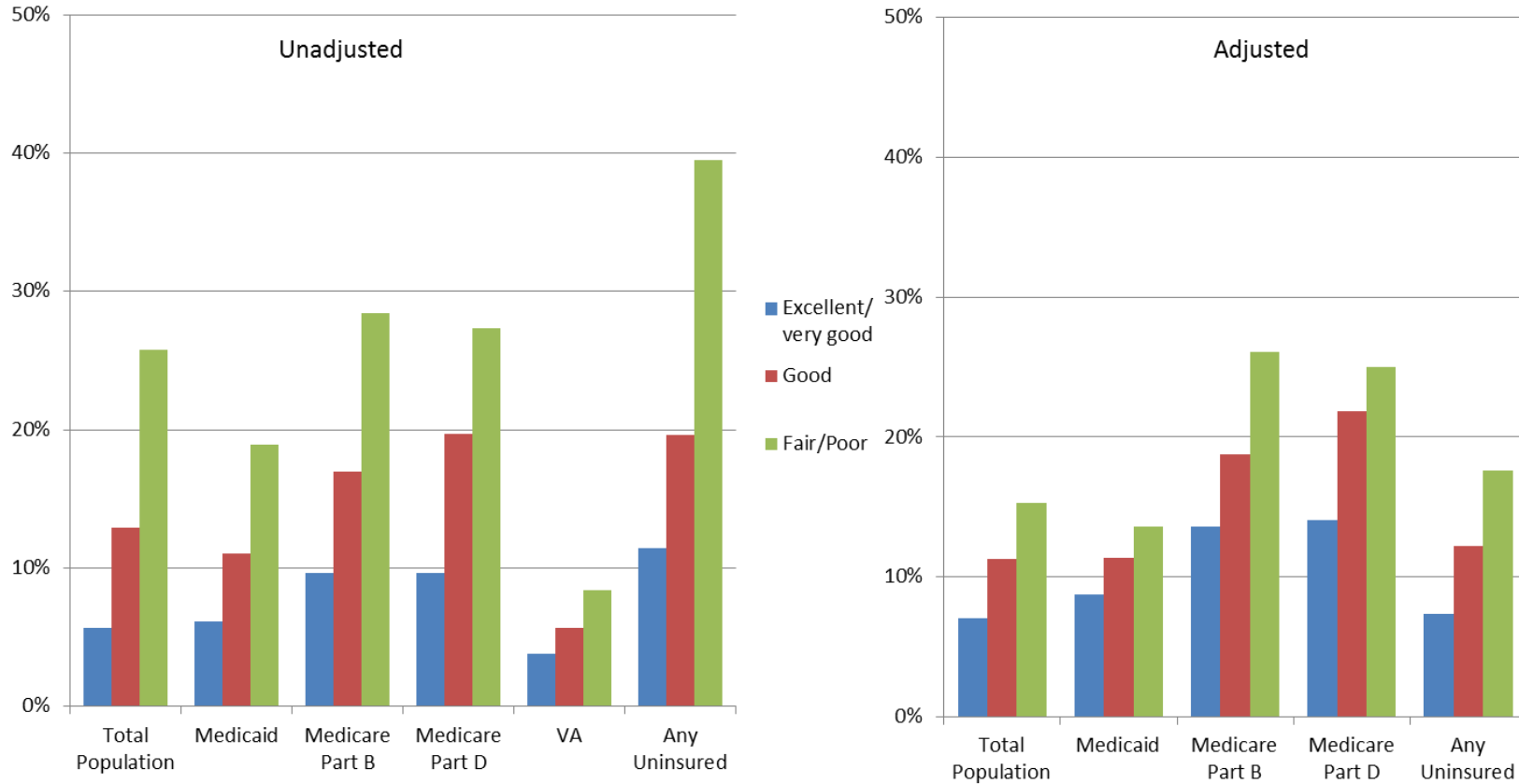
Prescription medication access and self-reported health by health insurance program

Higher percentages of adults ages 18-64 (Figure 3) and 65 and older (Figure 4) with fair/poor health reported not taking medication as prescribed due to cost as compared to those with good health, and higher percentages with good health reported not taking medication as prescribed due to cost as compared to those with excellent/very good health. This stepwise pattern was observed consistently across insurance programs, although the association was strongest among the uninsured. Among adults ages 18-64 with health insurance, not taking medication as prescribed due to cost was highest across all levels of self-reported health for Medicare Part B and Part D beneficiaries and lowest among individuals covered through the VHA. Among adults ages 65 and older, not taking medication as prescribed due to cost did not differ much across insurance programs.

Adjusting for patient characteristics did not substantially change the direction of the unadjusted patterns described above for adults ages 18 to 64 or 65 and older (Figures 3 and 4). However, among individuals ages 18-64 with fair/poor health, the percentage reporting that they were not taking medication as prescribed decreased. Among individuals ages 65 and older with fair/poor health, the percentage reporting not taking medication as prescribed due to cost also decreased. Due to limited sample size, estimates of adjusted percentages for individuals receiving insurance coverage through the VHA are not reported.

Unadjusted and adjusted results for the percentages of adults ages 18-64 and 65 and older reporting inability to afford prescription medication in the past 12 months were similar to the patterns presented above.

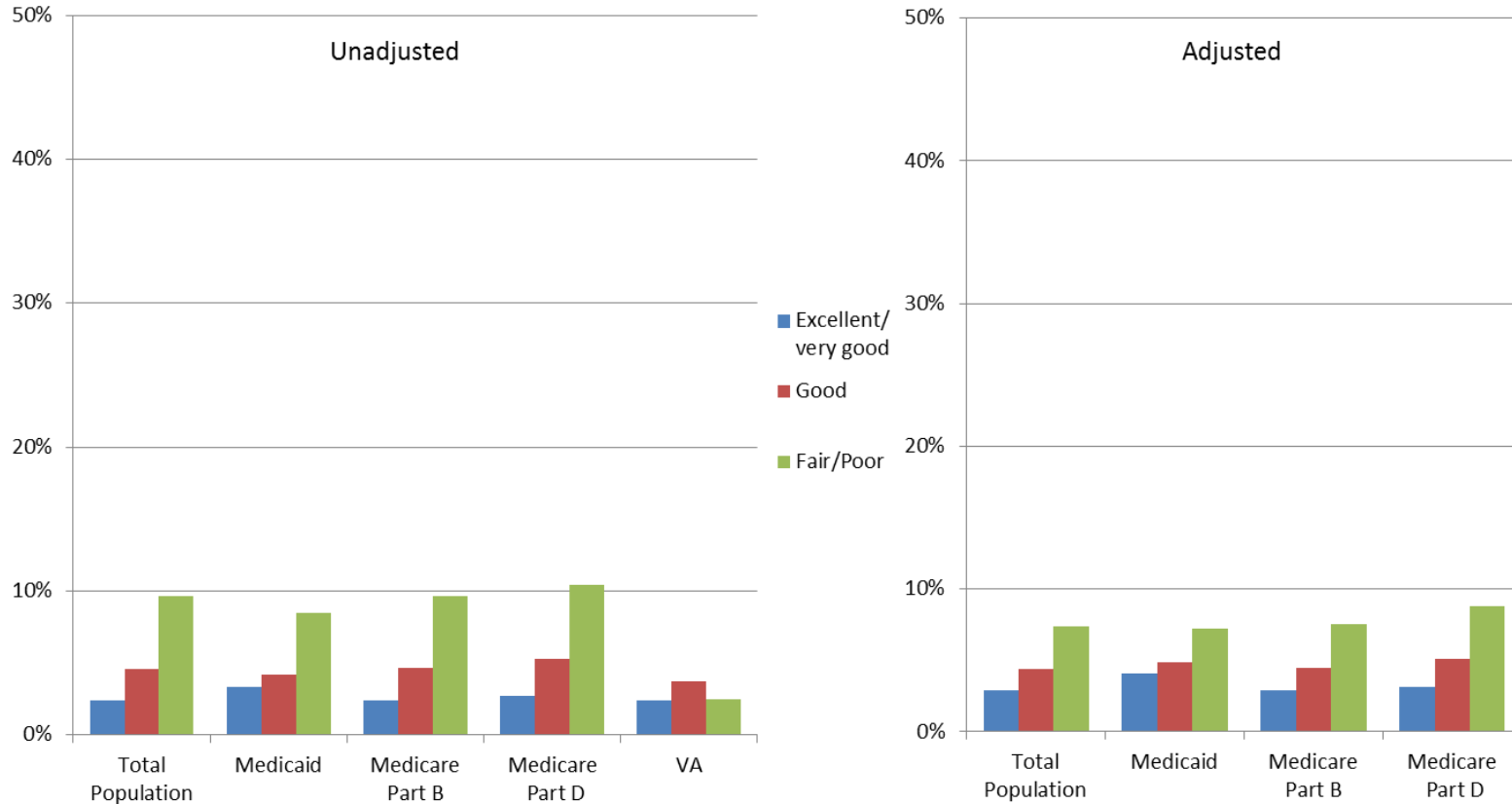
Figure 3. Self-Reported Health and Percentage Not Taking Medication as Prescribed Due to Cost by Program, Ages 18-64 Years



Source: NHIS 2011-2014

Note: Insurance categories are not mutually exclusive.

Figure 4. Self-Reported Health and Percentage Not Taking Medication as Prescribed Due to Cost by Program, Ages 65 and Older



Source: NHIS 2011-2014

Note: Insurance categories are not mutually exclusive.

Summary

Self-reported health is a subjective global evaluation of one's own health status that is often predictive of mortality. Research suggests that prescription drug coverage may be indirectly linked to self-reported health. Higher medication costs due to lack of prescription drug insurance or high cost-sharing have been consistently shown to reduce treatment initiation, adherence, and continuation among various patient groups. In turn, cost-related poor adherence and medication restriction have been found to be negatively associated with self-reported health.

In the United States, more than three-quarters of adults ages 18 to 64 (88.9 percent) and ages 65 and older (78.1 percent) report good, very good, or excellent health. The percentages of adults ages 18-64 years with insurance through Medicare Part B, Medicare Part D, Medicaid, VHA or without insurance coverage reporting good, very good, or excellent health are lower, but after adjustment for sociodemographic characteristics distributions of self-reported health are more similar across insurance programs, with at least 70 percent in all groups reporting good, very good, or excellent health. Similarly, the percentages of adults ages 65 years and older with Medicaid or VHA coverage reporting good, very good, or excellent health are lower than the general population, but after adjustment for sociodemographic characteristics distributions of self-reported health are more similar across insurance programs, with at least 66 percent in all groups reporting good, very good, or excellent health.

Self-reported health is associated with limitations in prescription drug access. Higher percentages of adults ages 18-64 years and ages 65 years and older with fair or poor health reported not taking medication as prescribed due to cost year as compared to those adults in the same age group with good or very good/excellent health. This stepwise pattern was observed consistently across insurance programs and when controlling for sociodemographic characteristics.

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CHAPTER 11: SUMMARY

Prescription drugs can effectively treat many acute and chronic diseases leading to improvements in quality of life, life expectancy, and overall population health. During most of the time period analyzed in this report, growth in prescription drug spending in the United States was moderated by a number of patent expirations and the resulting increased availability and use of generic versions of top selling brand-name drugs. Nonetheless, growth in prescription drug spending has been rising more quickly than overall health care spending [1, 2]. In recent years, growth in prescription drug spending has accelerated considerably due to increases in the number of newly available costly drugs, including specialty drugs and biologics; price increases in existing drugs; a relatively low number of patent expirations; increasing insurance coverage; increasing utilization; and population growth and aging [1, 2]. Prescription drug expenditures are projected to continue rising during the coming decade [2], adding to the nation's total health care bill and placing increasing fiscal pressures on commercial, federal, state, and family budgets.

This report was divided into three sections: innovation and prescription drug development, prescription drug spending, and patient access to prescription drugs. The sections on prescription drug spending and patient access contain data and quantitative analysis for government health insurance programs, including Medicare Part B, Medicare Part D, Medicaid and the Veterans Health Administration (VHA).

The section of the report on innovation provided an overview of new prescription drug approvals and the clinical trials process and an evaluation of the current cost and length of time necessary to bring new drugs to market. Prescription drug innovation is ongoing and between 2006 and 2015, the Food and Drug Administration approved an average of 29 novel drugs a year, with 45 approvals in 2015 alone [3]. Published estimates of the cost of new drug development range from \$1.2 billion to \$2.6 billion [4-7] and are highly sensitive to assumptions about pre-clinical and clinical development time, cost of capital, the likelihood of reaching approval following the start of clinical testing, and costs of preclinical development and clinical trials conducted among humans. Published estimates of the cost of new drug development are also highly sensitive to the incorporation of recent increases in Orphan drug approvals, which tend to have smaller trial sizes, higher success rates, and tax advantages for the sponsor.

The section of the report on prescription drug spending addressed overall spending and recent trends in spending using data from literature reviews and separate quantitative analyses for the government insurance programs. Although patterns of increases in overall prescription drug spending growth varied by program, spending on specialty drugs and biologics increased rapidly in all programs. Small numbers of drugs represent disproportionately high spending, especially in Medicare Part B, where spending on the top 10 drugs represented 47 percent of spending in 2014. Generic drugs accounted for the majority of dispensed prescriptions, but a relatively small percentage of spending. Importantly, spending by therapeutic class reflects underlying differences in eligibility and prescription drug coverage across programs.

A number of purchasing arrangements and utilization management strategies are used by commercial insurers to promote value and control cost, including negotiation with manufacturers and pharmacies, rebates, use of preferred drug lists or formularies with tiers, prior authorization requirements, step therapy, prescription quantity limits, value-based purchasing and payment, and risk-sharing or outcomes-based arrangements [8]. Arrangements to promote value and control cost are used to varying degrees by the different government programs.

In the patient access to prescription medications section of the report, relevant published literature was reviewed and data and analyses pertaining to patient access to drugs, satisfaction with care, and outcomes were presented for each of the four government health insurance programs. Access to prescription drugs varied substantially by age in the United States [9] and adults ages 18-64 years were twice as likely as older adults ages 65 years and older to report skipping doses, taking less medication, or delaying filling prescription drug medications because of cost in the past 12 months (9.7 percent vs 4.7 percent), despite lower prevalence of chronic conditions and medical need. Among younger adults ages 18-64 years, the prevalence of not taking drugs as prescribed because of cost varied by insurance program, although statistical adjustment for characteristics that vary across programs, such as comorbidity, had a significant effect on estimates of prescription drug access. Among adults aged 65 years and older, prevalence of not taking drugs as prescribed because of cost was relatively similar across insurance programs. Individuals with access to prescription drugs were also more likely to report higher overall satisfaction with health care and better self-reported health outcomes.

Between 2011 and 2014, access to prescription drugs improved as the percentage of adults not taking drugs as prescribed because of cost declined. Improvements in access to prescription drugs during this period likely reflect increased availability of health insurance coverage for the population ages 18-64 years and efforts to close the Medicare Part D coverage gap in the population ages 65 years and older. In addition, research showed that individuals who gained insurance coverage between 2013 and 2014 filled more prescriptions and had lower out-of-pocket spending per prescription [10]. Between 2003 and 2014, median monthly out-of-pocket spending for privately insured users of non-specialty drugs has declined, even though patient out-of-pocket spending for specialty drugs has increased [11]. Because a relatively small proportion of individuals use specialty drugs, these findings are consistent with overall improvements in patient access to prescription drugs, despite increasing prescription drug spending.

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APPENDIX: CONGRESSIONAL REQUEST

Prescription Drug Report - The agreement directs the Secretary of HHS in consultation with the Secretary of the Department of Veterans Affairs, to submit a report to the Committee on Appropriations of the House of Representatives and the Senate, using data only available under current law that is not proprietary, not later than 180 days after the date of the enactment of this Act to which this explanatory statement pertains regarding the following topics, as described further below: price changes of prescription drugs (net of rebates) since 2003; access to prescription drugs by patients in the four programs listed below; health outcomes and patient satisfaction with care in the four programs listed below; and an analysis of the current cost and length of time necessary to bring new drugs to market.

The report should include prescription drug prices (net of rebates) paid by Federal programs for the 10 most frequently prescribed drugs and the 10 highest cost drugs under the following programs:

1. The Medicare program under part B of title XVIII of the Social Security Act.
2. The Medicare prescription drug program under part D of title XVIII of the Social Security Act.
3. The Medicaid program under title XIX of the Social Security Act.
4. The Department of Veterans Affairs.

In addition, the report should include total annual prescription drug costs (net of rebates) to the Medicare program under part B of title XVIII of the Social Security Act, the Medicare prescription drug program under part D of title XVIII of such Act, the Medicaid program under title XIX of such Act, and the Department of Veterans Affairs as a percentage of total health care program expenditures. The report shall make note that the total annual prescription drugs costs do not adjust for biomedical inflation. The Secretary of HHS shall review how the Federal Government has achieved cost reductions for drugs since 2001.

The report should also include an evaluation of access to prescription drugs by the four programs listed above, measured consistently across each program using one or more metrics that are generally accepted by healthcare professionals and health policy experts as reliable and appropriate measures of patient access to prescription drugs. The evaluation of patient access shall take into account the extent to which each program uses: formularies (including the breadth and adequacy of such formularies); utilization management techniques; and the average interval between the time a patient attempts to fill a prescription and receipt of the prescription drug, as applicable.

The report should also include an evaluation of patient satisfaction with care (based on a survey with statistically significant results) and of patient outcomes in the four programs listed above, measured consistently across these programs using one or more metrics that are generally

accepted by healthcare professionals and health policy experts as reliable and appropriate measures of patient health outcomes and patient satisfaction with care, respectively.

Finally, the report should include an analysis of the current cost and length of time necessary to bring new drugs to market including the impact of biomedical inflation.