

PHARMACEUTICALS: INNOVATION, ACCESS, AND AFFORDABILITY CHARTBOOK

A Report from the
Johns Hopkins
Drug Access and
Affordability Initiative



JOHNS HOPKINS

BLOOMBERG SCHOOL
of PUBLIC HEALTH

The Johns Hopkins Drug Access and Affordability Initiative examines the level of innovation, affordability and access to pharmaceuticals in the United States. This chartbook provides data for policymakers, media, researchers, and the public.

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TABLE OF CONTENTS

1	Introduction
3	Section 1: Drug Affordability
3	1.1 How Are Drugs Priced in the United States?
6	1.2 How Much Does the United States Spend on Pharmaceuticals?
8	1.3 Who Pays for Pharmaceuticals in the United States?
15	1.4 How Much Do Patients Pay Out-of-pocket for Drugs?
18	Section 2: Access to Pharmaceuticals
18	2.1 What Types of Drugs Do Americans Purchase?
22	2.2 How Do Insurers Control Access to Drugs?
26	2.3 What Are Pharmaceutical Benefit Managers?
27	Section 3: Innovation
27	3.1 How Has the Drug Industry Changed Recently?
30	3.2 What Is the Public's Perception of Drug Pricing?
32	3.3 What Is the Cost to Develop a Drug?
33	3.4 What Are Orphan Drugs?
35	3.5 How Does The United States Compare To Other Countries?
37	Conclusion
38	References
39	Methodological Appendix

FIGURES

- FIGURE 1:** Relationship Between Price and Health
- FIGURE 2:** U.S. Pharmaceutical Market Overview
- FIGURE 3:** U.S. Pharmaceutical Net Revenue by Segment (2016) In Billions of Dollars
- FIGURE 4:** U.S. Retail Prescription Drug Expenditures
- FIGURE 5:** Growth in Drug Spending
- FIGURE 6:** Retail Prescription Drug Spending by Payer in 2016
- FIGURE 7:** Commercially Insured Drug Spending (2010-2016)
- FIGURE 8:** Commercial Insured Drug Spending by Top 10 Percentile (2016)
- FIGURE 9:** Medicare Spending by in Parts A, B, C And D
- FIGURE 10:** Medicare Part D Spending (2006-2016)
- FIGURE 11:** Distribution of Beneficiary Part B Spending (2016)
- FIGURE 12:** Average per Beneficiary Part B Spending (2016)
- FIGURE 13:** Medicaid Pharmaceutical Spending 2010-2014
- FIGURE 14:** Cost-Sharing in Commercial Market (2016)
- FIGURE 15:** Average Cost Share per Prescription Fill (2010-2016)
- FIGURE 16:** Cost-Sharing in Medicare Part D (2016)
- FIGURE 17:** Patient Assistance Program Expenditures
- FIGURE 18:** Top Ten Drugs in U.s. by Spending (2016 \$Billions)
- FIGURE 19:** Top Ten Drugs by Medicare Part D Spending (2016)
- FIGURE 20:** Top Ten Drugs in Medicare Part B by Spending (2016)
- FIGURE 21:** Medicare Part D Spending by Therapeutic Group
- FIGURE 22:** Medicare Part D Fills by Therapeutic Group
- FIGURE 23:** Medicare Part D Spending and Fills by Drug Type (2016)
- FIGURE 24:** Distribution of Plans in Large Employer Market
- FIGURE 25:** Medicare Formularies by State
- FIGURE 26:** Coverage of Drugs by Medicare Formularies
- FIGURE 27:** Hepatitis C Drugs In Medicaid
- FIGURE 28:** Hepatitis C Drug Coverage in Medicaid
- FIGURE 29:** Profit Growth of Pharmaceutical Benefit Managers
- FIGURE 30:** Trends in Global R&D
- FIGURE 31:** Perception on Drug Prices
- FIGURE 32:** Sources of High Drug Prices (2017)
- FIGURE 33:** Average Cost of Development per Drug
- FIGURE 34:** Generic Industry Consolidation
- FIGURE 35:** Generic Market Competition
- FIGURE 36:** Trend in the Number of Orphan Drug Approvals
- FIGURE 37:** Orphan Drug Approval Characteristics
- FIGURE 38:** U.S. Pharmaceutical Spending vs. Other Industrialized Nations in 2016
- FIGURE 39:** U.S., Canada, Japan, and U.K. Drug Price Index

INTRODUCTION

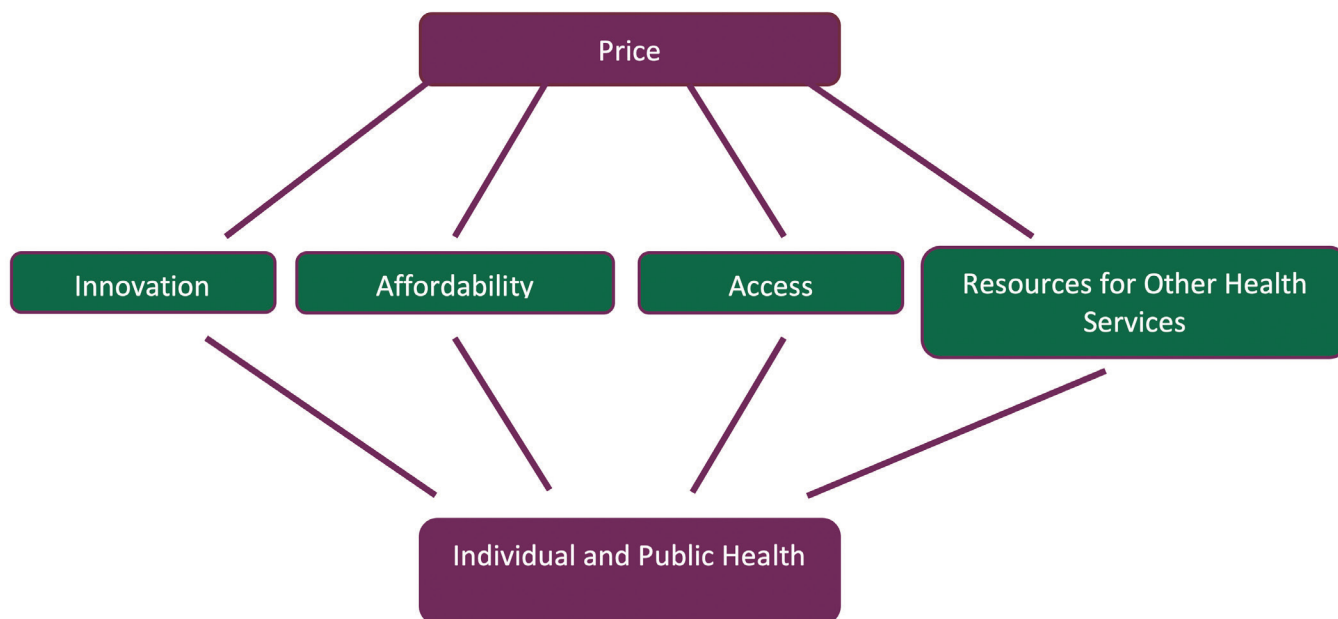
Pharmaceuticals provide important improvements to the health of many Americans. For example, contemporary antiviral treatments for hepatitis C, the leading cause of infectious disease deaths in the United States, have been shown to cure up to 99% of patients. However, high prices can limit the access and affordability of these same innovations. The tradeoff between innovation and affordability is the focus of the chartbook.

High drug prices are at the crux of patient access and affordability. Understanding the trends and reasons for high drug prices in the United States is complicated, as the pricing system in the US is both complex and lacks transparency. This makes it difficult for patients, physicians, policy

makers, the media, insurers, and other stakeholders to understand how the prices are determined, the effect on of prices on affordability, and the impact on pharmaceutical innovation and health outcomes. A simplified examination of the relationship between prices and health outcomes is outlined in **Figure 1**.

The objective of this chartbook is to provide a foundation for understanding drug affordability and access in the United States, and ultimately help to help policymakers develop and evaluate policies that address the prices of pharmaceuticals while balancing incentives for innovation with expanded access and affordability for patients.

FIGURE 1: RELATIONSHIP BETWEEN PRICE AND HEALTH



CHARTBOOK OVERVIEW

The chartbook contains 39 charts and is divided into three main sections:

- **Section 1—Affordability:** Are drugs affordable in the United States? How much are Americans paying for the drugs, and how much through insurers and government programs?
- **Section 2—Access:** Are patients having problems accessing drugs? What drugs are most and least accessible? Are people receiving the drugs they need?
- **Section 3—Innovation:** What factors influence innovation and development of new drugs? What impacts innovation in the pharmaceutical market? What are the costs to develop a drug in the United States? How has the pharmaceutical industry evolved in the past 10 years?

The conclusion summarizes key findings and important takeaways.

The methodological appendix summarizes the data sources and approaches that were used to create this chartbook.

This chartbook cannot cover all the topics related to affordability, access, and innovation. For example, the clinical and public health impact of pharmaceuticals are not the subject of this chartbook. Instead, we focus on the empirical evidence relating to affordability, access, and innovation, which are the essential components to any policies aiming to improve health outcomes from pharmaceuticals in the United States. The chartbook does not address the tradeoffs between resources spent on pharmaceuticals and other health services.

SECTION 1 - AFFORDABILITY

1.1

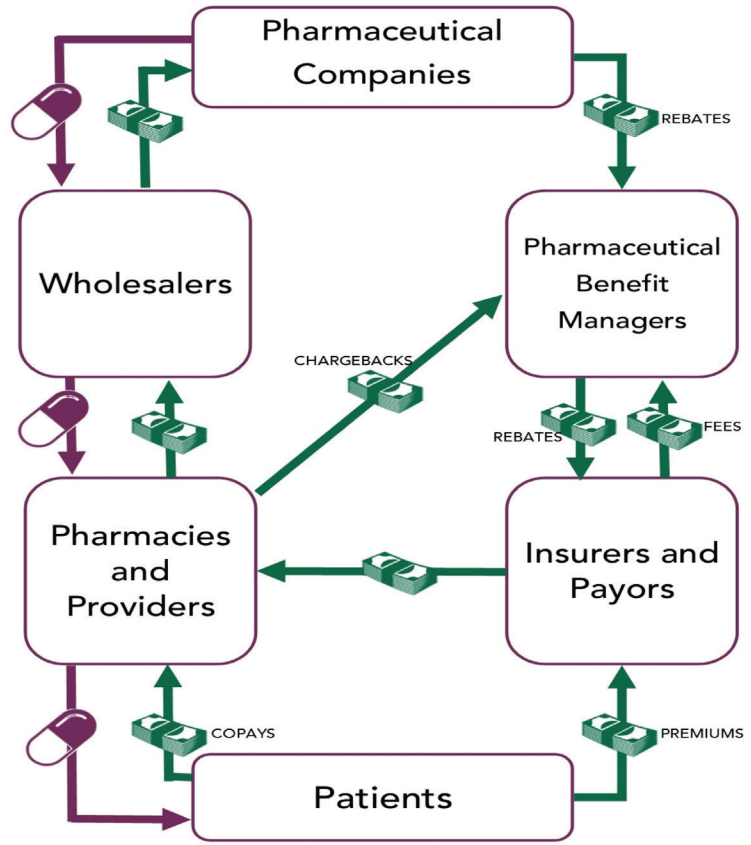
How Are Drugs Priced In The United States?

To understand drug prices in the United States, it is important to start with an overview of the pharmaceutical supply chain (see **Figure 2** for simplified overview of the US Pharmaceutical Market) and the multiple entities that influence drug prices.

The supply chain starts with pharmaceutical companies, who determine the list prices for their drugs. Branded pharmaceutical companies have complete autonomy to set their list prices.

The federal government approves patents and establishes market exclusivity periods that create monopolies for branded drug for fixed periods of time. These monopolies provide the incentive for pharmaceutical companies to engage in research and development.

FIGURE 2: US PHARMACEUTICAL MARKET OVERVIEW



Once a drug's patent and market exclusivity period ends, generic drugs and biosimilars can enter the market. For generic drugs, list prices are lower than the equivalent branded drug. However, the actual sales price for the generic drug is dependent on active competition from multiple generic manufacturers who compete on the basis of price. In some cases, price competition does not materialize. This is especially true for biosimilars and for drugs that have small patient populations. Generic drug companies without competitors can also raise prices at any time – this is how, for example, Martin Shkreli was able to raise the list price of Daraprim (an off-patent drug with no competitors) by 5000% in 2015.¹

It is important to distinguish between list and net prices. List prices are the prices drug manufacturers set for a drug, but rarely do public and private insurers actually pay list prices. Net prices, also known as the sales price, are the prices negotiated between drug manufacturers and insurers or pharmacy benefit managers (PBMs). Branded drug companies may offer rebates or discounts to PBMs and insurers in order to get favorable placement on formularies or less restrictive utilization controls. They also may give patient's coupons or fund patient assistance programs to help people afford the drugs. As a result, the net or sales price is often less than the list price.

Generic drug companies must compete on the basis of price in order to sell their drug because all generic copies of the branded drug are required to have identical active ingredients. Generic drug companies with competitors do not offer coupons or patient assistance because they are competing on the basis of price because all versions of the

generic drug must be identical. Similar to branded drugs, the actual sale price is often significantly lower than the list price. The greater the level of competition, the lower the sales price in most cases because generic companies compete with price.

Many insurers—including both government programs like Medicare and Medicaid and private health insurance companies—provide prescription drug coverage to patients. To do this, private insurers and Medicare prescription drug plans often contract with pharmacy benefit managers (PBMs) to negotiate prices and formulary placement with drug companies and negotiate prices with pharmacies to be included in their networks. PBMs represent purchasers of large quantities of drugs and can therefore negotiate aggressively with drug companies. A concern is that PBMs often receive profits based on the difference between list prices and the net or sales prices that they negotiate. This is commonly known as rebates or spread. This system creates incentives for PBMs to encourage drug companies to raise list prices so that PBMs can demonstrate greater value to their clients. However, it can mean greater cost-sharing for patients since cost-sharing is often based on list prices.

Once net prices have been negotiated, wholesalers distribute the medications from drug companies to pharmacies, hospitals, or other facilities. For most medications, wholesaler fees account for about 2% of the cost of the drug. Manufacturers may also use specialty wholesalers to set up limited distribution networks that restrict some medications to a small number of specialty pharmacies or other entities. These specialty networks may enhance

¹ Pollack, A. (2015). Drug goes from \$13.50 a tablet to \$750, overnight. *New York Times*, 20, 2015.

patient safety, but they can also serve as a tool to limit market competition.² Specialty networks can also charge more than 2% of the cost of the drug for distribution.

Hospitals, nursing homes, physicians, or pharmacies administer and dispense drugs to patients. Pharmacies, nursing homes, and hospitals receive a dispensing fee for this service. In addition to dispensing fees, pharmacies can also earn profits based on the difference between the prices at which they purchase drugs from wholesalers and the costs that they are reimbursed by insurance companies; the difference between prices is known as the “spread.” All these prices are confidential. Some drugs require patient supervision and complex administration by physicians when

being administered. These physician-administered drugs are reimbursed as a medical benefit by their insurance as opposed to a prescription drug benefit. Many physicians are reimbursed a percentage of a drug's cost for these physician-administered drugs, which creates a financial incentive for physicians to prescribe more costly drugs.

At the end of the supply chain are the patients. Patients pay more for drugs when list prices—which often are the basis for their cost-sharing payments—rise. Patients can sometimes receive drug coupons or other forms of patient assistance that can limit their cost-sharing. These arrangements can distort the market by altering the amount the patient is expected to pay.

² Karas, L., Shermock, K. M., Proctor, C., Socal, M., & Anderson, G. F. (2018). Limited distribution networks stifle competition in the generic and biosimilar drug industries. *The American journal of managed care*, 24(4), e122-e127.

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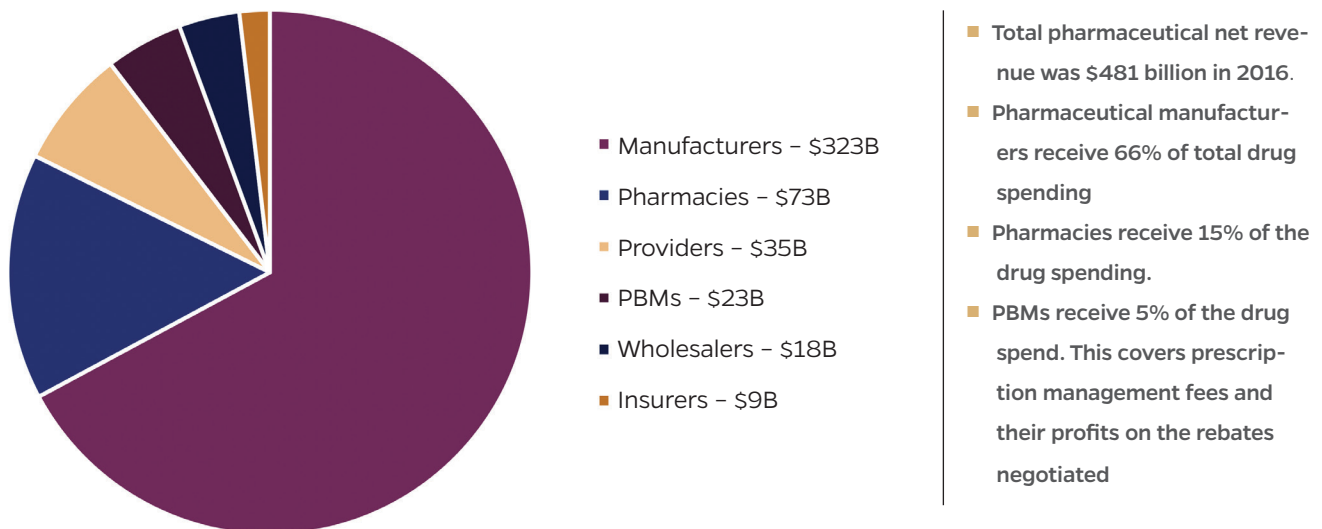
How Much Does the US Spend on Pharmaceuticals?

Spending on pharmaceuticals is a significant component of health care spending, with the percentage varying depending on how pharmaceutical spending is calculated. U.S. pharmaceutical spending as a share of overall U.S. health care spending ranged from 9.8% to 16.7% in 2016 depending on what items are included in the calculation.³

The main difference is whether pharmacy dispensing costs, physician-administered drugs, and hospital-dispensed drugs are included in the total of drug spending or in the physician and hospital components of spending. In dollar terms, the differing estimates range between \$329 billion to \$481 billion in 2016.

Each component of the supply chain shown in **Figure 2** receives a portion of the dollar spent on pharmaceuticals. The largest portion goes to the pharmaceutical companies. The distribution of revenues is shown in **Figure 3**.

FIGURE 3: U.S. PHARMACEUTICAL NET REVENUE IN BILLIONS OF DOLLARS (2016)⁴



³ A Look at Drug Spending in the U.S. (2018, February 27). *The Pew Charitable Trusts*. Retrieved September 15, 2018, from <http://www.pewtrusts.org/en/research-and-analysis/fact-sheets/2018/02/a-look-at-drug-spending-in-the-us>

⁴ Nancy, L. Y., Atteberry, P., & Bach, P. B. (2018). Spending On Prescription Drugs In The US: Where Does All The Money Go?. *Health Affairs*. <https://www.healthaffairs.org/doi/10.1377/hblog20180726.670593/full/> (Accessed September 2018).

RETAIL DRUG SPENDING

Retail drug spending is the largest component of drug spending and represents the amount spent by insurers and patients when patients fill their prescription at a pharmacy. The growth of retail drug spending increased more

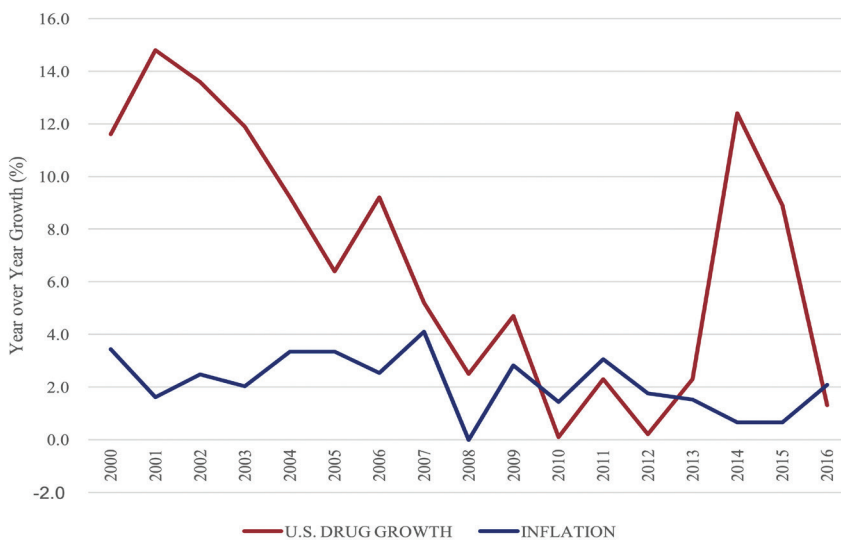
than five times faster than inflation between 2000 and 2016 (see **Figure 4**). Figure 5 compares the growth rate of retail prescription drug spending compared to overall U.S. inflation (CPI).

FIGURE 4: U.S. RETAIL PRESCRIPTION DRUG EXPENDITURES (2000-2016)⁵



U.S. retail prescription drug spending increased at an average annual rate of 5.9% from 2000 to 2016

FIGURE 5: GROWTH IN RETAIL DRUG SPENDING (2000-2016)⁶



- U.S. retail prescription drug spending increased 340% faster than overall inflation from 2000 to 2016.
- Drug spending increased 172% from 2000 to 2016; while the CPI increased by 39%
- Growth in retail drug spending is driven primarily by price growth not increased utilization.
- Growth in drug spending is variable depending on year and launch of new drugs

⁵ Centers for Medicare and Medicaid Services, Office of the Actuary, National Health Statistics Group; and US Department of Commerce, Bureau of Economic Analysis, and the US Census Bureau

⁶ibid

1.3

Who Pays for Pharmaceuticals in the US?

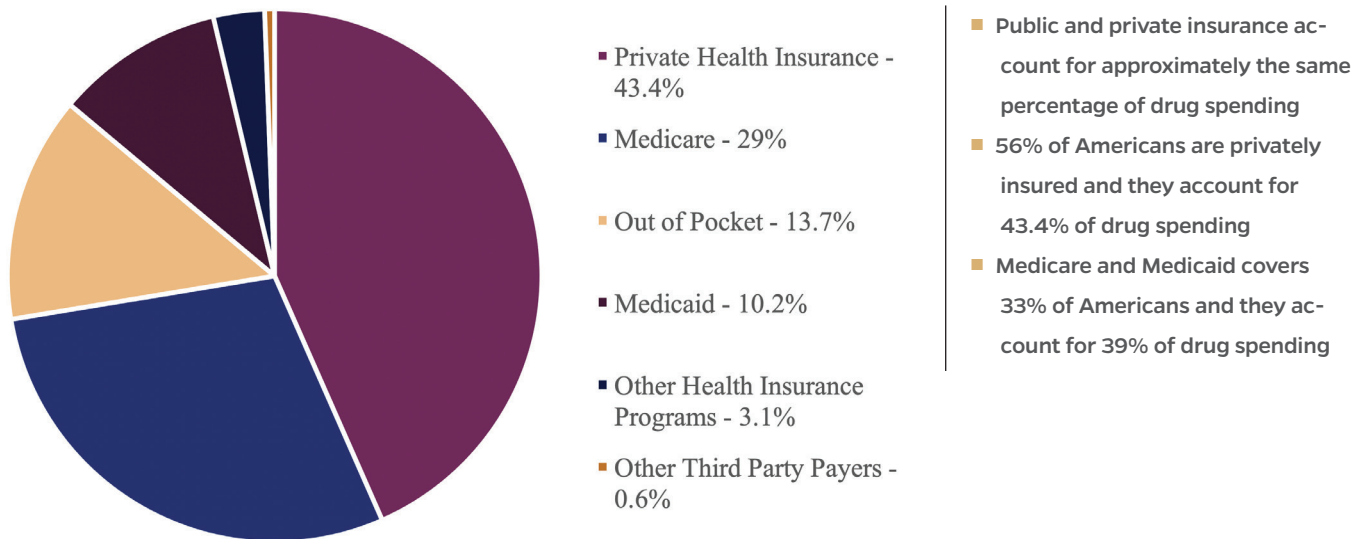
Like most other medical services, the US health care system relies on a variety of public and private health insurers to pay for the drugs. The largest collection of payers for pharmaceuticals is the private insurance market (43%). Most Americans receive health insurance through their employer, and most of these employer-sponsored insurance plans have a prescription drug benefit. People also

pay for pharmaceuticals either directly (out-of-pocket expenditures including co-pays and coinsurance) or indirectly via insurance premiums.

The two biggest public programs are Medicare and Medicaid, which when combined comprises 39% of the total pharmaceutical market. Other governmental programs that pay for drugs include Department of Veterans Affairs, Department of Defense, Public Health Service, Bureau of Prisons and others.

Figure 6 depicts the current mix of payers for pharmaceuticals in the U.S.

FIGURE 6: RETAIL PRESCRIPTION DRUG SPENDING BY PAYER IN 2016⁷



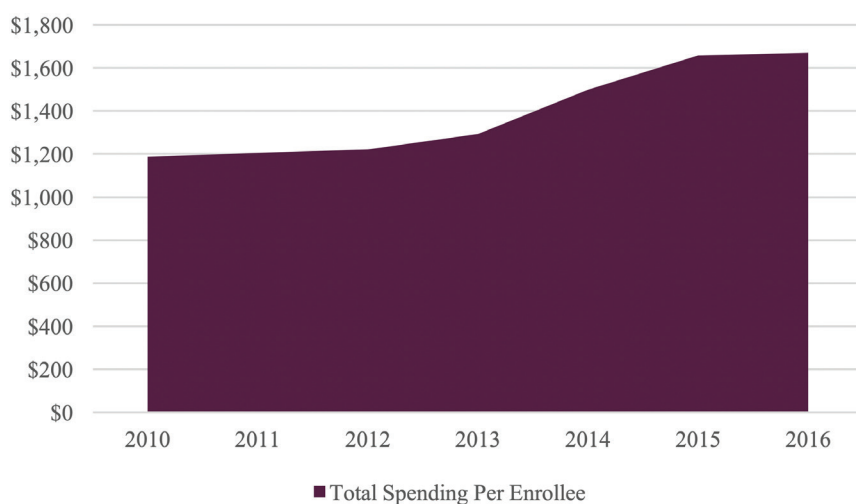
⁷Centers for Medicare and Medicaid Services, Office of the Actuary, National Health Statistics Group; and US Department of Commerce, Bureau of Economic Analysis, and the US Census Bureau.

PRIVATE INSURANCE MARKET

In the private insurance market, there has been rapid growth in pharmaceutical spending. From 2010 to 2016, total outpatient per capita drug spending increased from \$1188 to \$1670, or a 4.9% annual increase (Figure 7). This increase does not take into account rebates, but it is un-

certain how much of the rebates are retained by PBMs and insurers versus passed through to employers and ultimately patients. The top 10% of privately insured individuals accounted for 25% of prescriptions filled and over 75% of spending. This is primarily because they are taking very expensive drugs, although some are taking many drugs.

FIGURE 7: COMMERCIALY INSURED DRUG SPENDING (2010-2016)⁸

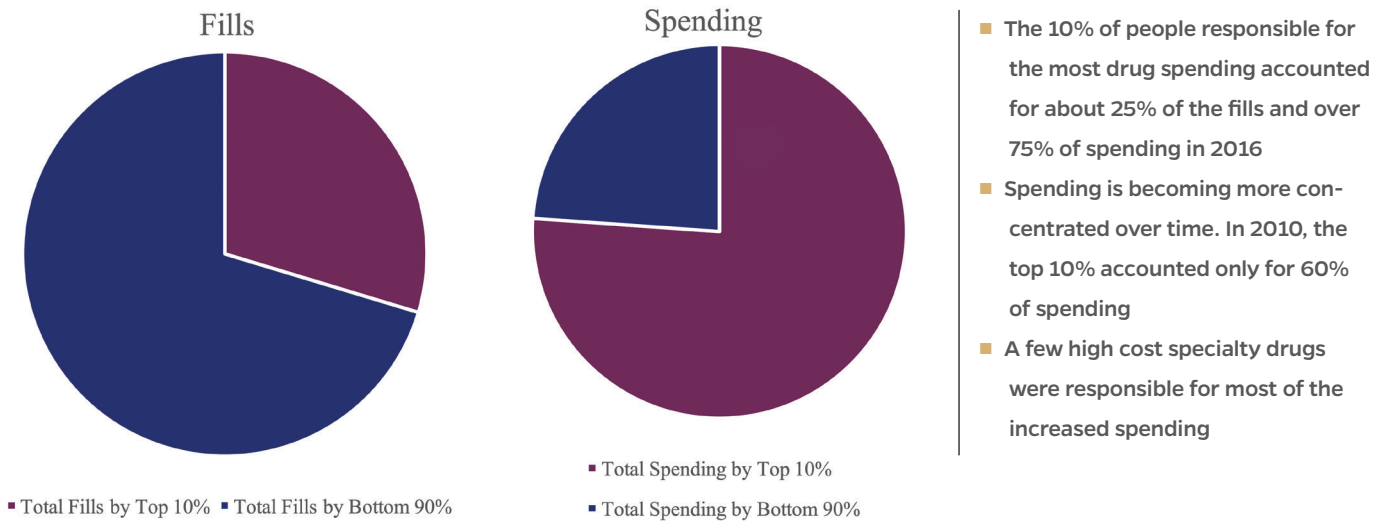


Privately insured drug spending by large employers has increased 4.9% per year from 2000-2016

Much of the recent spending growth is related to new and expensive pharmaceuticals such as Hepatitis C medications.

⁸ Analysis was conducted using Truven MarketScan[®] Commercial Database (Truven Health Analytics, Ann Arbor, MI, USA). <https://marketscan.truvenhealth.com> Refer to the Appendix for a description of the methodology.

FIGURE 8: COMMERCIAL INSURED DRUG SPENDING BY THE MOST EXPENSIVE 10 PERCENTILE OF US POPULATION IN 2016⁹



MEDICARE

The Medicare program purchases drugs in all four programs. (Figure 9)

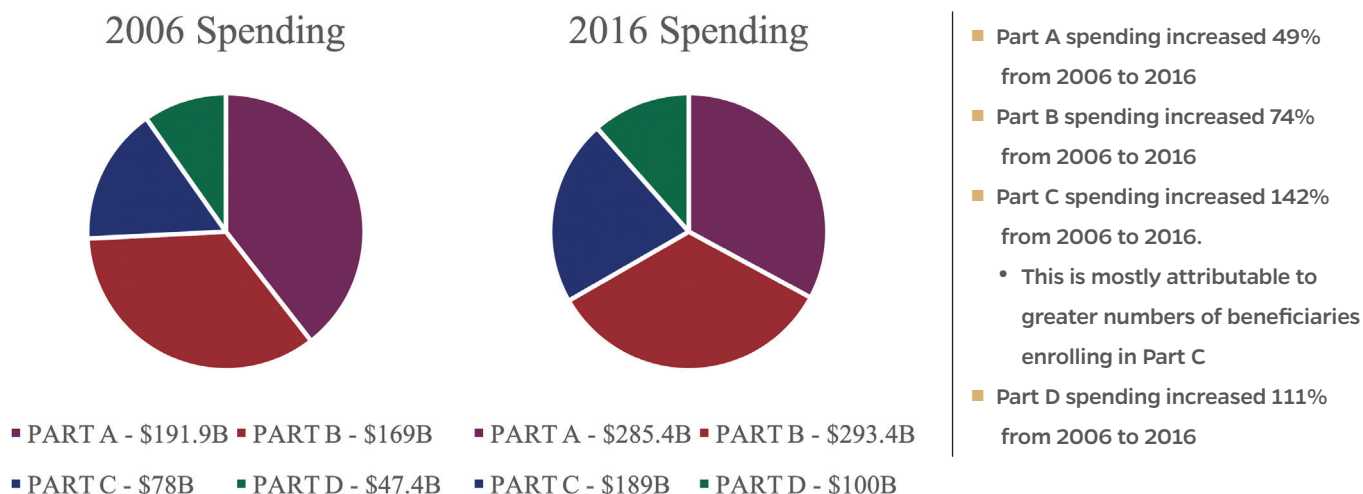
- Part A covers drugs administered as part of the inpatient hospital stay
- Part B covers outpatient drugs administered by physicians and other clinicians.
- Part C covers drugs provided by managed care organizations
- Part D covers outpatient drugs filled by pharmacies.

Of these four programs, Part D is the largest program providing drug coverage. It is an optional Medicare prescription drug benefit. Approximately 72% of all Medicare beneficiaries are enrolled in Part D; of these 42% are enrolled in a Medicare Advantage Part D plan while 58% are in a traditional standalone Part D plan.¹⁰ Medicare Part D spent \$100 billion on drugs in 2016. Medicare Part B spent \$27 billion on drugs in 2016.¹¹

⁹ Analysis was conducted using Truven Marketscan ® Commercial Database (Truven Health Analytics, Ann Arbor, MI, USA). <https://marketscan.truvenhealth.com> Refer to the Appendix for a description of the methodology.

¹⁰ Cubanski, J. C., Damico, A., & Neuman, T. (2018, May 17). Medicare Part D in 2018: The Latest on Enrollment, Premiums, and Cost Sharing-sharing. Retrieved from <https://www.kff.org/medicare/issue-brief/medicare-part-d-in-2018-the-latest-on-enrollment-premiums-and-cost-sharing/>

¹¹ U.S. Department of Health and Human Services, Office of the Assistant Secretary for Planning and Evaluation (ASPE). Comparison of U.S. and International Prices for Top Medicare Part B Drugs by Total Expenditures. October 2018. Available at <https://aspe.hhs.gov/system/files/pdf/259996/ComparisonUSInternationalPricesTopSpendingPartBDrugs.pdf>

FIGURE 9: MEDICARE SPENDING BY IN PARTS A, B, C and D^{12 13}

PART D

Medicare Part D, while the largest payers of drugs within the Medicare program, is an optional program for Medicare beneficiaries, and those who elect to enroll, must pay a monthly premium, although Medicare heavily subsidizes the monthly premium. Once enrolled, beneficiaries select a Part D plan sponsor. All Part D plans have similar benefit structures, with a small annual deductible, an initial coverage phase, a non-covered phase known as the “donut hole”, and once a beneficiary exceeds a certain level of out-of-pocket spending, approximately \$5,000 in 2018, the beneficiary enters a catastrophic phase. Many high-cost drugs will push a beneficiary into catastrophic cover-

age with their first prescription fill.

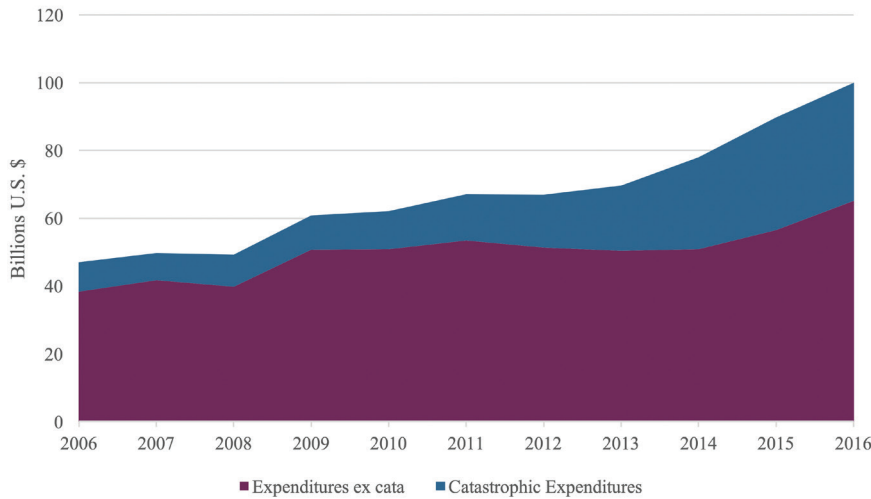
Medicare Part D has more than doubled from 2006-2016. However, Part D catastrophic spending increased over three times faster than all Part D spending (Figure 10). This higher growth rate is largely due to increased prices and use of high-cost drugs. For policymakers, the growth in catastrophic coverage is concerning because there is less incentive for Part D plans to control spending once a beneficiary enters catastrophic coverage and beneficiaries are still on the hook for 5% of drug costs in catastrophic coverage.

¹² Boards of Trustees, 2007 Annual Report of the Boards of Trustees of the Federal Hospital Insurance and Federal Supplementary Medical Insurance Trust Funds, 23 April 2007, <https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/ReportsTrustFunds/downloads/tr2007.pdf> (accessed 8 October 2018).

¹³ Boards of Trustees, 2017 Annual Report of the Boards of Trustees of the Federal Hospital Insurance and Federal Supplementary Medical Insurance Trust Funds, 13 July 2017, <https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/ReportsTrustFunds/Downloads/TR2017.pdf> (accessed 8 October 2018).

¹⁴ Padula, W. V., Ballreich, J., & Anderson, G. F. (2018). Paying for Drugs After the Medicare Part D Beneficiary Reaches the Catastrophic Limit: Lessons on Cost Sharing from Other US Policy Partnerships Between Government and Commercial Industry. *Applied health economics and health policy*, 16(6), 753-763.

FIGURE 10: MEDICARE PART D SPENDING (2006-2016)¹⁵

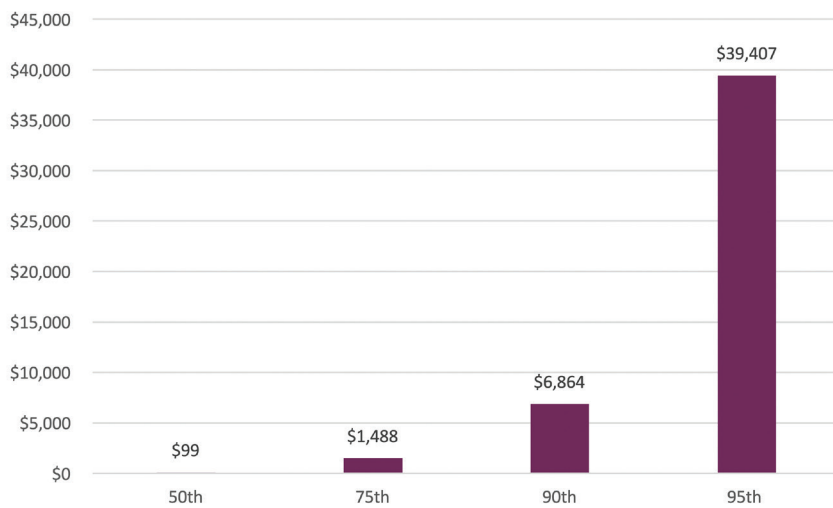


- Medicare Part D spending increased 101% from 2006 to 2016.
- Medicare Part D catastrophic spending increased 335% from 2006 to 2016.
- Much of the higher growth in catastrophic spending is attributable to a few high-priced specialty drugs

Part B covers a range of outpatient drugs with physician-administered drugs being the most common. As shown in **Figure 11**, the average out of pocket spending per claim in Part B for Medicare beneficiaries taking at least one Part B drug is \$1,753. Many of the Part B drugs

are very inexpensive. However, like most drug sending, spending is concentrated in a few drugs and a few beneficiaries. While the average is \$1,753, the 99th percentile of spending is \$39,407.

FIGURE 11: DISTRIBUTION OF BENEFICIARY PART B SPENDING IN 2016



- Average Part B spending per beneficiary with at least 1 drug claim was \$1,735
- While most beneficiaries had minimal Part B drug spending the final 5% had exceptionally large out of pocket spending

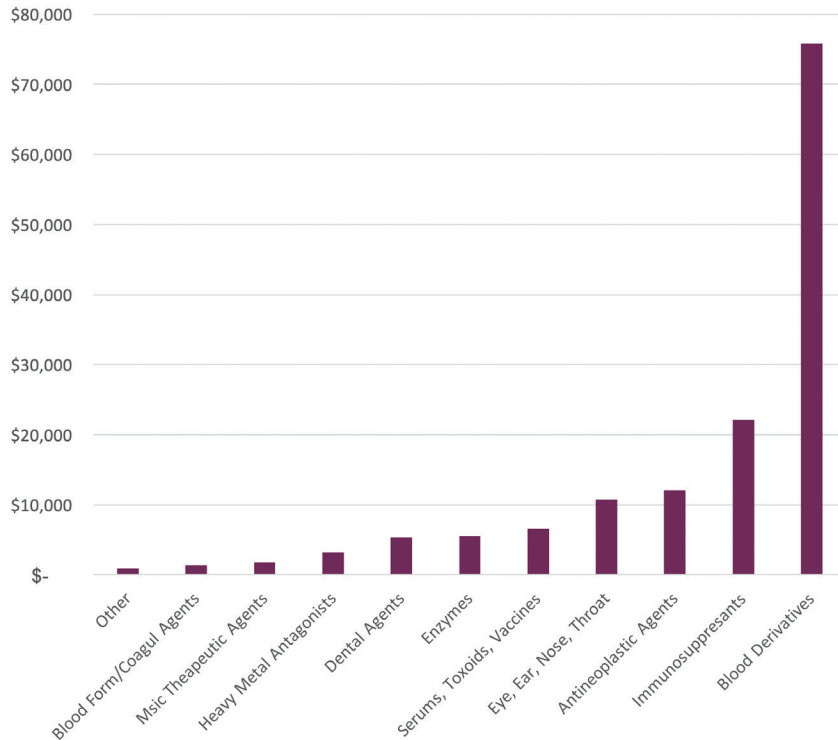
¹⁵ Boards of Trustees, 2017 Annual Report of the Boards of Trustees of the Federal Hospital Insurance and Federal Supplementary Medical Insurance Trust Funds, 13 July 2017, <https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/ReportsTrustFunds/Downloads/TR2017.pdf> (accessed 8 October 2018).

¹⁶ Analysis of Medicare’s 20% restricted sample. See Methodology section for further discussion

Spending in Part B is for a relative few categories of drugs; most commonly drugs to treat cancers and blood diseases. For many Part B drugs, Medicare represents over half

of the drugs market.¹⁷ **Figure 12** shows the per person drug spending in Part B across different disease types of people actually taking a drug in that category.

FIGURE 12: AVERAGE PART B SPENDING FOR SPECIFIC CATEGORIES OF DISEASES IN 2016¹⁸



MEDICAID

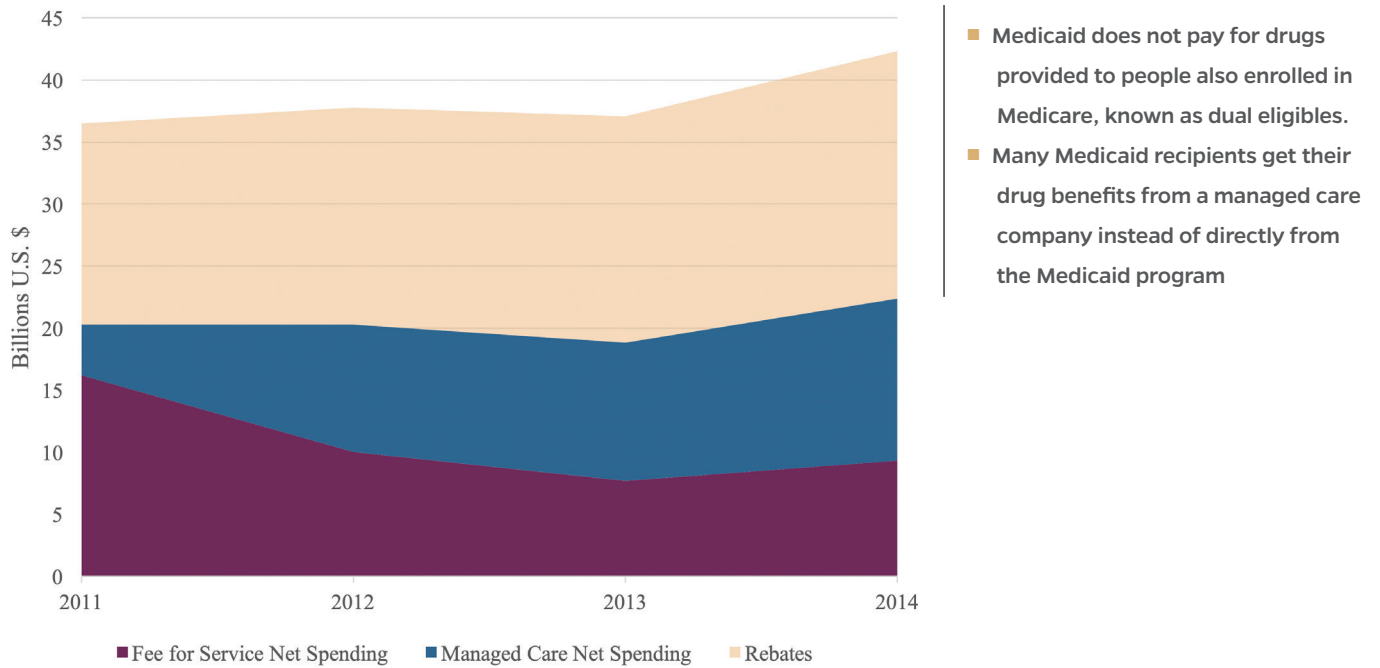
Per the Omnibus Budget Reconciliation Act of 1990 (OBRA-90), Medicaid programs must cover all drugs. However, in return the Medicaid program receives rebates according to a set formula based in part of prices paid by other payers, and Medicaid programs can receive supplemental re-

bates as well. These rebates can be significant, amounting to nearly half of Medicaid drug spending (see **Figure 13**). With the rebates, Medicaid programs often pays the lowest prices for drugs compared to other programs in the U.S.

¹⁷ GAO Report 18-83: MEDICARE PART B: Medicare Represented at Least Half of the Market for 22 of the 84 Most Expensive Drugs in 2015. Accessed: <https://www.gao.gov/products/GAO-18-83>

¹⁸ Analysis of Medicare's 20% restricted sample. See Methodology section for further details.

FIGURE 13: MEDICAID PHARMACEUTICAL SPENDING (2010-2014)¹⁹



- Medicaid does not pay for drugs provided to people also enrolled in Medicare, known as dual eligibles.
- Many Medicaid recipients get their drug benefits from a managed care company instead of directly from the Medicaid program

¹⁹ Medicaid and CHIP Payment and Access Commission 2016 Report on Medicaid Prescription Drug Expenditures. <https://www.macpac.gov/publication/medicaid-spending-for-prescription-drugs/> (Accessed September 1, 2018).

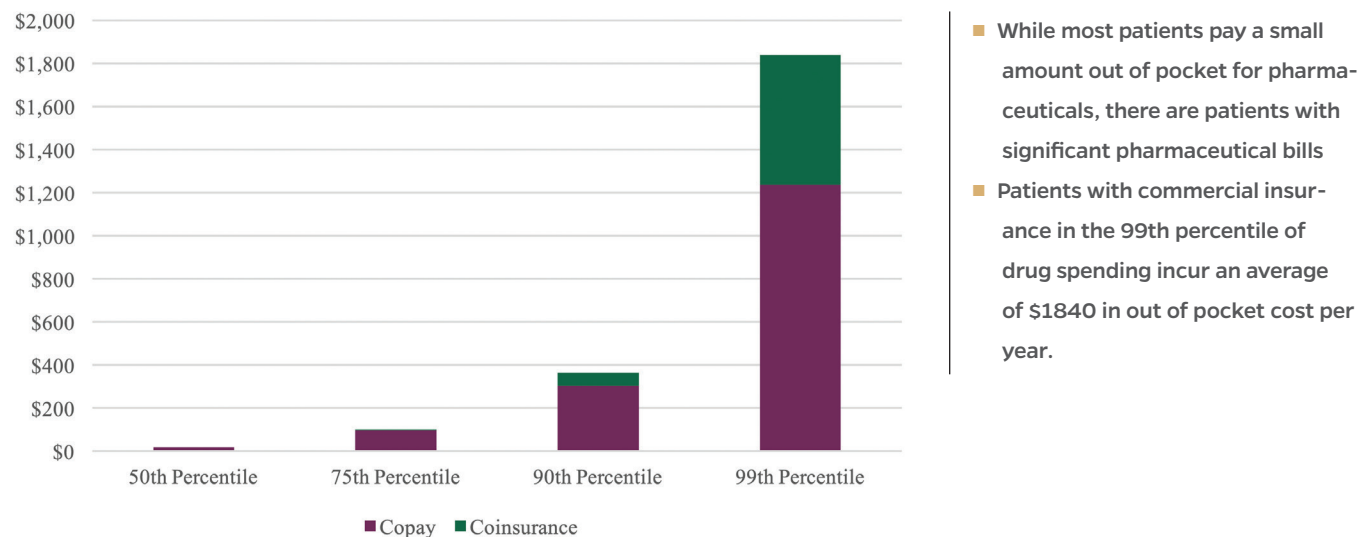
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How Much do Patients Pay Out of Pocket for Drugs?

Pharmaceuticals provide patients with effective and at times life-saving treatments for a variety of conditions. However, for pharmaceuticals to be effective, patients

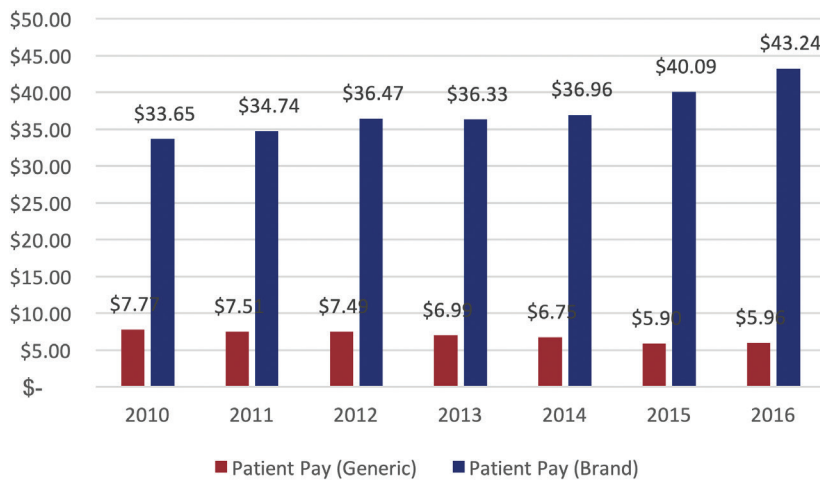
must be able to access these pharmaceuticals. While insurance is responsible for most of the direct cost of many pharmaceuticals, for certain drugs the patient can face high out-of-pocket costs when filling their prescriptions. These drugs typically are only used by a few patients, but they are responsible for a significant portion of out-of-pocket spending by commercially insured patients and Medicare beneficiaries alike.

FIGURE 14: COST-SHARING IN COMMERCIAL MARKET (2016)²⁰



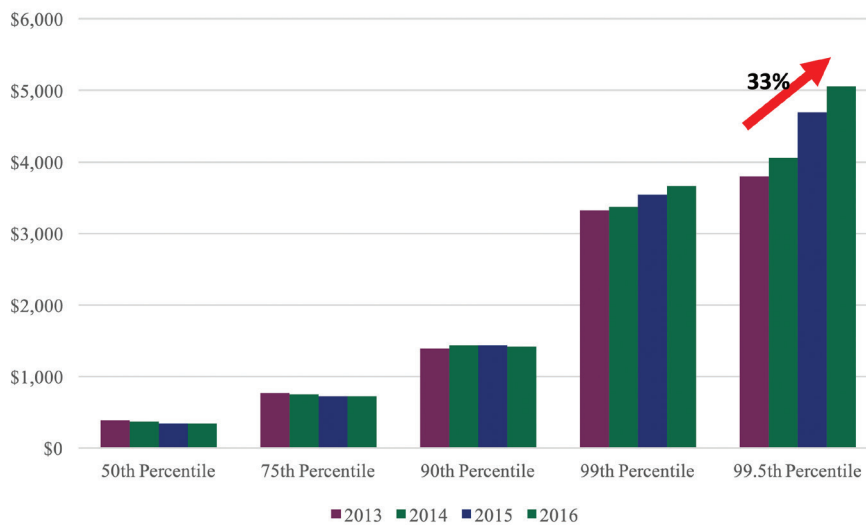
²⁰ Analysis was conducted using Truven MarketScan® Commercial Database (Truven Health Analytics, Ann Arbor, MI, USA). <https://marketscan.truvenhealth.com> Refer to the Appendix for a description of the methodology

FIGURE 15: AVERAGE OUT OF POCKET SPENDING PER PRESCRIPTION FOR BRANDED AND GENERIC DRUGS (2010-2016)²¹



- Average patient's out of pocket cost for generic drugs declined 23% from 2010 to 2016
- Average patient's out of pocket cost for brand drugs increased 28% from 2010 to 2016

FIGURE 16: COST-SHARING IN MEDICARE PART D (2016)



²¹ Analysis was conducted using Truven MarketScan® Commercial Database (Truven Health Analytics, Ann Arbor, MI, USA). <https://marketscan.truvenhealth.com> Refer to the Appendix for a description of the methodology.

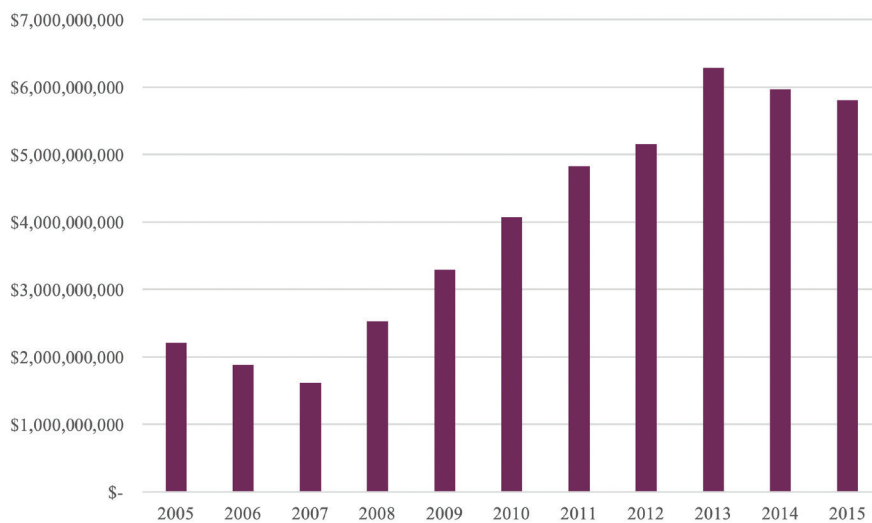
²² Analysis of Medicare's 20% restricted sample. See Methodology section for further discussion.

PATIENT ASSISTANCE PROGRAMS

Many pharmaceutical companies recognize the burden of cost-sharing on patients' access to medications, especially high-priced medications. To help patients gain access to these medications, and therefore for the drug company to sell more medications, many pharmaceutical companies have donated significant money to Patient Assistance Programs. Patient Assistance Programs are charities that pro-

vide financial assistance to mostly low-income patients to cover a portion or all of the patients' cost-sharing responsibility. However, the person must be insured to qualify. For 15 large Patient Assistance Programs, annual expenditures by these patient assistance programs have grown three-fold from 2005 to 2015 to nearly \$6 billion (**Figure 17**).

FIGURE 17: PATIENT ASSISTANCE PROGRAM SPENDING 2005-2015²³



²³ Frerick, A., "The Cloak of Social Responsibility: Pharmaceutical Corporate Charity," Tax Notes, November 28, 2016, at https://papers.ssrn.com/sol3/papers.cfm?abstract_id=2874391.

SECTION 2 - ACCESS

2.1

What Types of Drugs Do Americans Purchase?

There are many different types of drugs. One distinction is between small molecule drugs and biologics. Small molecule drugs are drugs with simple molecular structures that are relatively easy to manufacture and represent most of the drugs prescribed. Biologics are drugs that are large molecule and involve complex manufacturing utilizing biologic processes. They tend to have high prices and represent some of the largest spend drugs (see **Figure 18** for the top 10 drugs by sales).

Most drugs that are prescribed are small molecule generic drugs, but because of their low prices, they represent a smaller fraction of total spending on pharmaceuticals. The “generic” versions of biologic drugs are called biosimilars.

There are few biosimilars available in the U.S. while many more biosimilars are available in Europe.

Some drugs are specialty drugs. These are the drugs that are branded and are high cost designed to treat specific conditions. In 2018, Medicare defines these drugs as costing more than \$670 per month. They are responsible for much of the recent increase in drug spending.

Some drugs treat rare or orphan diseases. The most commonly used definition for orphan diseases are diseases that affect less than 200,000 people in the U.S. Orphan drugs have a special FDA approval pathway that once approved grants pharmaceutical companies tax credits for research and development as well as additional years of market exclusivity.

While thousands of drugs exist, relatively few drugs represent a significant portion of U.S. drug spending (see **Figure 18**). The top ten drugs represent 14.3% of all drug spending

FIGURE 18: TOP TEN DRUGS IN U.S. BY SPENDING (2016 \$billions)²⁴



“B” indicates a biologic drug, “S” indicates a specialty drug, “O” indicates the drug has multiple organ drug

²⁴ IQVIA Institute for Human Data Science. Medicines use and spending in the U.S.: a review of 2017 and outlook to 2022. April 2018, <https://www.iqvia.com/institute/reports/medicine-use-and-spending-in-the-us-review-of-2017-outlook-to-2022> (Accessed 15 September 2018)

FIGURE 19: TOP TEN DRUGS BY MEDICARE PART D SPENDING IN 2016²⁵

DRUG	Total Spending	Number of Beneficiaries	Spending per Beneficiary	Condition	Orphan Approval
Harvoni	\$4,399,701,570	52,804	\$83,321	Hepatitis C	yes
Revlimid	\$2,661,602,600	35,376	\$75,237	Cancer	yes
Lantus Solostar	\$2,526,426,477	1,075,461	\$2,349	Diabetes	
Januvia	\$2,440,387,993	864,638	\$2,822	Diabetes	
Crestor	\$2,323,133,630	1,560,382	\$1,488	High Cholesterol	yes
Advair Diskus	\$2,320,125,120	1,196,224	\$1,939	Asthma/COPD	
Lyrica	\$2,099,262,044	852,699	\$2,461	Fibromyalgia	
Xarelto	\$1,955,000,084	807,973	\$2,419	Blood clots	
Eliquis	\$1,926,316,211	827,097	\$2,329	Blood clots	
Spiriva	\$1,819,084,753	903,652	\$2,013	Asthma/COPD	yes

The ten drugs with the most spending in Medicare Part D accounted for \$28.4 billion in 2016 or 31.6% of spending in Medicare Part D.

Medicare beneficiaries pay very different levels of cost-sharing for these drugs. The amount of cost-sharing is highly correlated with the list price

FIGURE 20: TOP TEN DRUGS IN MEDICARE PART B BY SPENDING IN 2016²⁶

DRUG	Total Spending	Spending Per User	Average Provider Margin (ASP+6%)	Condition
Injection, aflibercept, 1 mg	\$2,207,882,153	\$10,497	\$630	Age-related Macular Degeneration
Injection, rituximab, 100 mg	\$1,663,513,650	\$23,812	\$1,429	Rheumatoid Arthritis
Injection, pegfilgrastim, 6 mg	\$1,374,291,300	\$14,330	\$860	Cancer
Injection, infliximab, excludes biosimilar, 10 mg	\$1,336,041,105	\$22,896	\$1,374	Rheumatoid Arthritis
Injection, nivolumab, 1 mg	\$1,219,517,891	\$44,082	\$2,645	Cancer
Injection, bevacizumab, 10 mg	\$1,110,360,974	\$5,357	\$321	Cancer
Injection, denosumab, 1 mg	\$1,085,135,202	\$2,592	\$156	Osteoporosis
Injection, ranibizumab, 0.1 mg	\$1,044,105,659	\$9,814	\$589	Age-related Macular Degeneration
Injection, trastuzumab, 10 mg	\$702,888,944	\$33,990	\$2,039	Cancer
Pneumococcal vaccine for injection into muscle	\$667,483,118	\$170	\$10	Pneumonia

The top ten drugs amounted to \$11.2 billion in 2016, which represented 41.5% of total Part B spending on drugs or 3.8% of total spending in Part B.

In 2016, physicians were paid 6% to administer these drugs in addition to their professional fee. This creates a financial incentive for physicians to prescribe more expensive drugs. This has now dropped to 4.4%

²⁵ Medicare Drug Spending Dashboard. (2018, May 23). Retrieved from <https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/Information-on-Prescription-Drugs/MedicarePartD.html>

²⁶ Medicare Drug Spending Dashboard. (2018, May 23). Retrieved from <https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/Information-on-Prescription-Drugs/MedicarePartD.html>

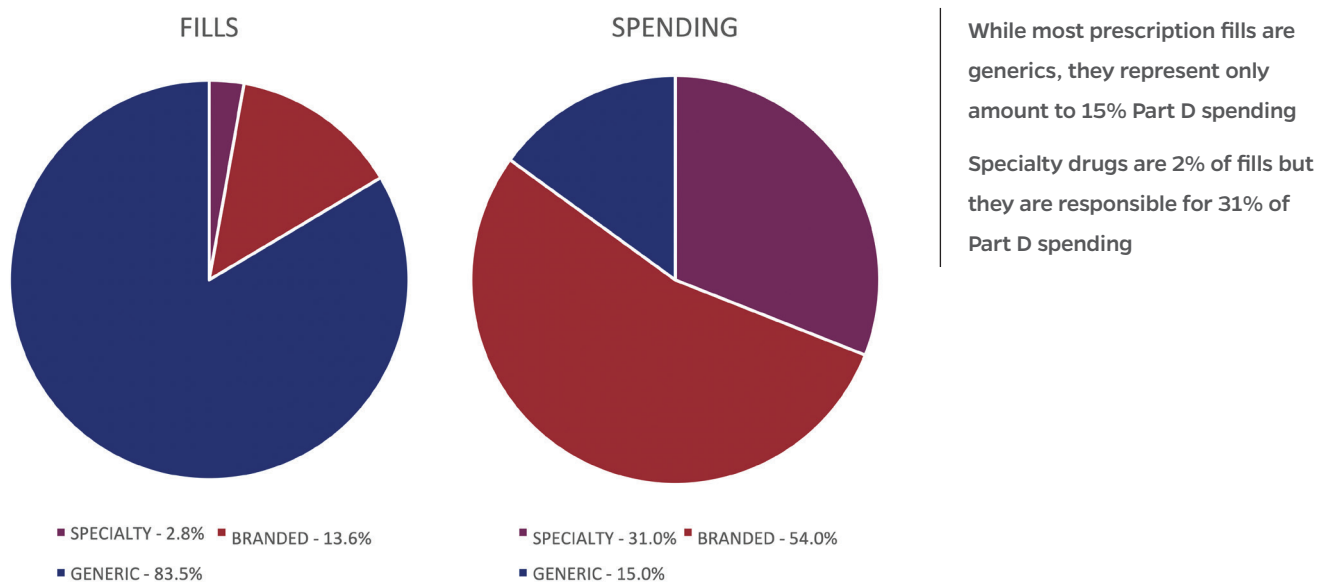
TYPES OF DRUGS USED IN MEDICARE

Medicare is the single largest payer of drugs in the United States, and by examining the type of drugs used in Medicare, one can get a sense of the types of drugs used in the entire market.

The first major observation is that most drugs are generics. We see in **Figure 21** generic drugs account for nearly 84% of prescription fills, whereas branded non-specialty

drugs account for 13.6% of the fills, and specialty drugs, which are defined as costing over \$670 per month, account for just 2.8% of the fills. However, generics are very low cost, so despite accounting for nearly 84% of the fills, they represent just 15% of the spending. Branded and specialty drugs account for most of the drug spending in Medicare Part D.

FIGURE 21: MEDICARE PART D SPENDING AND FILLS BY DRUG TYPE IN 2016²⁷

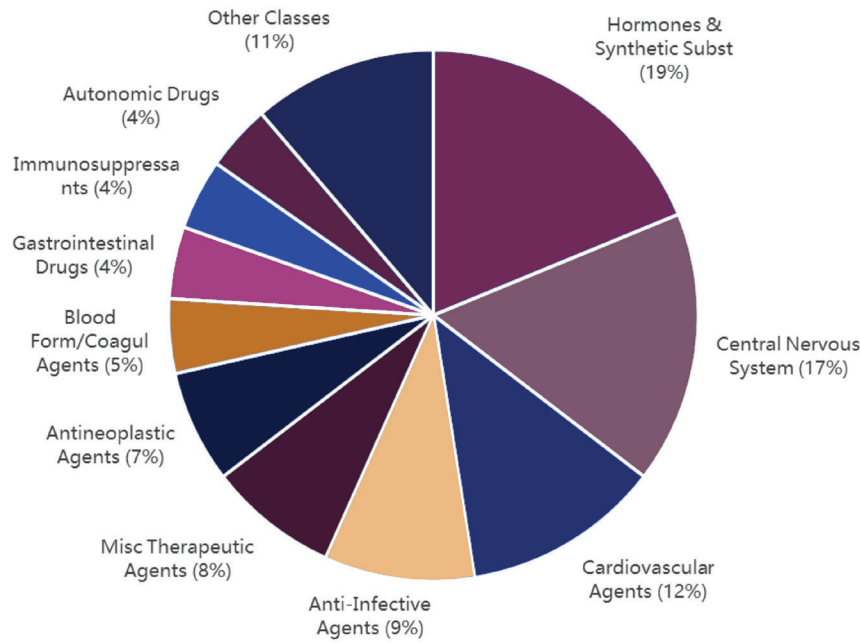


When looking at the therapeutic categories of drugs, we see spending spread out across 31 therapeutic groups (**Figure 22**). However, when we examine the distribution of fills across therapeutic groups, we see the fills much more

concentrated in certain therapeutic groups notably the Central Nervous System drugs and Cardiovascular Agents (**Figure 23**).

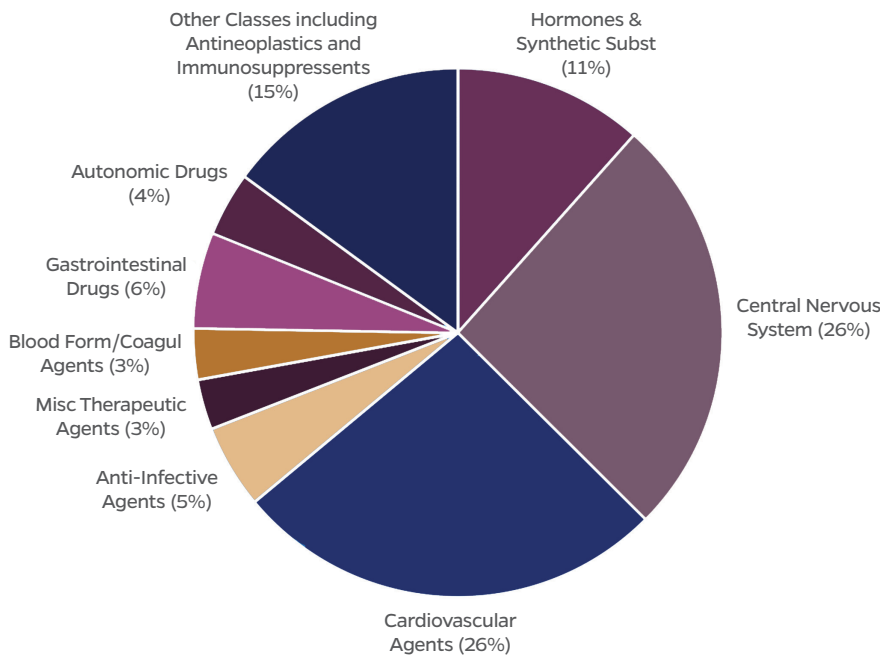
²⁷ Analysis of Medicare's 20% restricted sample. See *Methodology* section for further discussion.

FIGURE 22: MEDICARE PART D SPENDING BY THERAPEUTIC GROUP IN 2016²⁸



- Medicare Part D spending is spread across 31 therapeutic groups.
- The top 10 groups account for 90% of spending
- For some therapeutic groups, the level of spending is similar to the number of fills. But for others such as Antineoplastic Agents, which treat cancer, the spending percentage is a much proportion than the number of fills (see Figure 23).

FIGURE 23: MEDICARE PART D FILLS BY THERAPEUTIC GROUP IN 2016²⁹



²⁸ Analysis of Medicare's 20% restricted sample. See *Methodology* section for further discussion.

²⁹ Analysis of Medicare's 20% restricted sample. See *Methodology* section for further discussion.

2.2

How Do Insurers Control Access to Drugs?

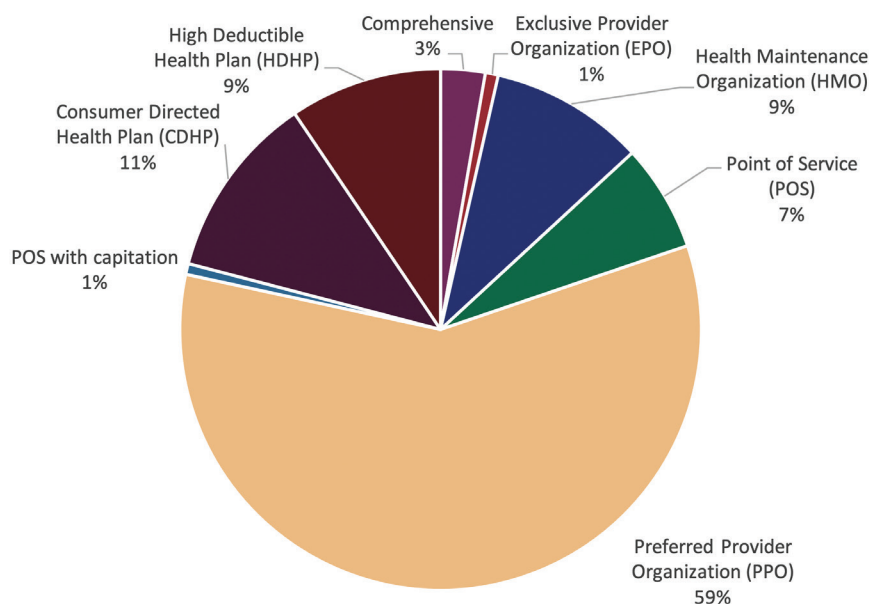
On average, health insurers pay 85% of a drug's cost. Nearly all insurers use a formulary to control drug access. A formulary dictates what drugs insurance covers and helps determine how much they pay. Formularies may also implement utilization management policies to control usage. For example, some drugs may have a step-therapy requirement, which would require prescribers to try drugs in a certain order for specific conditions or diagnoses. State Medicaid programs are the only major insurer without a formulary.

PRIVATE INSURERS

In the private insurance market, employers can choose from a number of different plan designs to offer employees. Some plans have high deductibles, which means patients bear a greater financial burden with prescription drugs, while other plans use restrictive networks to get lower negotiated prices and this may require a patient filling a drug at specific pharmacies.

Many employers carve out the prescription drug insurance from health insurance and this may result in misaligned incentives between drug and health insurers since there can be tradeoffs between spending on drugs and spending on medical services. There is a wide variety of types of insurance plans (**Figure 24**), and even across plan types, employers can choose varying levels of cost-sharing or formularies to offer employees.

FIGURE 24: DISTRIBUTION OF PLANS IN LARGE EMPLOYER MARKET³⁰



- The most common plan type is Preferred Provider Organizations (PPOs), which incentivizes patients to see providers within a network. For prescription drugs, PPOs may require patients get in-network provider authorization before filing an expensive brand name drug
- Patients with Consumer Directed Health Plans (CDHP) and High Deductible Health Plans (HDHP) may face high deductibles before plans help pay for prescription drugs. This can expose patients to high list prices for drugs.

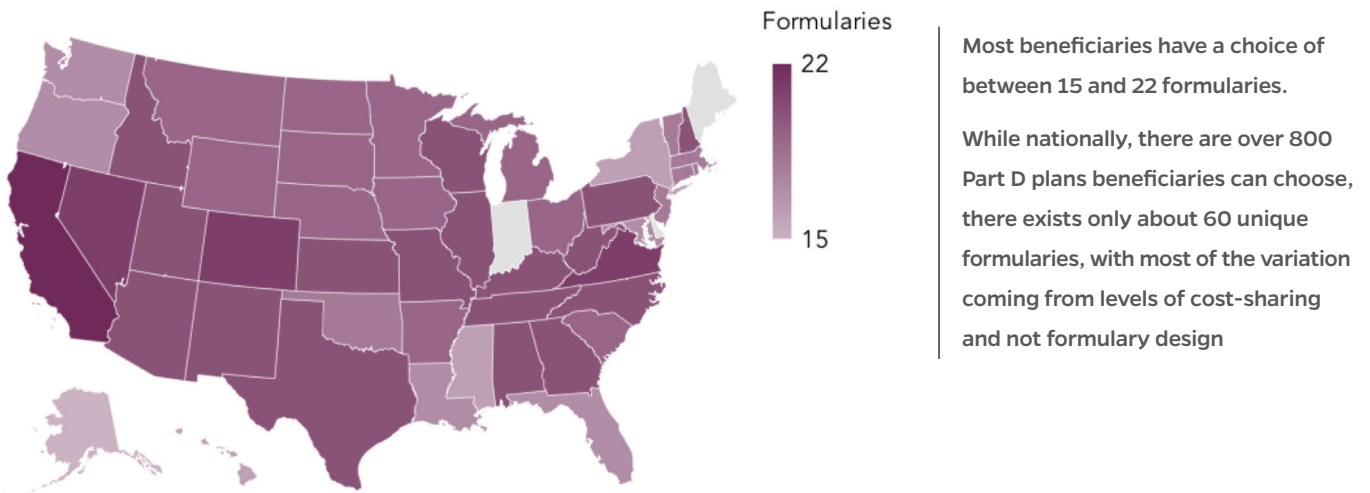
³⁰ Analysis was conducted using Truven Marketscan® Commercial Database (Truven Health Analytics, Ann Arbor, MI, USA). <https://marketscan.truvenhealth.com> Refer to the Appendix for a description of the methodology

MEDICARE

For Medicare Part D beneficiaries, the choice of formularies depends on where they live. The number of formularies in each state is shown in **Figure 25**. These formularies are controlled by the Part D plans. The plans are administered by Pharmaceutical Benefit Managers (PBM), who negoti-

ate drug favorable placements on formularies in return for rebates on the drug prices. Because of the negotiations, not all drugs are offered across all plans. In **Figure 26** the percentages of all drugs that are covered by all formularies are presented.

FIGURE 25: MEDICARE FORMULARIES BY STATE³¹

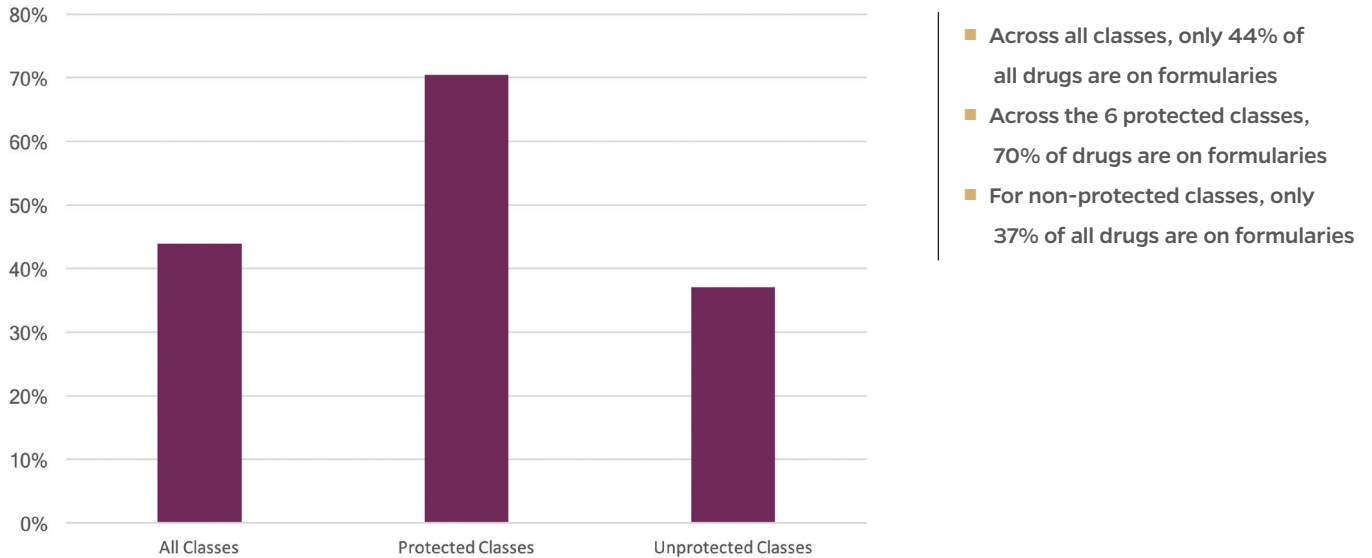


³¹ Analysis of Medicare’s 20% restricted sample. See *Methodology* section for further discussion.

Medicare Part D Plans have some freedom in designing their formularies, but they are required to cover all drugs across six therapeutic classes. However, recent analysis

suggests this is not true. In **Figure 26** we see most drugs are not universally offered across all plans. Even for the protected classes, we see only 70% of all drugs offered.

FIGURE 26: COVERAGE OF DRUGS BY MEDICARE FORMULARIES³²



MEDICAID

Medicaid is required to cover all drugs. However, Medicaid can place certain restrictions on their use. An example of this is access to expensive direct acting antivirals to treat hepatitis C, the first of which were approved in December 2013. While effective, the high prices of these drugs re-

sulted in state Medicaid programs restricting access to patients. Despite a growth in these drugs utilization in state Medicaid programs (see **Figure 27**), for many states, most people with chronic hepatitis C with Medicaid still do not have access to the drug (see **Figure 28**).

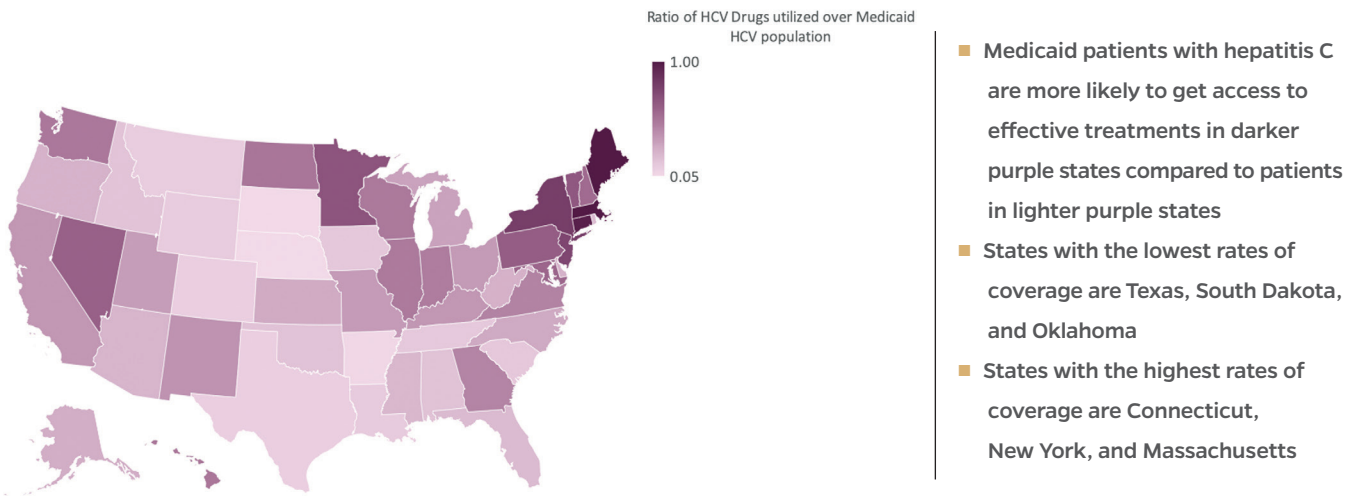
³² Analysis of Medicare's 20% restricted sample. See *Methodology* section for further discussion.

FIGURE 27: HEPATITIS C DRUGS IN MEDICAID³³



- Some states quickly prescribed new direct acting antivirals, while others did not
- Prescribing rates went down in recent years despite a large untreated hepatitis C population

FIGURE 28: AMOUNT OF HEPATITIS C DRUGS UTILIZED FOR HEPATITIS C INFECTED MEDICAID POPULATIONS IN 2017³⁴



- Medicaid patients with hepatitis C are more likely to get access to effective treatments in darker purple states compared to patients in lighter purple states
- States with the lowest rates of coverage are Texas, South Dakota, and Oklahoma
- States with the highest rates of coverage are Connecticut, New York, and Massachusetts

Darker shades indicate higher levels of Hepatitis C drug treatment for infected Medicaid populations. Values range from .05 to 1, suggesting enough hepatitis C drug treatments have been utilized by Medicaid from 2014 to 2017 to treat 5% to nearly 100% of the estimated infected Medicaid populations. Poor surveillance and reliance on older data may underestimate hepatitis C Medicaid populations.

³³ Analysis of state Medicaid Drug utilization from data.medicaid.gov. See *Methodology* section for details.

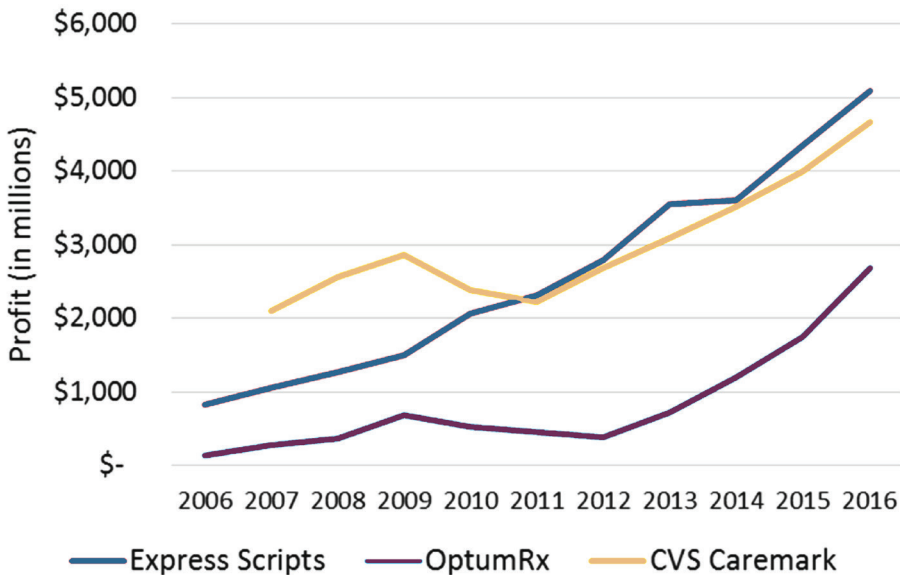
2.3

What are Pharmaceutical Benefit Managers?

The US pharmaceutical market consists of more than just drug companies and insurers. A major player in the market is Pharmaceutical Benefit Managers (PBMs). They negotiate between the drug companies and health insurers, using their market power to lower prices.

With consolidation, the three largest PBMs control 80% of the PBM market. They have been able to use their market power to extract rebates from drug manufacturers. These rebates have resulted in significant profit growth recently by PBMs (see **Figure 29**). The increase in market power of PBMs has also corresponded to an increase in list price for consumers, slower increases in net prices, and a larger portion of rebates to PBMs.

FIGURE 29: PROFIT GROWTH OF PHARMACEUTICAL BENEFIT MANAGERS



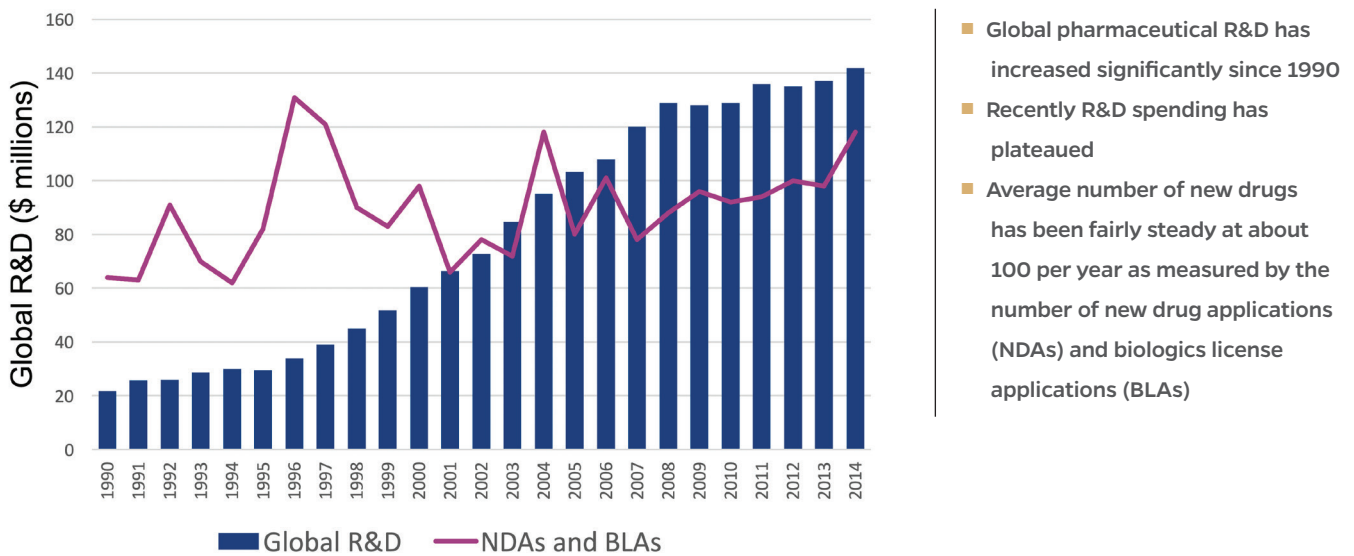
SECTION 3 - INNOVATION

3.1

How Has the Drug Industry Changed Recently?

The research and development cost for new drugs is often a costly and is the primary justification for the market protections granted to pharmaceuticals. The size of global research and development and its productivity measured by new drug approvals is seen in **Figure 30**.

FIGURE 30: TRENDS IN GLOBAL R&D³⁵



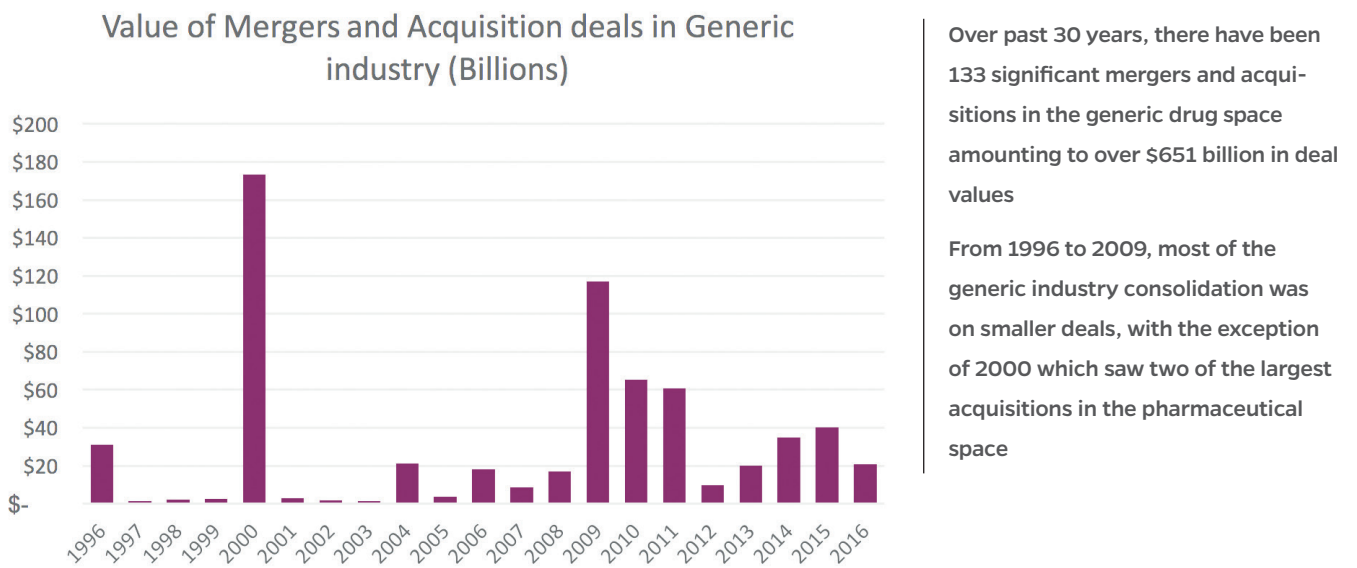
³⁵ Ballreich, J., Bai, G., Hsu, M., Traver, A., & Anderson, G.F. (2016). Dual Approaches for Estimating Drug Development Costs.

GENERICS INDUSTRY

While mergers and acquisitions are a natural part of any industry, in the past 10 years the generic industry has seen a wave of consolidation including many of the largest manufacturers (see **Figure 31**). When industries consoli-

date, there is less competition and with less competition and generic drug prices may rise. This is because price competition is important in the generic industry because the generic companies compete on price because the products are the same.

FIGURE 31: GENERIC INDUSTRY CONSOLIDATION BETWEEN 1996 AND 2016³⁶



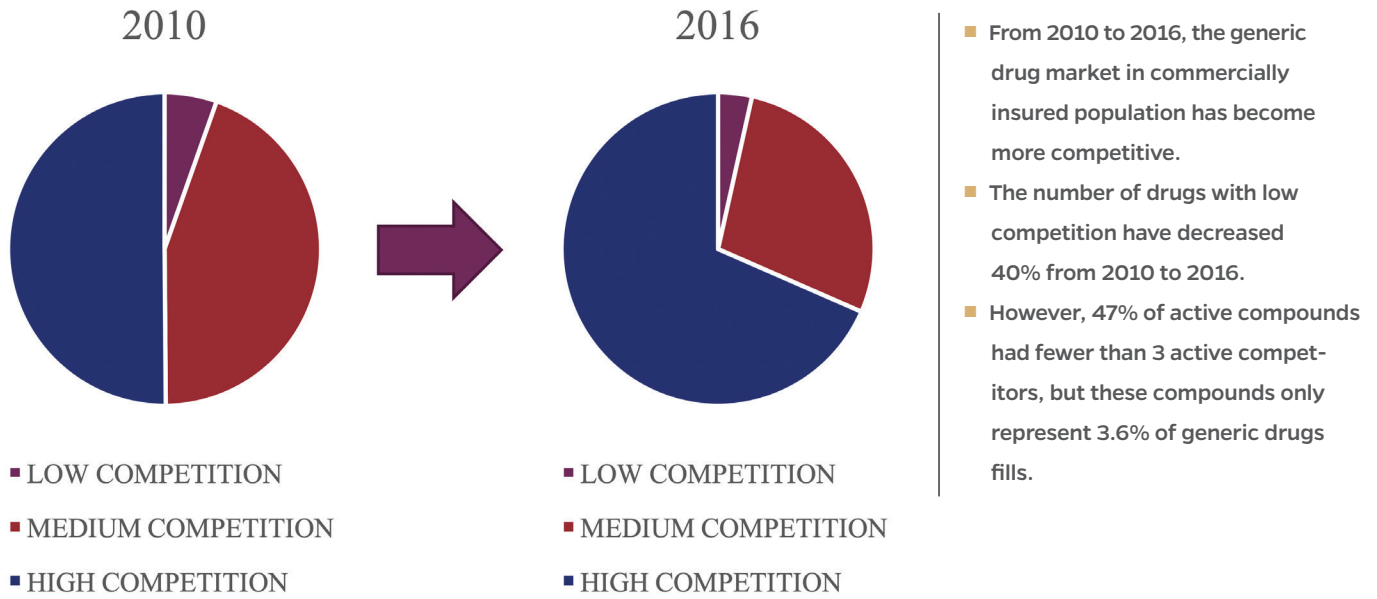
Generic drugs are not protected by patents and market exclusivity periods and therefore should be in highly competitive markets. However, in the commercially insured market, we find many generic drug markets as having low or medium levels of competition (**Figure 32**).

Herfindahl-Hirschman Index, which is a measure of market concentration. The concern for policymakers is drugs with low competition can experience significant price increases, such as Daraprim, a generic that Martin Shkreli raised the price by 5000%.

Levels of competition are commonly categorized by the

³⁶ Based on analyses of Securities Data Company (SDC) Platinum, an online historical financial transactions database by Thomson Reuters; Federal Trade Commission data; and FDA's Orange Book

FIGURE 32: LEVELS OF COMPETITION ACROSS GENERIC DRUGS IN 2010 AND 2016³⁷



³⁷ Analysis was conducted using Truven MarketScan® Commercial Database (Truven Health Analytics, Ann Arbor, MI, USA). <https://marketscan.truvenhealth.com> Refer to the Appendix for a description of the methodology.

3.2

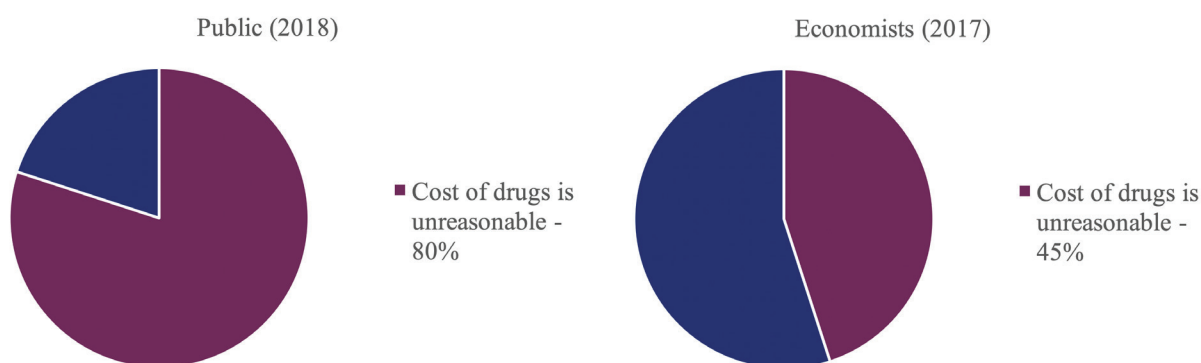
What is the Public's Perception of Drug Pricing?

The price of drugs has long been a concern for the public. The first important hearings were the 1960 Kefauver Hearings on drug prices in the United States Senate.³⁸ Some of the recent attention has focused on drug price scandals. According to a 2018 Kaiser Family Foundation poll, 80% of

the public believes branded drug prices are unreasonable (see **Figure 33**).

Economists are more likely to accept market prices as being fair. As a result, it is surprising that according to a 2017 survey of economists, 45% of economists believe drug prices are unfair (see **Figure 31**). When asked why prices are unfair, the economists largely suggested concerns over access and not the drug company's profit-maximizing behaviors or the spending on research and development (See **Figure 34**).

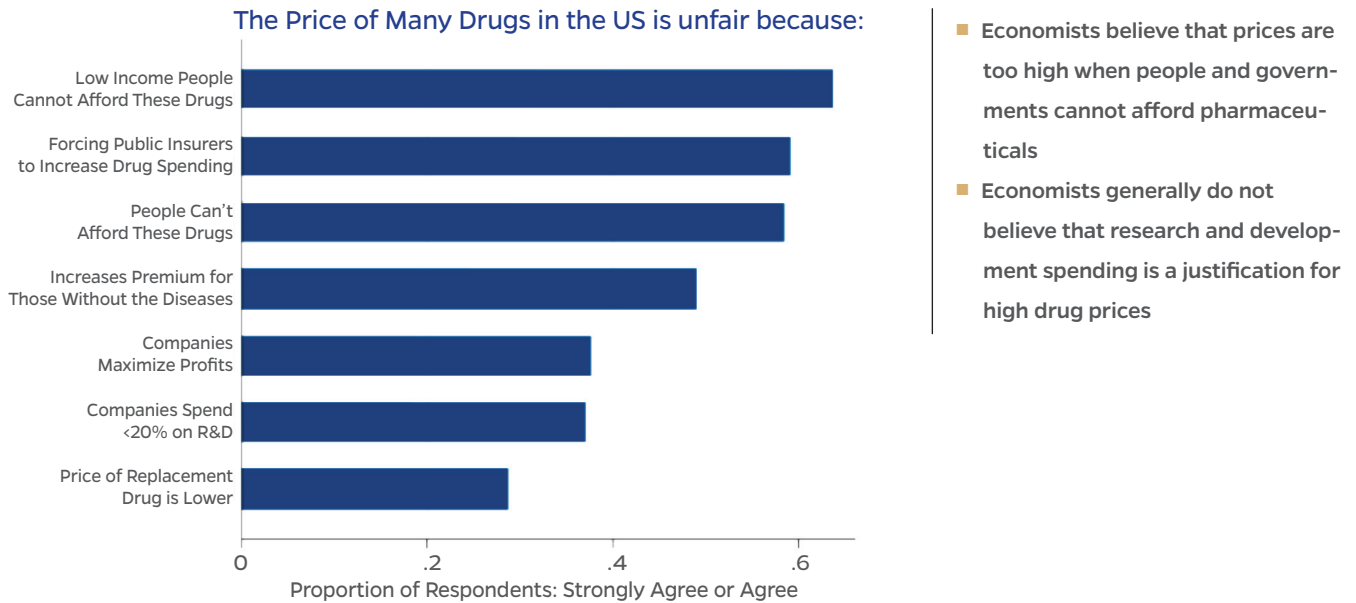
FIGURE 33: PERCEPTION ON DRUG PRICES^{39 40}



³⁸ Greene, J. A., & Podolsky, S. H. (2012). Reform, regulation, and pharmaceuticals—the Kefauver–Harris Amendments at 50. *New England Journal of Medicine*, 367(16), 1481-1483.

³⁹ Public Opinion on Prescription Drugs and Their Prices. (2018, November 28). Retrieved from <https://www.kff.org/slideshow/public-opinion-on-prescription-drugs-and-their-prices/>

⁴⁰ Trujillo, A. J., Karmarkar, T., Alexander, C., Padula, W., Greene, J., & Anderson, G. (2018). Fairness in drug prices: do economists think differently from the public?. *Health Economics, Policy and Law*, 1-12.

FIGURE 34: ECONOMIST PERCEPTIONS OF WHY DRUG PRICES ARE UNFAIR IN 2017⁴¹

⁴¹ Trujillo, A. J., Karmarkar, T., Alexander, C., Padula, W., Greene, J., & Anderson, G. (2018). Fairness in drug prices: do economists think differently from the public?. *Health Economics, Policy and Law*, 1-12.

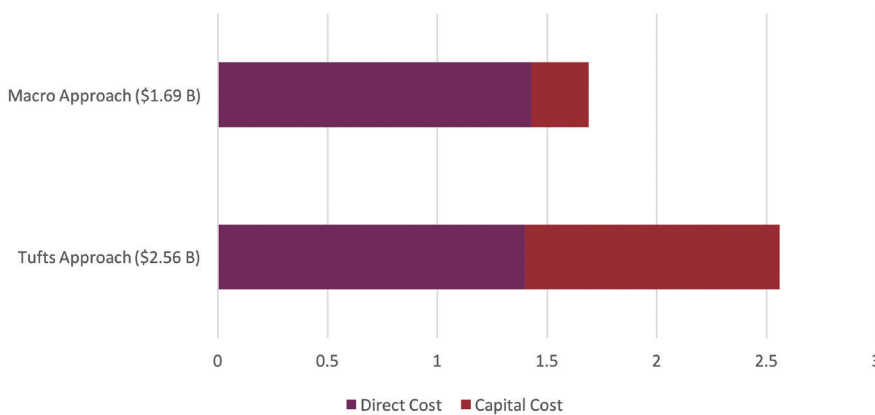
3.3

What is the Cost to Develop a Drug?

There is considerable debate concerning the exact cost of R&D for a drug and many of the studies use proprietary data. Using publicly available data, we estimate the cost to bring a drug to market at \$1.7 billion.⁴² This was esti-

mated by examining the total global pharmaceutical R&D spend, published literature on the time it takes for development, and number of new drugs. The most commonly cited estimate for the cost to develop a drug is from team at Tufts University, who use proprietary data. In 2016, they estimate the cost to be \$2.6 billion.⁴³ One reason for such a high value is that a high percentage of the reported cost in the \$2.6 billion figure is the opportunity cost of capital, which they estimated to be 10.5%.

FIGURE 35: AVERAGE COST OF R&D PER DRUG



Drug development is fraught with failures and takes time. Economists value the time and investment risk as a “cost of capital”.

The commonly cited Tufts approach showing \$2.6 billion uses a cost of capital assumption of 10.5%, which is substantially higher than borrowing costs

⁴² Ballreich, J., Hsu, M., Thayer, A., Bai, G., & Anderson, G. (2017, May). Two Transparent Methods for Estimating Drug Research And Development Costs. *VALUE IN HEALTH* (Vol. 20, No. 5, pp. A16).

⁴³ DiMasi, J. A., Grabowski, H. G., & Hansen, R. W. (2016). Innovation in the pharmaceutical industry: new estimates of R&D costs. *Journal of health economics*, 47, 20-33.

3.4

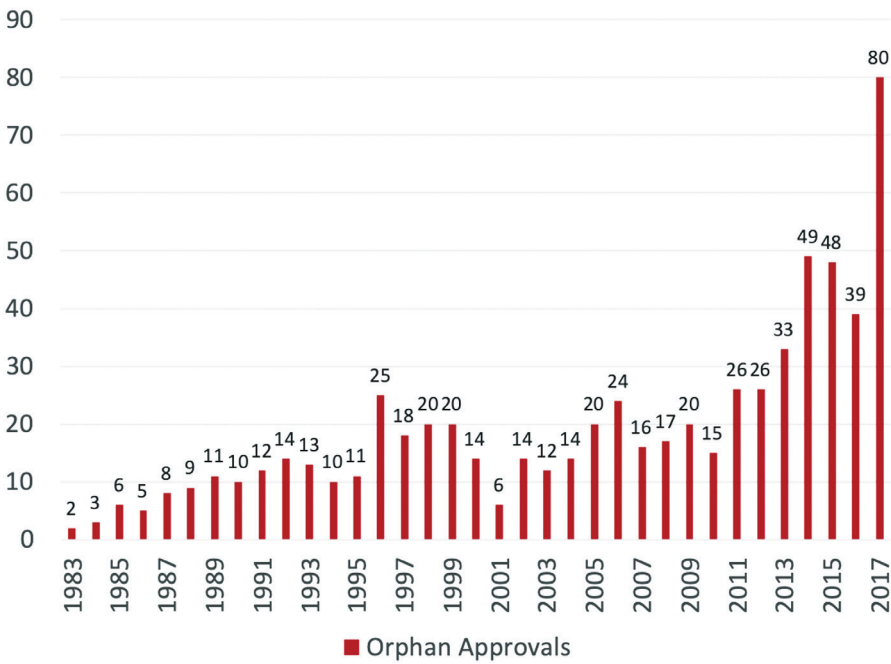
What are Orphan Drugs?

Orphan drugs treat rare diseases, with rare being defined as a disease affecting fewer than 200,000 people. Since these diseases have small markets, there were few drugs being developed for these diseases before the passage of

the Orphan Drug Act (ODA) in 1983.⁴⁴ The ODA provides significant financial incentives for the development of orphan drugs.

In recent years, there has been tremendous growth in the number of orphan drugs as seen in **Figure 36**. Some of these orphan drugs are new drugs while others were already approved for other diseases. Recently, we have seen more drugs apply and receive multiple orphan approvals (See **Figure 37**).

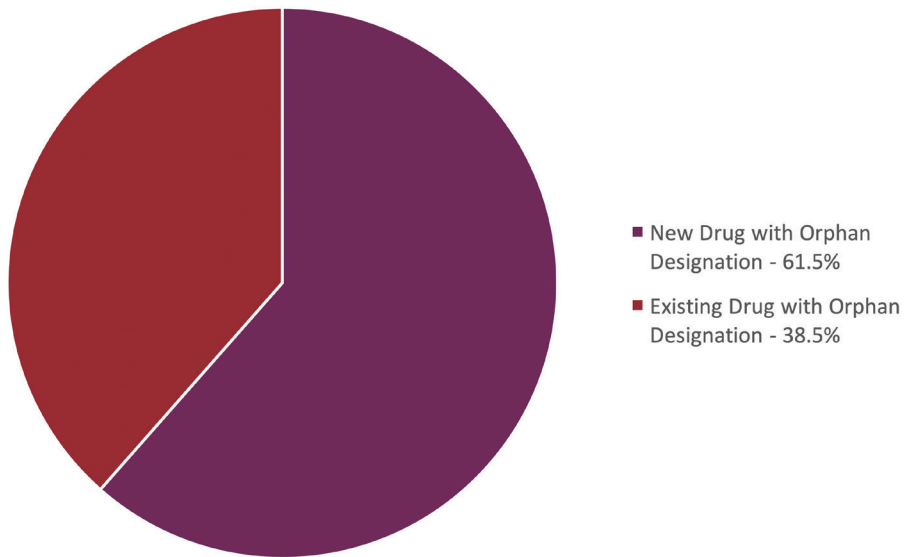
FIGURE 36: TREND IN THE NUMBER OR ORPHAN DRUG APPROVALS⁴⁵



An increasing number of drugs have orphan status are being developed
 However, less than 5% of the approximately 5000 rare diseases have an available drug treatment

⁴⁴ Rohde, D. D. (2000). The Orphan Drug Act: An Engine of Innovation-At What Cost. *Food & Drug LJ*, 55, 125.

⁴⁵ Analysis of Orphan Drug data from the Food and Drug Administration

FIGURE 37: ORPHAN DRUG APPROVALS FOR NEW AND EXISTING DRUGS⁴⁶

- Some orphan drugs have received additional years of market exclusivity by identifying more and more orphan populations
- Some of these “orphan” drugs are also “blockbuster” drugs and may have additional orphan approvals
- Some drugs stack orphan approvals by making applications to the FDA when the orphan status will run out. Drugs with more than 1 approval represent 1/6th of orphan drugs. Many of these are blockbuster drugs.

⁴⁶ GAO Report 19-83: ORPHAN DRUGS: FDA Could Improve Designation Review Consistency; Rare Disease Drug Development Challenges Continue. Accessed: <https://www.gao.gov/products/GAO-19-83>

3.5

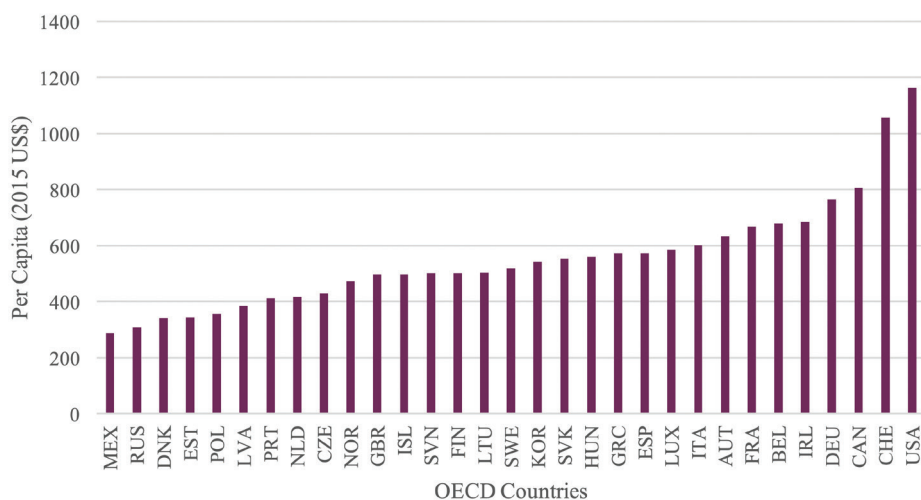
How Does Drug Spending in the US Compare to Other Industrialized Countries?

The U.S. has the highest level of pharmaceutical spending among all industrialized nations. The U.S. spends 2-3 times

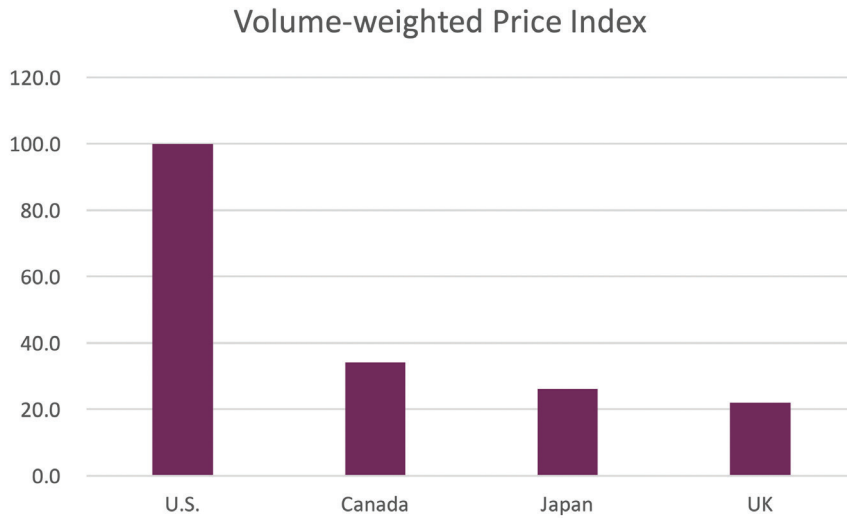
more per person than most other industrialized countries on pharmaceuticals (**Figure 38**).

Much of this spending differential is driven by higher prices and not greater utilization. **Figure 39** compares prices paid in the U.S. to Ontario, Canada, the UK and Japan for branded drugs that have been on the market for more than 3 years. We show the list prices and the prices after rebates in the U.S.

FIGURE 38: U.S. PHARMACEUTICAL SPENDING VS OTHER INDUSTRIALIZED NATIONS (2016)⁴⁷



⁴⁷ OECD (2019), Pharmaceutical spending (indicator). doi: 10.1787/998feb6-en (Accessed on 28 January 2019)

FIGURE 39: U.S., CANADA, JAPAN, and U.K. DRUG PRICE INDEX⁴⁸

U.S. branded pharmaceutical prices average 3-4 times higher than Canada, Japan, and the U.K.⁴⁹

For example, the blockbuster drug HUMIRA® costs on average \$2,504 per month in the United States after discounts. In the United Kingdom, it costs \$1,158, while in Japan, HUMIRA® costs \$980 per month

⁴⁸ Langreth, R., Migliozi, B., & Gokhale, K. (2015, December 18). The U.S. Pays a Lot More for Top Drugs Than Other Countries. Retrieved from <https://www.bloomberg.com/graphics/2015-drug-prices/>

⁴⁹ Kang, S. Y., DiStefano, M. J., Socal, M. P., & Anderson, G. F. (2019). Using External Reference Pricing In Medicare Part D To Reduce Drug Price Differentials With Other Countries. *Health Affairs*, 38(5), 804-811.

CONCLUSION

This chartbook provides policymakers and the public insights into the many facets after US pharmaceutical market. The three individual sections, Affordability, Access, and Innovation, summarize the necessary balance between affordability and innovation. Some of the key points are:

- Pharmaceutical spending is highly concentrated in a small group of patients.
- Pharmaceutical spending is becoming increasingly concentrated in a small group of very expensive drugs.
- While many patients can afford their medications, some have high levels of cost-sharing for expensive drugs which can create affordability issues.

REFERENCES

- Offsetting Effects of Prescription Drug Use on Medicare's Spending for Medical Services. (Nov 2012). Retrieved from <https://www.cbo.gov/publication/43741>
- A Look at Drug Spending in the U.S. (2018). Retrieved from <https://www.pewtrusts.org/en/research-and-analysis/fact-sheets/2018/02/a-look-at-drug-spending-in-the-us>
- Spending On Prescription Drugs In The US: Where Does All The Money Go?, " Health Affairs Blog, July 31, 2018. Centers for Medicare and Medicaid Services, Office of the Actuary, National Health Statistics Group; and US Department of Commerce, Bureau of Economic Analysis, and the US Census Bureau.
- Truven MarketScan® Commercial Database (Truven Health Analytics, Ann Arbor, MI, USA)
- Cubanski, J. C., Damico, A., & Neuman, T. (2018, May 17). Medicare Part D in 2018: The Latest on Enrollment, Premiums, and Cost-sharing. Retrieved from <https://www.kff.org/medicare/issue-brief/medicare-part-d-in-2018-the-latest-on-enrollment-premiums-and-cost-sharing/>
- CMS 2007 and 2017 Trustees Report
- Analysis of Medicare's 20% restricted sample. See *Methodology* section for further discussion.
- Medicaid and CHIP Payment and Access Commission 2016 Report on Medicaid Prescription Drug Expenditures
- Medicines Use and Spending in the U.S. (IQVIA 2017)
- Public Opinion on Prescription Drugs and Their Prices. (2018, November 28). Retrieved from <https://www.kff.org/slideshow/public-opinion-on-prescription-drugs-and-their-prices/>
- Trujillo, A. J., Karmarkar, T., Alexander, C., Padula, W., Greene, J., & Anderson, G. (2018). Fairness in drug prices: do economists think differently from the public?. *Health Economics, Policy and Law*, 1-12.
- Ballreich, J., Bai, G., Hsu, M., Traver, A., & Anderson, G.F. (2016). Dual Approaches for Estimating Drug Development Costs.
- Rohde, D. D. (2000). The Orphan Drug Act: An Engine of Innovation-At What Cost. *Food & Drug LJ*, 55, 125.
- Analysis of Orphan Drug data from the Food and Drug Administration
- Alexander, G. C., Ballreich, J., Socal, M. P., Karmarkar, T., Trujillo, A., Greene, J., ... & Anderson, G. (2017). Reducing branded prescription drug prices: A review of policy options. *Pharmacotherapy: The Journal of Human Pharmacology and Drug Therapy*, 37(11), 1469-1478.
- Kang, S. Y., DiStefano, M. J., Socal, M. P., & Anderson, G. F. (2019). Using External Reference Pricing In Medicare Part D To Reduce Drug Price Differentials With Other Countries. *Health Affairs*, 38(5), 804-811.

METHODOLOGICAL APPENDIX

The data underlying the figures are sourced from both primary and secondary sources. Data for figures 3, 7, 9, 17, 18, 30, 31, 33, 34, 35, 37, and 39 are from previously reported studies.

For figures 7, 8, 14, 15, 30, and 32, we analyzed data from Truven MarketScan® commercial claims database. This database covers outpatient prescription drug claims for beneficiaries covered by employer-sponsored insurance. These claims represent million unique beneficiaries for the years 2010, 2011, 2012, 2013, 2014, 2015 and 2016 respectively. Exploratory data analysis was used to identify and exclude outliers from the analysis.

For figures 11, 12, 16, 21, 22, 23, 25, 26, we analyzed Medicare claims from the Medicare 20% sample database. We primarily used the 2013–2016 Prescription Drug

Encounters file. We restricted our sample to beneficiaries who were enrolled the full year. For formulary information, we utilized Plan characteristics files and formulary files. Exploratory data analysis was used to identify and exclude outliers from the analysis.

For figures 19 and 20, we analyzed Medicare drug spending dashboard data. This publicly available data provides Medicare and beneficiaries spending by drug. Information on drug characteristics including orphan status, biologics status were identified using the FDA website. Specialty drug designation was based on Medicare criteria notably \$670 drugcost per month.

For figure 27 and 28, we analyzed state level Medicaid drug utilization data. This data can be sourced from data.medicaid.gov.



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