PHARMACEUTICALS: INNOVATION, ACCESS, AND AFFORDABILITY CHARTBOOK

A Report from the Johns Hopkins Drug Access and Affordability Initiative
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.

**ACKNOWLEDGEMENTS**

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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 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**
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.
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

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patient safety, but they can also serve as a tool to limit market competition. 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 drugs 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.

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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.

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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)

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)

- 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.

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1 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
2 ibid
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.

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1.3 Who Pays for Pharmaceuticals in the US?

Figure 6: Retail Prescription Drug Spending by Payer in 2016

- Private Health Insurance - 43.4%
- Medicare - 29%
- Out of Pocket - 13.7%
- Medicaid - 10.2%
- Other Health Insurance Programs - 3.1%
- Other Third Party Payers - 0.6%
- Public and private insurance account for approximately the same percentage of drug spending
- 56% of Americans are privately insured and they account for 43.4% of drug spending
- Medicare and Medicaid covers 33% of Americans and they account for 39% of drug spending

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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 uncertain 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: COMMERCIALLY INSURED DRUG SPENDING (2010-2016)

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.

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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 2016

- The 10% of people responsible for the most drug spending accounted for about 25% of the fills and over 75% of spending in 2016.
- Spending is becoming more concentrated over time. In 2010, the top 10% accounted only for 60% of spending.
- A few high cost specialty drugs were responsible for most of the increased spending.

<table>
<thead>
<tr>
<th>Fills</th>
<th>Spending</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total Fills by Top 10%</td>
<td>Total Spending by Top 10%</td>
</tr>
<tr>
<td>Total Fills by Bottom 90%</td>
<td>Total Spending by Bottom 90%</td>
</tr>
</tbody>
</table>

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. Medicare Part D spent $100 billion on drugs in 2016. Medicare Part B spent $27 billion on drugs in 2016.

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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.


**PART D**

Medicare Part D, while the largest payer 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 coverage 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.

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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

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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.\textsuperscript{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.

\textbf{FIGURE 12: AVERAGE PART B SPENDING FOR SPECIFIC CATEGORIES OF DISEASES IN 2016}\textsuperscript{18}

![Graph showing average Part B spending for specific categories of diseases in 2016.](image)

\textbf{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 rebates 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.


\textsuperscript{18} Analysis of Medicare’s 20% restricted sample. See Methodology section for further details.
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.

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1.4 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)**

- While most patients pay a small amount out of pocket for pharmaceuticals, there are patients with significant pharmaceutical bills
- Patients with commercial insurance in the 99th percentile of drug spending incur an average of $1840 in out of pocket cost per year.

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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)

- 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.

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 16: Cost-sharing in Medicare Part D (2016)

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

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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 provide 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 2005-2015

![Graph showing patient assistance program spending from 2005 to 2015.](image)

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 biological 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)](image)

- The ten drugs with the highest total sales represented $64.3 billion in 2016 or 14.3% of gross drug spending
- Four of the top ten drugs are biologics
- Four of the top ten drugs are specialty drugs
- Six of the top ten drugs have orphan designations

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

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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.

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%.

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**TABLE 1: TOP TEN DRUGS BY MEDICARE PART D SPENDING IN 2016**

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

**TABLE 2: TOP TEN DRUGS IN MEDICARE PART B BY SPENDING IN 2016**

<table>
<thead>
<tr>
<th>DRUG</th>
<th>Total Spending</th>
<th>Spending Per User</th>
<th>Average Provider Margin (ASP+6%)</th>
<th>Condition</th>
</tr>
</thead>
<tbody>
<tr>
<td>Injection, aflibercept, 1 mg</td>
<td>$2,207,882,153</td>
<td>$10,497</td>
<td>$630</td>
<td>Age-related Macular Degeneration</td>
</tr>
<tr>
<td>Injection, rituximab, 100 mg</td>
<td>$1,663,513,650</td>
<td>$23,812</td>
<td>$1,429</td>
<td>Rheumatoid Arthritis</td>
</tr>
<tr>
<td>Injection, pegfilgrastim, 6 mg</td>
<td>$1,374,291,300</td>
<td>$14,330</td>
<td>$860</td>
<td>Cancer</td>
</tr>
<tr>
<td>Injection, infliximab, 10 mg</td>
<td>$1,336,041,105</td>
<td>$22,896</td>
<td>$1,374</td>
<td>Rheumatoid Arthritis</td>
</tr>
<tr>
<td>Injection, nivolumab, 1 mg</td>
<td>$1,219,517,891</td>
<td>$44,082</td>
<td>$2,645</td>
<td>Cancer</td>
</tr>
<tr>
<td>Injection, bevacizumab, 10 mg</td>
<td>$1,110,360,974</td>
<td>$5,357</td>
<td>$321</td>
<td>Cancer</td>
</tr>
<tr>
<td>Injection, denosumab, 1 mg</td>
<td>$1,085,135,202</td>
<td>$2,592</td>
<td>$156</td>
<td>Osteoporosis</td>
</tr>
<tr>
<td>Injection, ranibizumab, 0.1 mg</td>
<td>$1,044,105,659</td>
<td>$9,814</td>
<td>$589</td>
<td>Age-related Macular Degeneration</td>
</tr>
<tr>
<td>Injection, trastuzumab, 10 mg</td>
<td>$702,888,944</td>
<td>$33,990</td>
<td>$2,039</td>
<td>Cancer</td>
</tr>
<tr>
<td>Pneumococcal vaccine for injection into muscle</td>
<td>$667,483,118</td>
<td>$170</td>
<td>$10</td>
<td>Pneumonia</td>
</tr>
</tbody>
</table>
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 2016

While most prescription fills are generics, they represent only amount to 15% Part D spending
Specialty drugs are 2% of fills but they are responsible for 31% of Part D spending

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).

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

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

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 MARKET

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.

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 negotiate 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 STATE

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 FORMULARIES**

- Across all classes, only 44% of all drugs are on formularies
- Across the 6 protected classes, 70% of drugs are on formularies
- For non-protected classes, only 37% of all drugs are on formularies

**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 resulted 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).

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

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.

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**

![Graph showing profit growth of PBM companies over time.](image-url)
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&D

- Global pharmaceutical R&D has increased significantly since 1990
- Recently R&D spending has plateaued
- Average number of new drugs has been fairly steady at about 100 per year as measured by the number of new drug applications (NDAs) and biologics license applications (BLAs)

**GENERIC 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 consolidate, 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.

![Value of Mergers and Acquisition deals in Generic industry (Billions)](image)

**FIGURE 31: GENERIC INDUSTRY CONSOLIDATION BETWEEN 1996 AND 2016**

Over past 30 years, there have been 133 significant mergers and acquisitions in the generic drug space amounting to over $651 billion in deal values.

From 1996 to 2009, most of the generic industry consolidation was on smaller deals, with the exception of 2000 which saw two of the largest acquisitions in the pharmaceutical space.

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).

Levels of competition are commonly categorized by the 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%.

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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.
From 2010 to 2016, the generic drug market in commercially insured population has become more competitive.

The number of drugs with low competition have decreased 40% from 2010 to 2016.

However, 47% of active compounds had fewer than 3 active competitors, but these compounds only represent 3.6% of generic drugs fills.

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**FIGURE 32: LEVELS OF COMPETITION ACROSS GENERIC DRUGS IN 2010 AND 2016**

- Low Competition
- Medium Competition
- High Competition

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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.
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. 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

![Perception on Drug Prices](image)

- Public (2018): Cost of drugs is unreasonable - 80%
- Economists (2017): Cost of drugs is unreasonable - 45%

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Economists believe that prices are too high when people and governments cannot afford pharmaceuticals.

Economists generally do not believe that research and development spending is a justification for high drug prices.

### The Price of Many Drugs in the US is unfair because:

- Low Income People Cannot Afford These Drugs
- Forcing Public Insurers to Increase Drug Spending
- People Can’t Afford These Drugs
- Increases Premium for Those Without the Diseases
- Companies Maximize Profits
- Companies Spend <20% on R&D
- Price of Replacement Drug is Lower

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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.\(^4^2\) This was estimated 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.\(^4^3\) 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%.


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. 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).

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

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

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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

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


CONCLUSION

This chartbook provides policymakers and the public insights into the many facets of the 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


Truven Marketscan® Commercial Database (Truven Health Analytics, Ann Arbor, MI, USA)


CMS 2007 and 2017 Trustees Report

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


Analysis of Orphan Drug data from the Food and Drug Administration


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.