



BIOPHARMACEUTICALS IN PERSPECTIVE

FALL 2020

PhARMA
RESEARCH • PROGRESS • HOPE

CHART PACK



ENSURING A BETTER FUTURE FOR HER CHILD

Emily H.

Emily is a mom from Montana who feels strongly about making her voice heard: “I decided to be part of Voters for Cures because my son, Cooper, has a disorder called autonomic dysfunction. What that means is that the things we don’t think about that our bodies just do on their own, his doesn’t. He has a really hard time sweating and regulating blood flow to vital parts of his body. I am really hoping that by becoming a part of Voters for Cures that our leaders at all levels of government will hear my family’s story and start to take into consideration how policy changes affect us and millions of other families like us.”

Caring for a kid with a serious illness isn’t easy. “It took us a long time to get answers for him—9 years. And it took going across the country to a specialty clinic.” Now Emily is raising her voice to ensure that Cooper and kids across the country have access to the medicines and new innovation they need. “Developing new innovative treatments and medicines is crucial to ensuring a future for our children. Our nation must explore every avenue to developing new cures for debilitating and life-threatening disease, and there is no better time to innovate than the present. As we look to the future, I want to make sure policymakers in Washington enact policy that allows innovation to flourish. Some proposals in DC threaten this progress and for that, I’m concerned.”

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INTRODUCTION



This chart pack provides facts and figures about prescription medicines and their role in the health care system. Topics include medicines' impact on health and quality of life, the drug discovery and development process, health care spending and costs, the challenges of addressing treatment gaps and improving the use of prescribed therapies, the contributions of the biopharmaceutical sector, and costs and access in other developed countries.

Data and information in this publication were drawn from a wide range of sources, including government agency reports, peer-reviewed journals, and the Pharmaceutical Research and Manufacturers of America's (PhRMA's) own research and analysis. PhRMA hopes this publication provides useful context for discussions regarding the role of medicines and the US economy.



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ADVANCES IN TREATMENT

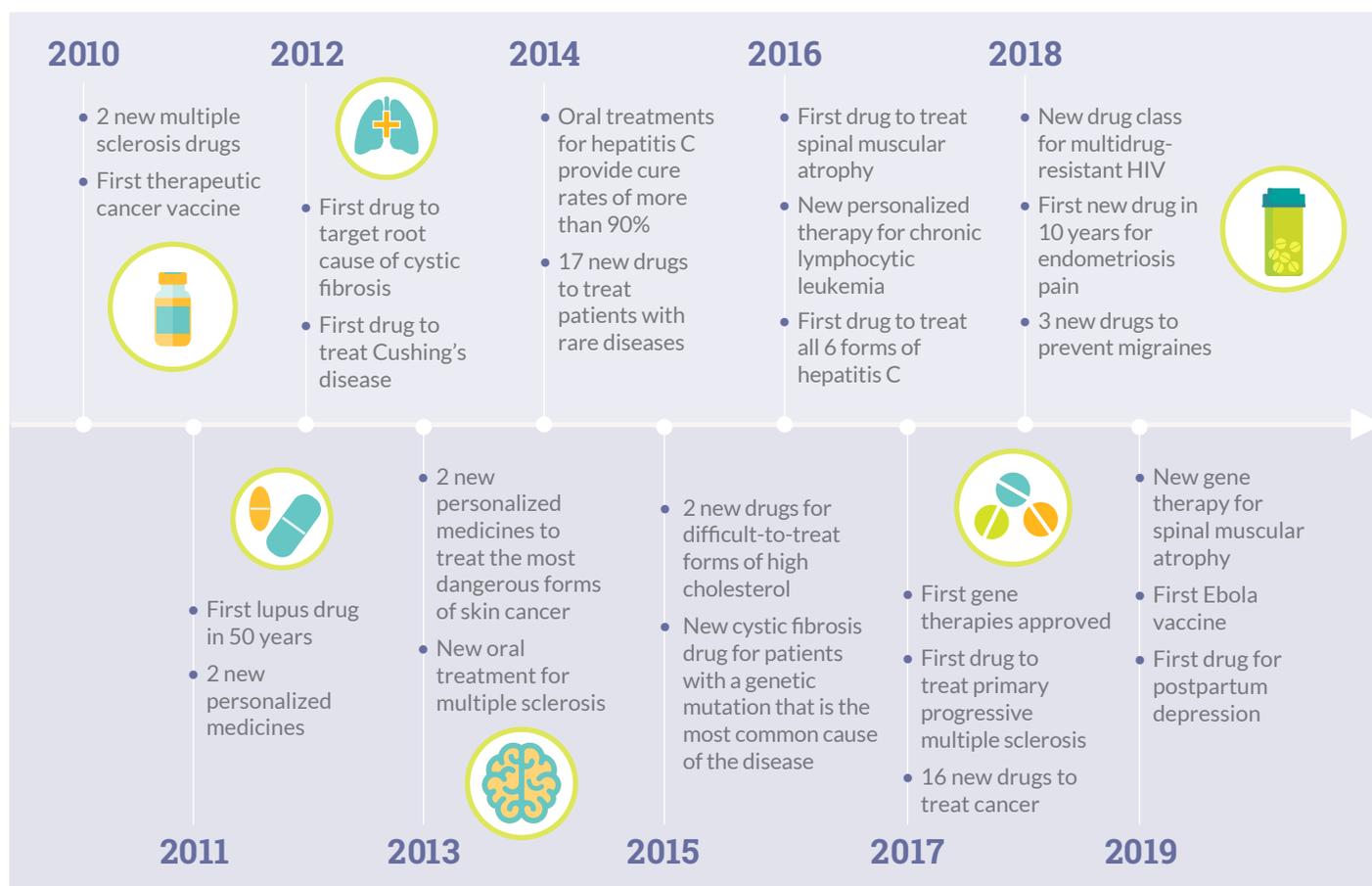
Medicines' Impact on
Health and Quality of Life



Prescription medicines have yielded important advances, helping patients live longer and healthier lives. Over the past 25 years, prescription medicines have transformed the trajectory of many debilitating diseases and conditions, including heart disease, HIV/AIDS, cancer, and hepatitis C, resulting in decreased death rates, improved health outcomes, and better quality of life for patients.

Today, new drugs are targeting the underlying causes of disease in ways never seen before, and diseases previously regarded as deadly are now manageable and even curable. In this new era of medicine, breakthrough science and personalized therapies are revolutionizing the way we treat patients with a broad range of chronic and rare conditions. Looking forward, continued advances in biopharmaceutical innovation will be critical in addressing unmet needs, improving public health, and solving future health care challenges.

A Decade of Advances



Source: FDA¹

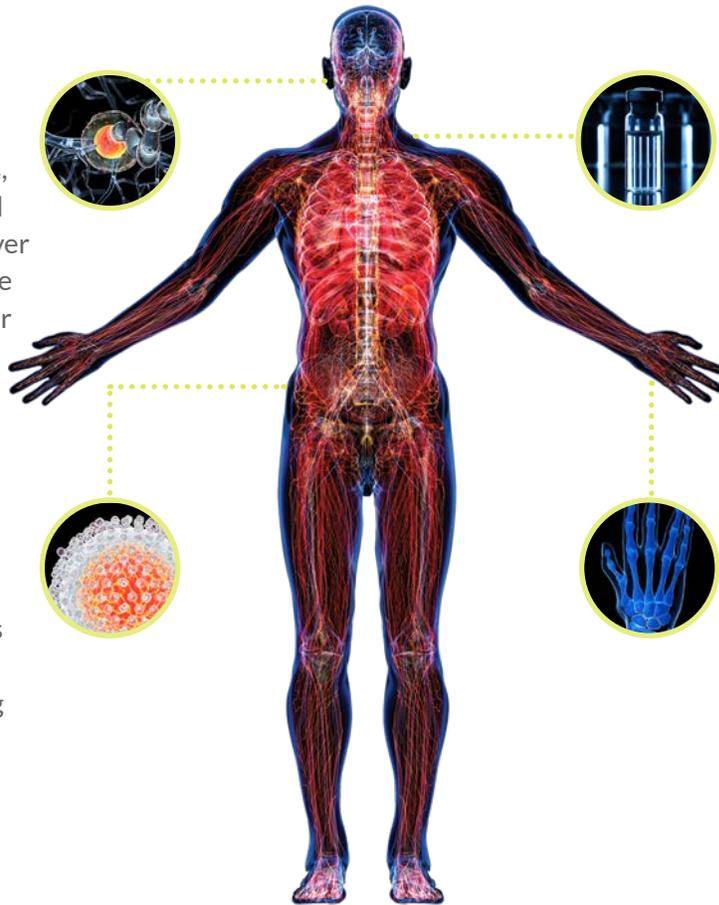
Medicines Are Transforming the Treatment of Many Diseases

Multiple Sclerosis (MS)

Advances in recent years, including convenient oral medicines and the first-ever treatment for progressive MS, offer patients greater opportunity to better manage MS and slow disease progression.²

Hepatitis C

Recent therapeutic advances can cure the disease and help patients avoid serious disease complications—including cirrhosis, advanced liver disease, liver cancer, and death.³



Cancer

New therapies have contributed to a 29% decline in cancer death rates since they peaked in 1991—translating to 2.9 million deaths avoided.⁴

Rheumatoid Arthritis (RA)

Biologic and oral disease-modifying agents have transformed the RA treatment paradigm, shifting from a focus on managing symptoms to aiming for slowed disease progression and even remission.^{5,6}

Sources: PhRMA^{2,3}; Siegel RL et al⁴; American College of Rheumatology⁵; Boston Healthcare Associates⁶

Medicines Are Transforming the Treatment of Many Rare Diseases

Collectively, rare diseases affect 30 million Americans. Treatments are available for only 5% of rare diseases, but recent advances are providing important new options to many patients for the first time.⁷

Spinal Muscular Atrophy (SMA)⁸

SMA involves loss of motor neurons leading to progressive muscle weakness and wasting and, in severe cases, respiratory failure. The first gene therapy was approved in 2019 to treat children less than 2 years of age with the most common form of SMA and the leading genetic cause of infant mortality.

Acquired Thrombotic Thrombocytopenic Purpura (aTTP)⁹

aTTP is a life-threatening blood disorder affecting about 3 adults per million annually. The disease leads to disturbances in the nervous system and organs as a result of clots that form in the smallest of arteries. The first treatment for aTTP was approved in 2019.



Tenosynovial Giant Cell Tumor¹⁰

This condition involves non-cancerous tumors around the joint areas which can cause painful damage to surrounding tissue and structures of the body. If left untreated, these tumors can lead to severe disability. The first treatment was approved in 2019 for patients who are not candidates for surgery.

Erythropoietic Protoporphyrin (EPP)¹¹

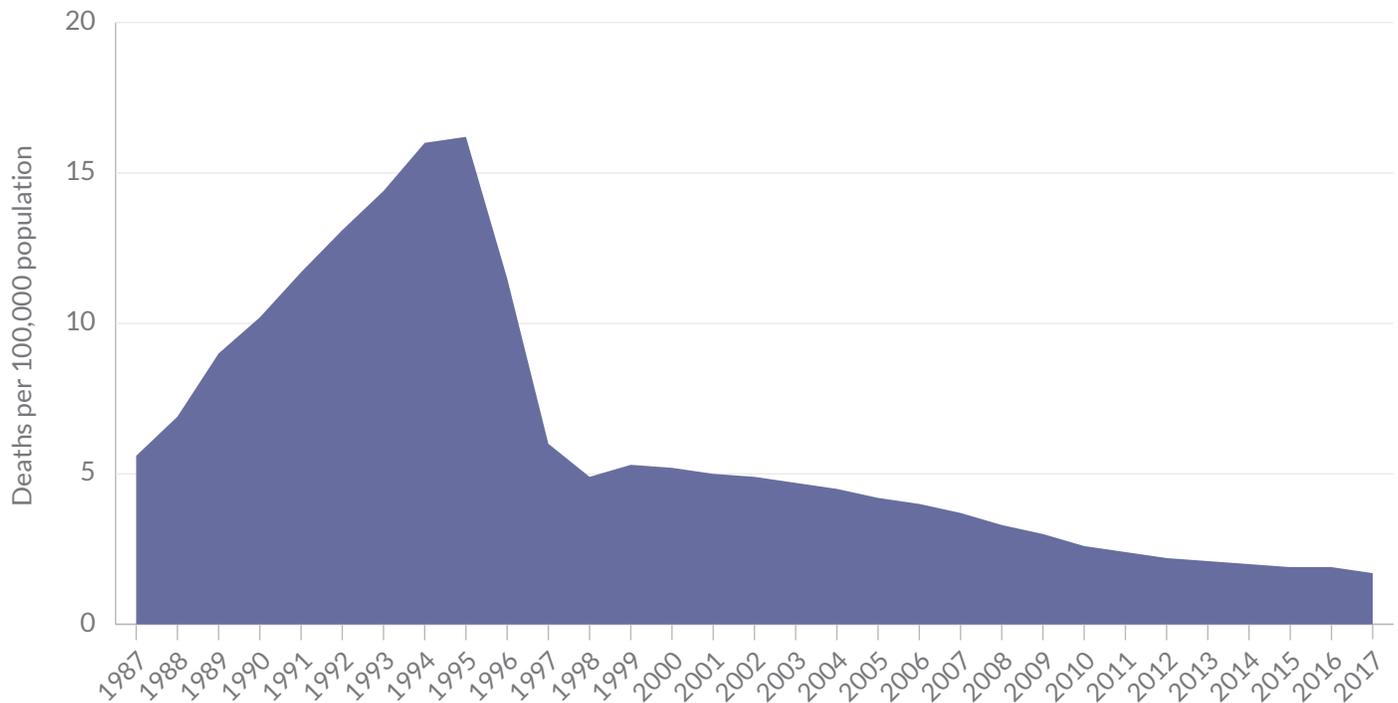
EPP is a disorder affecting about 4,000 US children and adults. The most common symptom is severe pain upon exposure to artificial and natural light which can lead to burns and ulcers on the skin. The first treatment to increase pain-free light exposure for EPP patients was approved in 2019.

Sources: Global Genes⁷; FDA⁸⁻¹¹

AIDS Mortality in the United States

The number of AIDS deaths in the United States decreased dramatically following the introduction of highly active antiretroviral therapy (HAART) combinations in 1996. As a result of HAART and all the important medical innovations that followed, it is estimated that more than 862,000 premature deaths have been avoided in the United States alone.¹²

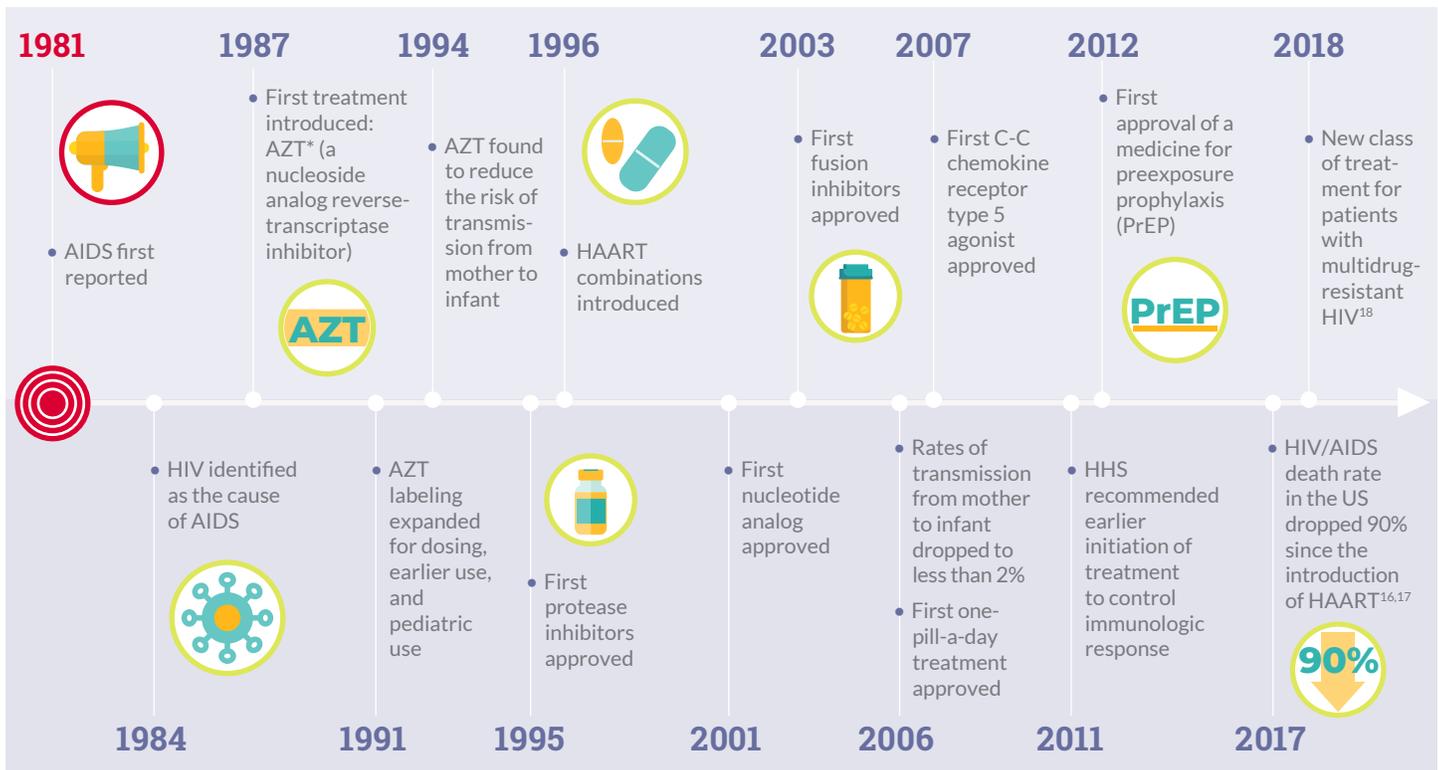
Annual Number of AIDS Deaths in the United States^{13,14}



Sources: Truven Health Analytics¹²; CDC^{13,14}

HIV/AIDS: Treatment Advances Build Over Time

Dramatic declines in death rates did not occur with one single breakthrough but rather through a series of advances providing important treatment options for patients over time.¹⁵

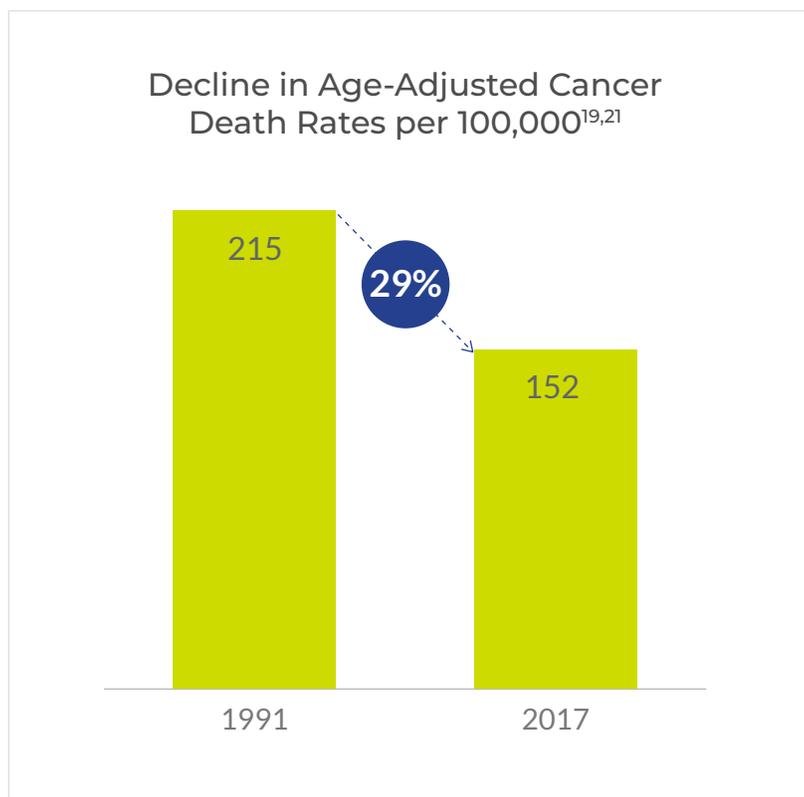


*AZT: Azidothymidine

Sources: Boston Healthcare Associates¹⁵; CDC^{16,17}; FDA¹⁸

Cancers: Decline in Death Rates

Since peaking in the 1990s, cancer death rates have declined 29%.¹⁹ Approximately 73% of survival gains in cancer are attributable to new treatments, including medicines.²⁰ New medicines have contributed to a 2.2% decline in death rates in the most recently recorded year, the largest single-year drop ever reported.¹⁹



The **ACCELERATED DROPS** in lung cancer mortality as well as in melanoma that we're seeing are likely due at least in part to **ADVANCES IN CANCER TREATMENT** over the past decade, such as immunotherapy [emphasis added]. . . . They are a profound reminder of how rapidly this area of research is expanding, and now leading to real hope for cancer patients.”

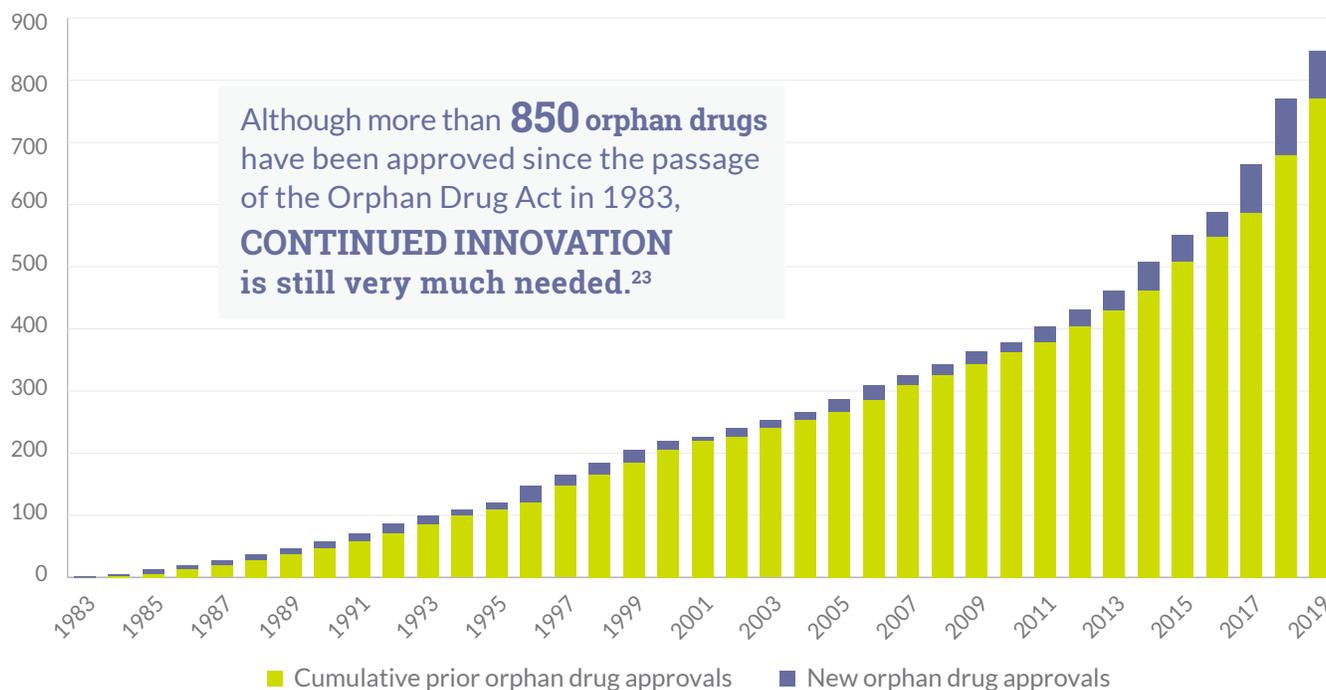
William G. Cance, MD,
Chief Medical and Scientific Officer,
American Cancer Society²²

Sources: Siegel RL et al¹⁹; Seabury SA et al²⁰; National Cancer Institute²¹; American Cancer Society²²

Rare Diseases: Drug Approvals Meet Unmet Needs

Rare diseases are those that affect 200,000 or fewer people in the United States.²³

Number of Drug Approvals for Rare Diseases^{24*}



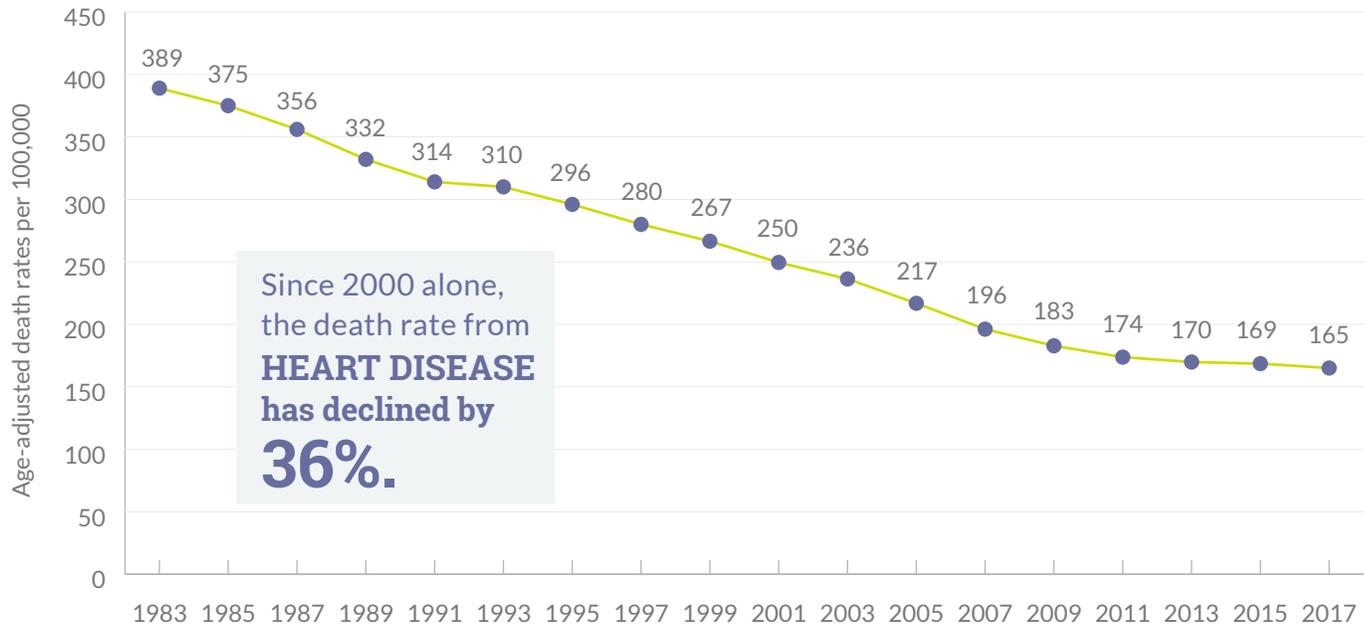
*Drug approvals for rare diseases include initial approvals of new medicines and approvals for new indications of existing medicines.

Sources: NIH²³; FDA²⁴

Cardiovascular Disease: Declining Rates of Death

Tremendous strides have been made in reducing cardiovascular disease morbidity and mortality, thanks in part to new medicines.

US Death Rates Due to Diseases of the Heart*



*Age-adjusted death rates based on year 2000 US standard population. 1980-1998 causes of death are classified by the International Classification of Diseases, Ninth Revision (ICD-9). Beginning in 1999, causes of death have been classified by the International Statistical Classification of Diseases and Related Health Problems, Tenth Revision (ICD-10).

Sources: CDC^{25,26}

Medicines Are Improving Patients' Quality of Life

Relative to medical technology available a decade ago, new treatments for complex chronic conditions are better tolerated, more efficacious, and more convenient, thereby improving not only life expectancy, but quality of life for patients.

Ten-Year Decline in Number of Patients With Complex Chronic Conditions* Experiencing Quality-of-Life Impairment†



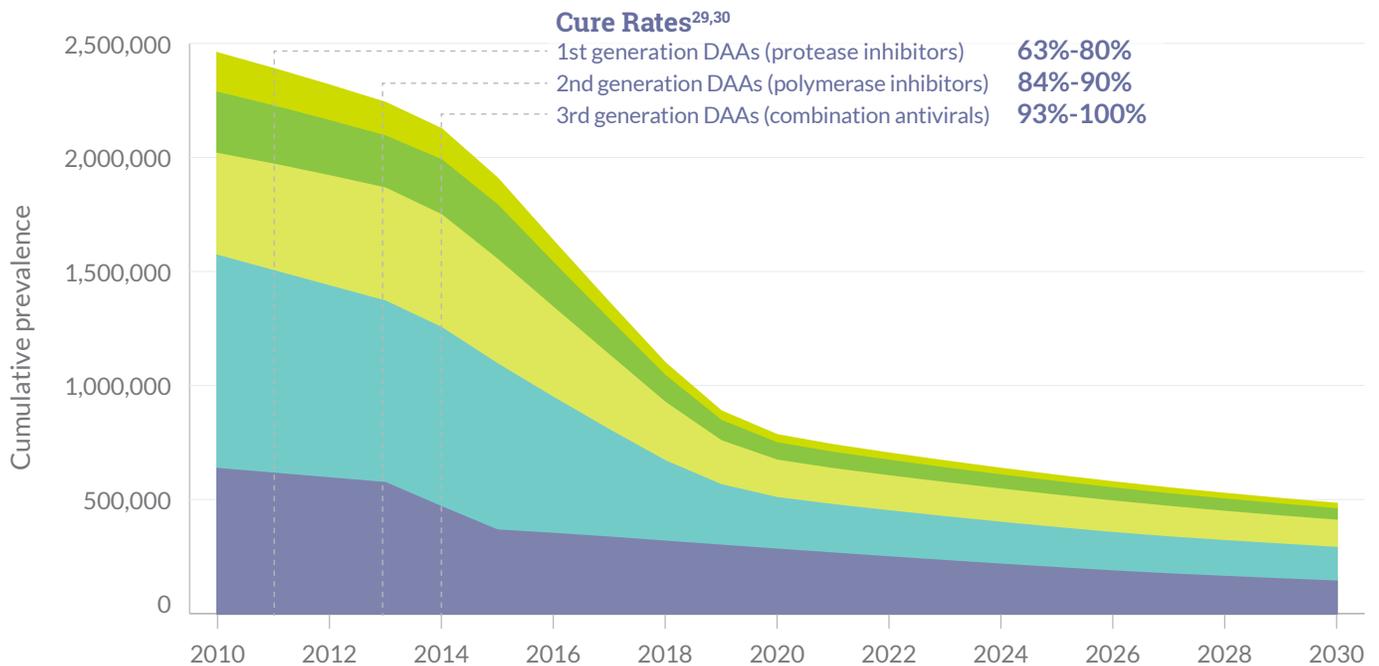
*HIV, rheumatoid arthritis, leukemias, non-Hodgkin's lymphoma, multiple sclerosis, and lupus

†Chart reflects unweighted estimates reported in study.

Hepatitis C: Advances Driving Down Prevalence of Disease

The introduction of direct-acting antivirals (DAAs) and subsequent improvements in cure rates revolutionized the treatment of hepatitis C (HCV), significantly driving down prevalence of disease.

Decreasing Number of HCV-Infected Patients, by Insurance Type, US 2010-2030^{28*}



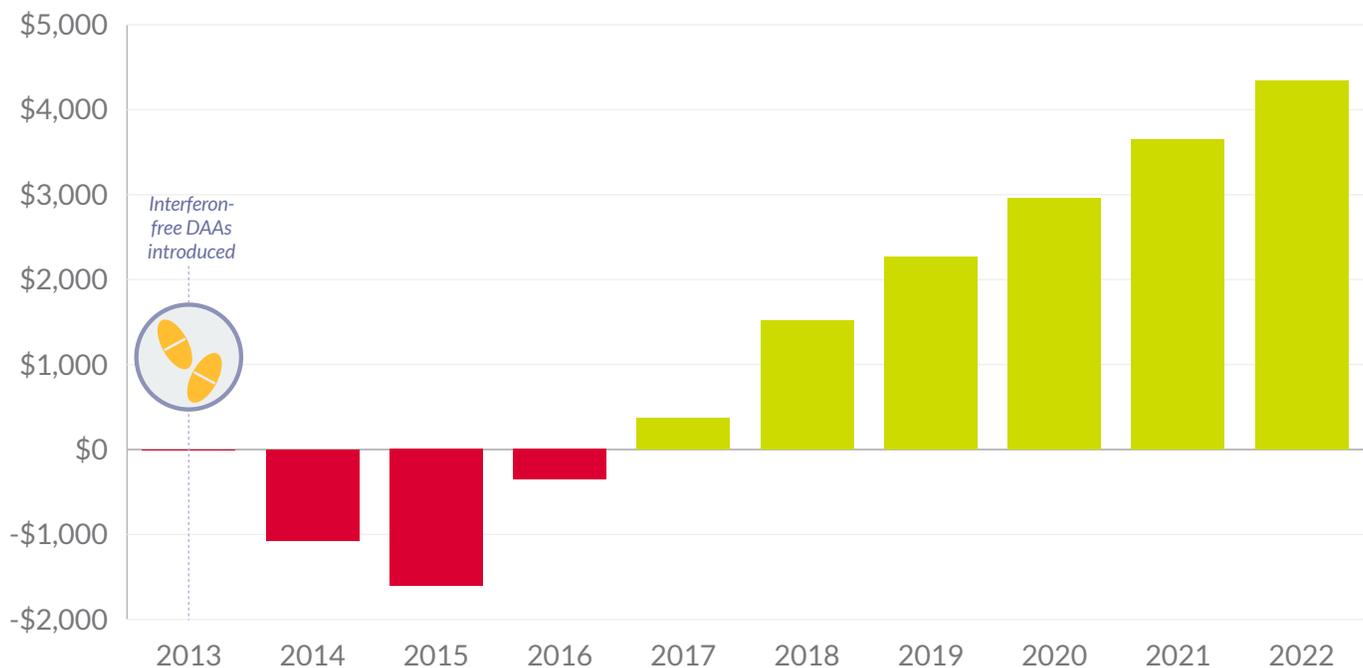
*Model takes into account launch of DAAs, change in HCV screening policies, and implementation of the Affordable Care Act.

Sources: Chhatwal J et al²⁸; PhRMA²⁹; FDA³⁰

Hepatitis C Medicines Produce Savings in Medicaid

By 2019, the total cumulative costs of HCV medicines since the introduction of curative interferon-free DAA therapy were fully offset by the total cumulative savings in health care costs resulting from avoided disease complications in Medicaid. By 2020, Medicaid realized an estimated \$12 billion in cumulative savings.

HCV DAA Therapy: Short-term Costs and Long-term Savings in Medicaid, 2013-2022 (in Millions)

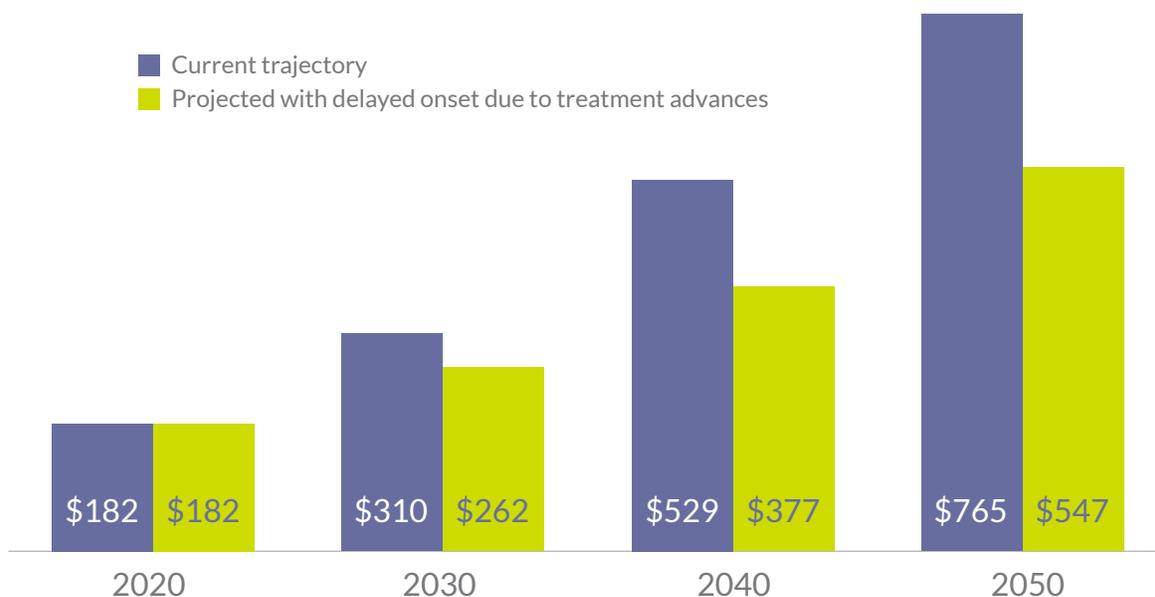


Source: Roebuck MC et al³¹

Unmet Need: Future Impact of New Treatments for Alzheimer's Disease

The development of a new treatment that delays the onset of Alzheimer's disease could reduce Medicare and Medicaid spending on patients by \$218 billion annually by 2050.*

Projected Annual Medicare and Medicaid Spending With and Without New Treatment Advances (in Billions)[†]



*Assumes research advances that delay the average age of onset of Alzheimer's disease by 5 years beginning in 2025.

[†]Projected savings to Medicare and Medicaid assume research breakthroughs that slow the progression of Alzheimer's disease. This would dramatically reduce spending for comorbid conditions and expensive nursing home care.

Source: Alzheimer's Association³²

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RESEARCH AND DEVELOPMENT

The Process of Drug
Discovery and Development



Ensuring continued advances in treatments will be indispensable in addressing society's health and economic challenges in the years ahead. We are in a new era of medicine where breakthrough science is bringing tremendous hope to patients. The pipeline for new medicines has never been more promising, with more than 8,000 medicines in development around the world and more than half of PhRMA members researching and developing potential treatments and vaccines for COVID-19. Over the past decade, PhRMA member companies have invested more than half a trillion dollars in biopharmaceutical research and development (R&D), accounting for the majority of private biopharmaceutical R&D spending. Development of new medicines is a long and rigorous process, with many setbacks along the way. As scientific complexities create new challenges in R&D, biopharmaceutical companies are working to create efficiencies and enter new collaborations across the biomedical research ecosystem.

More than 8,000 Medicines in Development Globally¹

Biopharmaceutical researchers are pursuing many innovative scientific approaches that are driving therapeutic advances.



ALS (Amyotrophic Lateral Sclerosis)

Stem cell therapies aim to replace and/or protect damaged motor neurons and slow disease progression.



ALZHEIMER'S DISEASE

CRISPR-Cas9 genetic screening is being utilized to look for changes in amyloid beta production, a believed cause of the disease. Other approaches include immunotherapies, vaccines, or antibodies that target abnormal tau proteins, which may cause cognitive impairment.



CANCER

Allogeneic, or "off the shelf," cell therapy involves the personalized modification of immune-boosting cells, from healthy donors, and infusing them into a cancer patient to target and kill cancer cells. Some of these cell therapies are being designed to be delivered in the outpatient setting.



HEMOPHILIA

Adeno-associated viral (AAV) vector-mediated gene therapies enable patients to clot blood and can reduce the need for chronic treatment to prevent bleeding episodes.

Source: PhRMA analysis of Adis R&D Insight database¹

About 4,500 Medicines in Development in the United States

Biopharmaceutical researchers are working on new medicines* for many diseases and on select prevention and treatment approaches.



VACCINES
260



PEDIATRIC DISEASES
560



DIABETES
160



SICKLE CELL DISEASE
20



MENTAL ILLNESS
138



CELL & GENE THERAPIES
362



ASTHMA & ALLERGY
130



NEUROLOGICAL DISORDERS
537

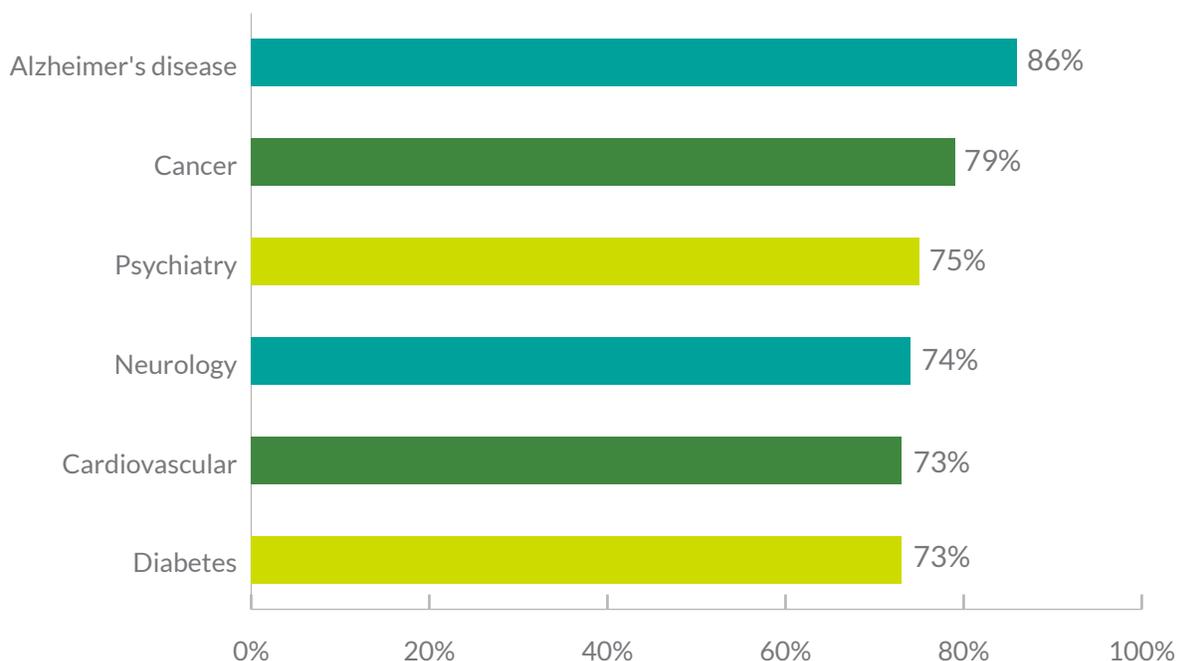
*Defined as single products that are counted only once regardless of the number of indications pursued

Source: PhRMA analysis of Adis R&D Insight database²

Potential First-in-Class Medicines in the Pipeline

An average of 74% of drugs in the clinical pipeline are potential first-in-class medicines.

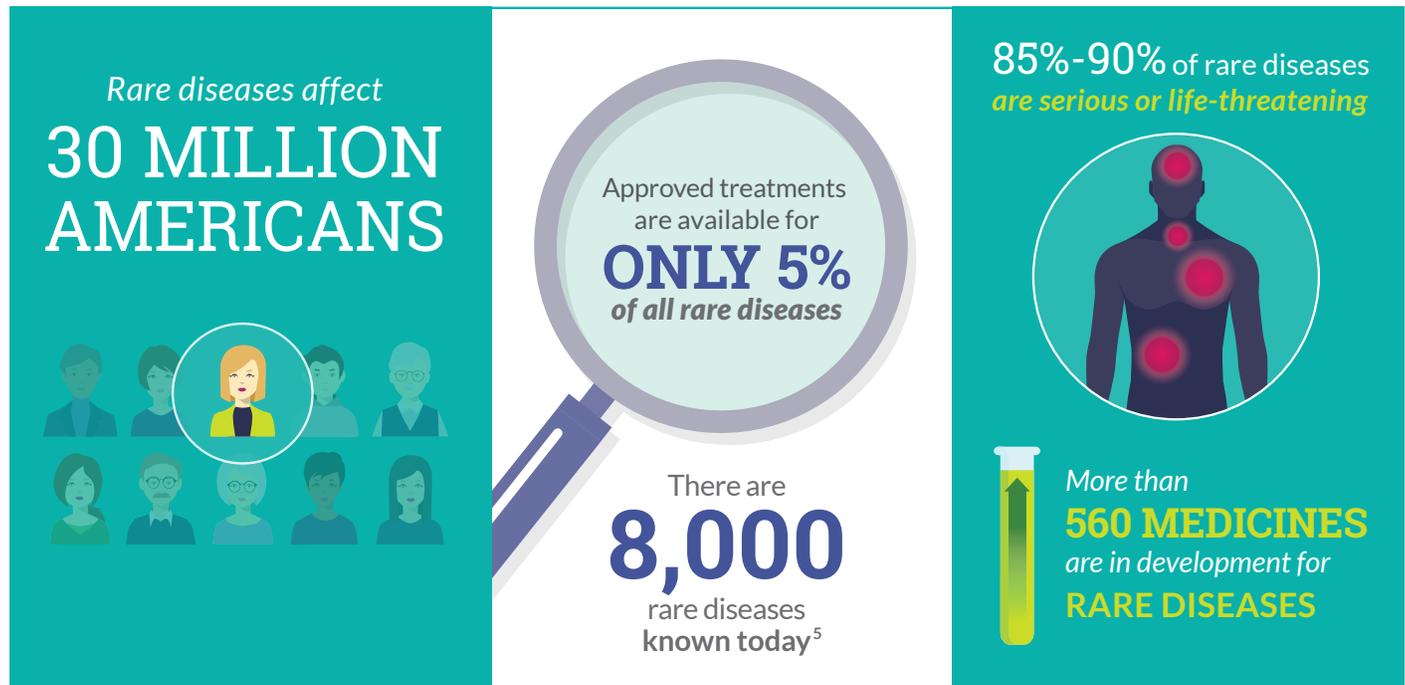
Percentage of Products in Clinical Development and Regulatory Review That Are Potentially First-in-Class, Selected Therapeutic Areas, 2016



Source: Analysis Group³

Harnessing Innovation in Rare Diseases

Since the passage of the Orphan Drug Act in 1983, we have seen tremendous advances in treatments for rare diseases,* with more than 850 orphan drug approvals (compared with fewer than 10 in the decade before passage).⁴



*Rare diseases are defined as conditions for which there are fewer than 200,000 patients diagnosed in the United States.

Sources: FDA⁴; Danese E et al⁵; PhRMA⁶

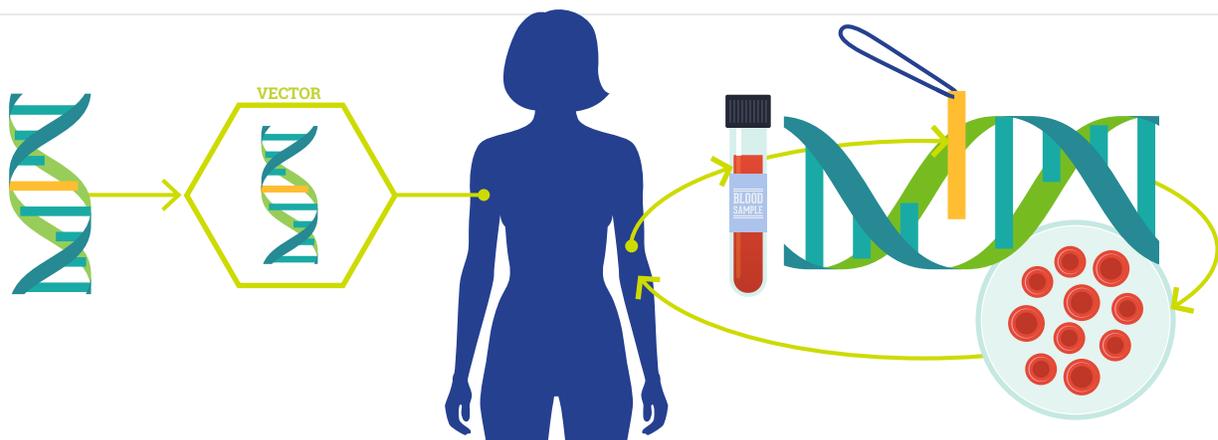
Cell and Gene Therapies Are Revolutionizing the Treatment of Many Diseases

Cell and gene therapies belong to an emerging field of personalized medicine that helps our bodies fight the root causes of diseases at the cellular and genetic levels.



These therapies, once only conceptual, are rapidly becoming a therapeutic reality for an increasing number of patients with a wide range of diseases, including rare genetic disorders and autoimmune diseases.”

Stephen M. Hahn, MD, FDA Commissioner⁷



In Vivo Gene Therapy

Sometimes a gene therapy is administered directly into the patient, where inside the body a vector brings the corrected, silenced, or replacement DNA to the cells.

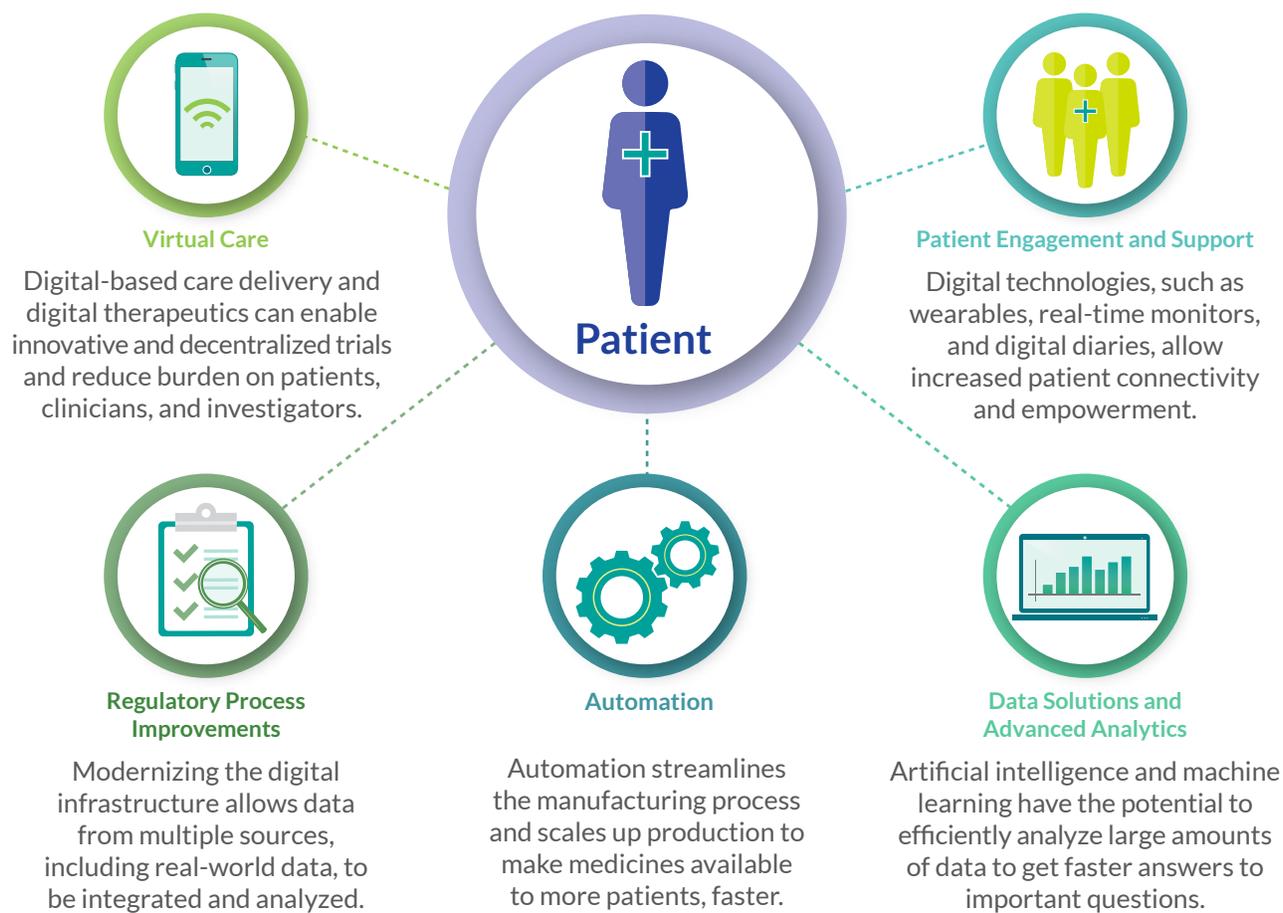
Ex Vivo Gene Therapy and CAR T-Cell Therapy

Sometimes cells are taken from the patient, modified outside of the body, multiplied in a lab, and then returned to the patient.

Sources: FDA⁷; PhRMA⁸

Digital Technologies May Enhance Drug Development and Improve Patient Care

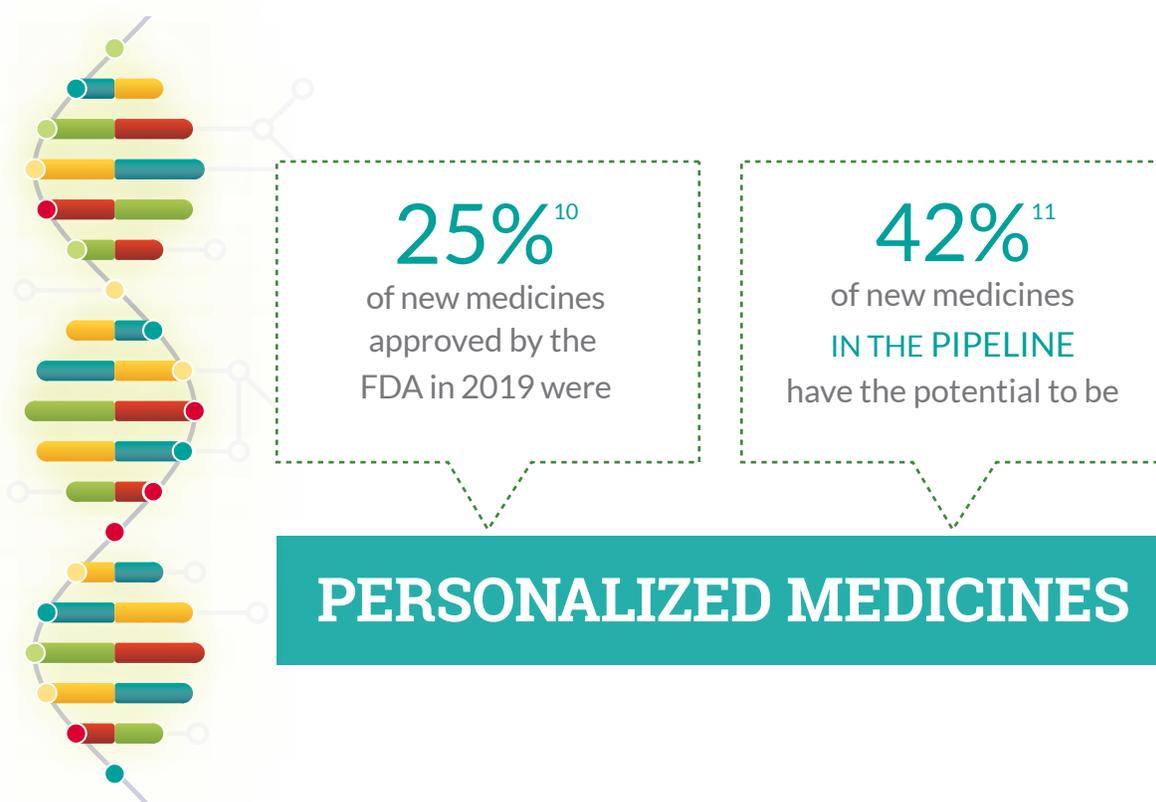
Technological advances are increasing access to clinical trials, accelerating biopharmaceutical R&D of new treatments, enabling efficient information exchange, enhancing clinical decision-making, generating information that is important to patients, and furthering patient engagement and safety.



Source: Adapted from Avalere Health⁹

Biopharmaceutical Companies Are Committed to Advancing Personalized Medicine

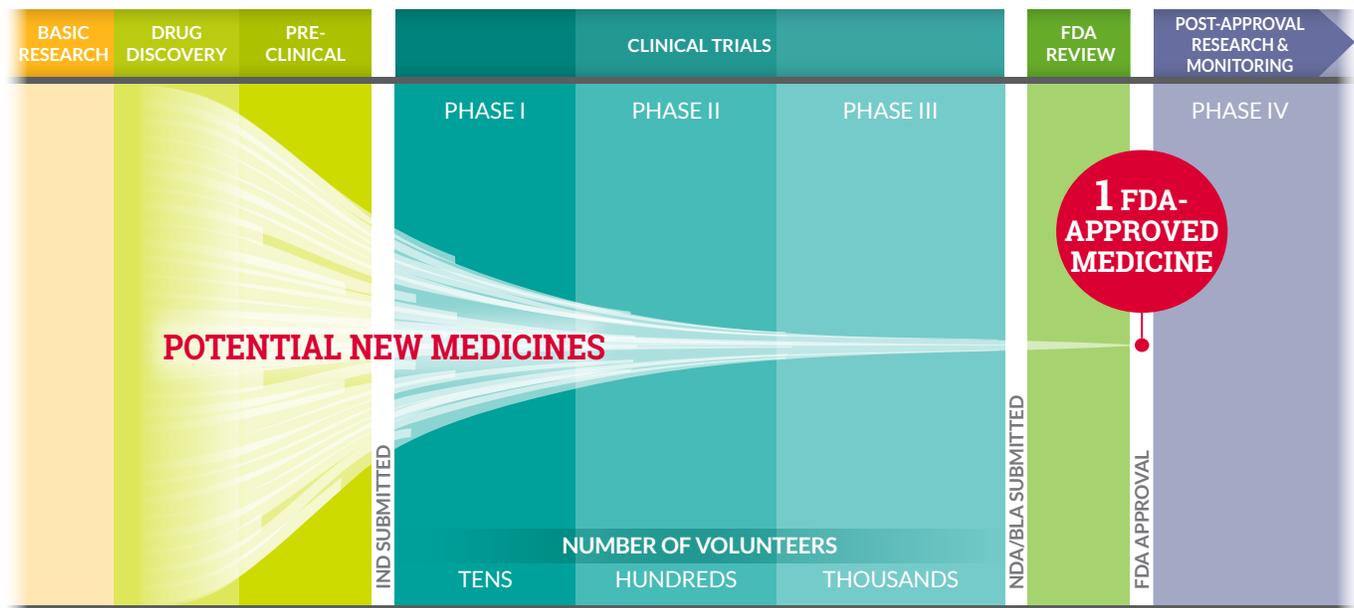
The transformation of health care from a one-size-fits-all approach to a targeted one that employs each patient's own genetic information is advancing.



Sources: Personalized Medicine Coalition¹⁰; Tufts CSDD¹¹

The R&D Process for New Drugs Is Lengthy and Costly, With High Risk of Failure

From drug discovery through FDA approval, developing a new medicine takes, on average, 10 to 15 years and costs \$2.6 billion.* Less than 12% of the candidate medicines that make it into Phase I clinical trials are approved by the FDA.

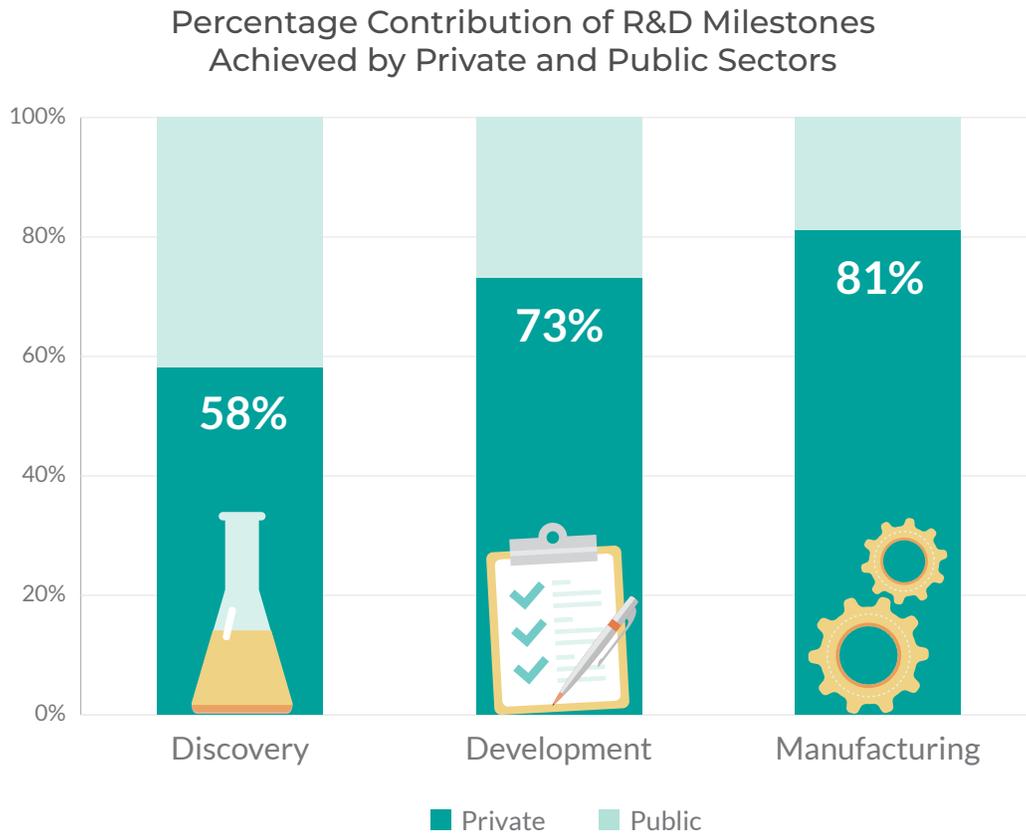


Key: IND=Investigational new drug application, NDA=New drug application, BLA=Biologics license application

*The average R&D cost required to bring a new FDA-approved medicine to patients is estimated to be \$2.6 billion over the past decade (in 2013 dollars), including the cost of the many potential medicines that do not make it through to FDA approval.

Sources: PhRMA adaptation of DiMasi JA et al¹²; Tufts CSDD¹³; FDA¹⁴

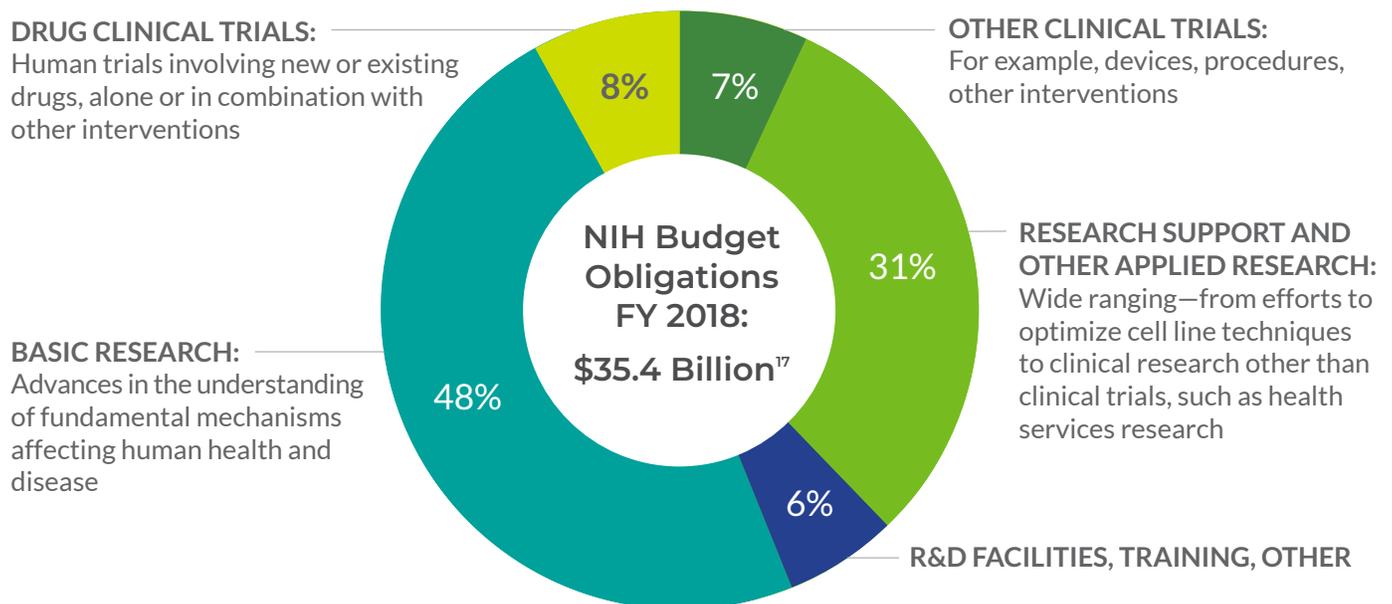
Private Sector Scientific and Industrial Expertise Is Required to Develop and Manufacture New Medicines



Source: Chakravarthy R et al¹⁵

Biopharmaceutical Industry Does the Majority of Research to Advance Basic Science Into New Medicines

An estimated 8% (\$2.9 billion) of the 2018 NIH budget supported clinical trials involving new or existing drugs, alone or in combination with other interventions. In comparison, the US biopharmaceutical industry invested \$102 billion in R&D in 2018.^{16*}

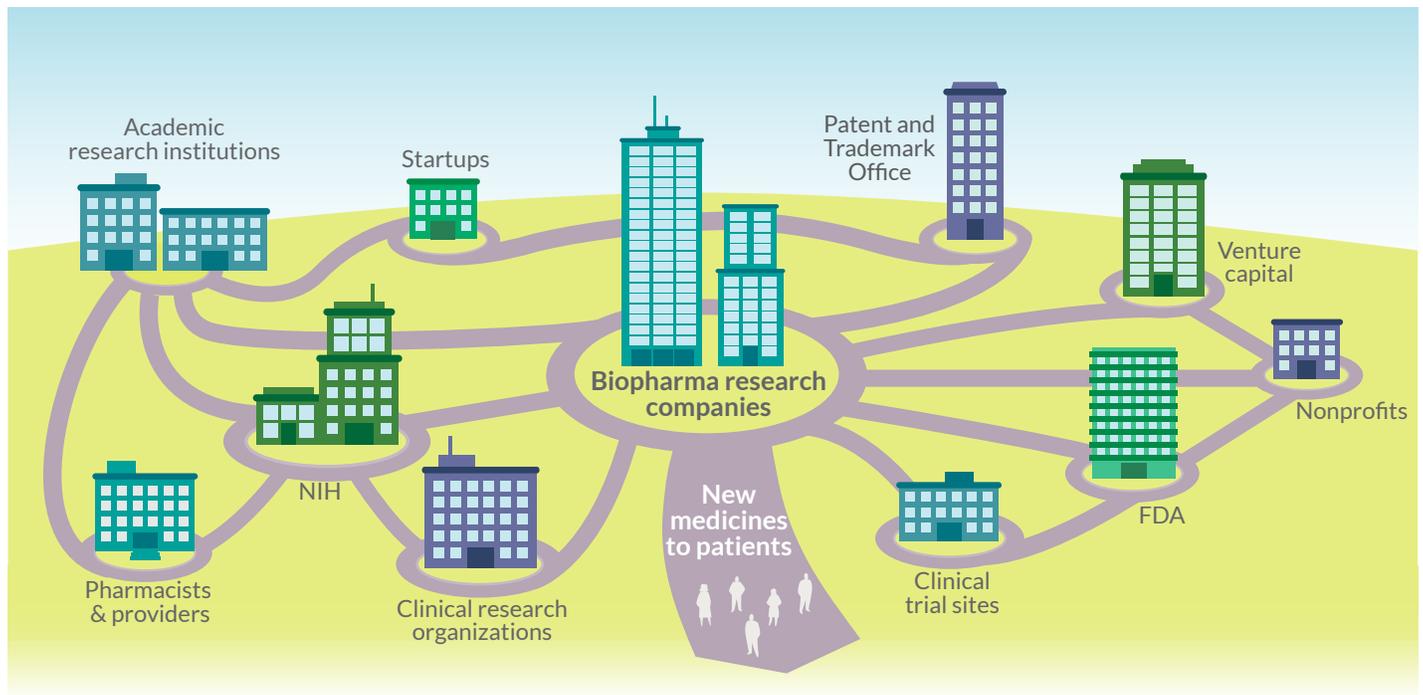


*NIH budget refers to fiscal year 2018. Biopharmaceutical investment refers to 2018 calendar year.

Sources: Research!America¹⁶; Analysis Group¹⁷

Innovative Biopharmaceutical Companies Sit at the Heart of a Dynamic R&D Ecosystem in the United States

The vibrant US biomedical R&D ecosystem is critical in bringing new medicines to patients and maintaining US leadership in biopharmaceutical R&D.



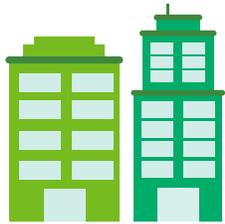
Technology Transfer Between Universities and Industry Has Resulted in Economic Growth and Continued Innovation

The Bayh-Dole Act (1980) created a uniform framework for the sharing of technology between universities and the private sector that facilitates timely and effective commercialization of federally funded research.

POSITIVE IMPACT OF BAYH-DOLE ACROSS ALL INDUSTRIES

Commercialization of federally funded research has increased dramatically.

In 2018:



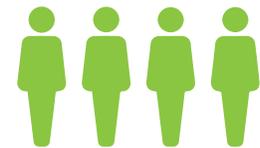
MORE THAN 1,000
startup companies
were formed¹⁸



NEARLY 830
commercial products
stemming from university
research were introduced¹⁸



Close to
\$865 BILLION
contributed to US GDP¹⁹



ABOUT 5.9 MILLION
US jobs supported
across all industries¹⁹

Sources: Association of University Technology Managers¹⁸; Pressman L et al¹⁹

Collaboration Is Key in Researching and Developing New Medicines

The rapid pace of scientific and technological advances is propelling a new era in biopharmaceutical innovation in the United States. As the science becomes more complex, partnerships are crucial to advancing biomedical progress. Below are examples of key collaborative efforts across the R&D spectrum.



Collaboration through precompetitive disease-specific data sharing and defining disease-specific biological pathways for diagnostics and treatments in Alzheimer's disease, type 2 diabetes, rheumatoid arthritis, Parkinson's disease, and lupus²⁰



A global nonprofit partnership dedicated to advancing antibacterial research to tackle the global rising threat of drug-resistant bacteria by accelerating preclinical candidates toward clinical development²¹



Bolstering research and data sharing to improve prevention and treatment for opioid misuse, accelerate treatments, and enhance pain management by developing new biomarkers²²



ACTIV—coordinated research strategy for prioritizing and speeding development of COVID-19 treatments and vaccines²³
Operation Warp Speed—aimed at accelerating development, manufacturing, and distribution of COVID-19 countermeasures²⁴



THE PARTNERS

biopharmaceutical companies, NIH, FDA, patient and disease organizations

academia, charitable foundations, NIH, global partners

biopharmaceutical companies, NIH, FNIH, patient and disease organizations

NIH, FDA, CDC, BARDA, other government agencies, biopharmaceutical companies

Sources: NIH²⁰; CARB-X²¹; NIH^{22,23}; HHS²⁴

Collaboration Is Key in Manufacturing and Delivering New Medicines

Industry is coming together with diverse stakeholders to solve the challenging complexities of manufacturing and delivery in a modernized way. Examples of innovative collaborative efforts are listed below.

<p>Drug Supply Chain Security Act Pilot Project Program²⁵</p> <p>FDA</p>	<p>NIIMBL (National Institute for Innovation in Manufacturing Biopharmaceuticals)²⁶</p> <p>NIST</p>	<p>NEW Drug Development ParadIGmS Initiative²⁷</p> <p>MIT</p>	<p>End the HIV Epidemic²⁸</p> <p>CDC</p>
<p>To assist drug supply chain stakeholders in developing the electronic, interoperable system that will identify and trace certain prescription drugs as they are distributed within the United States</p>	<p>Collaborating on innovative manufacturing technologies, reference standards, and measures that bring medicines, such as cell and gene therapies, to market efficiently, while maintaining safety and efficacy</p>	<p>Collaborating to develop solutions for systemwide barriers to biomedical innovation and patient care, from trial designs to innovative financing methods, by driving sustainable patient-centered innovation</p>	<p>Industry working with the CDC to enable state and local partnerships to develop and implement protocols intended to ensure that uninsured people at risk for HIV have access to the medicines they need</p>
 <p>COLLABORATORS</p>			
<p>FDA, biopharmaceutical companies, retail manufacturers, health care systems, academia</p>	<p>industry, academia, states, National Institute of Standards and Technology (NIST), FDA, NIH, DOD, BARDA</p>	<p>academia, biopharmaceutical companies, NIH, research organizations, patient organizations</p>	<p>CDC, biopharmaceutical industry, state and local entities</p>

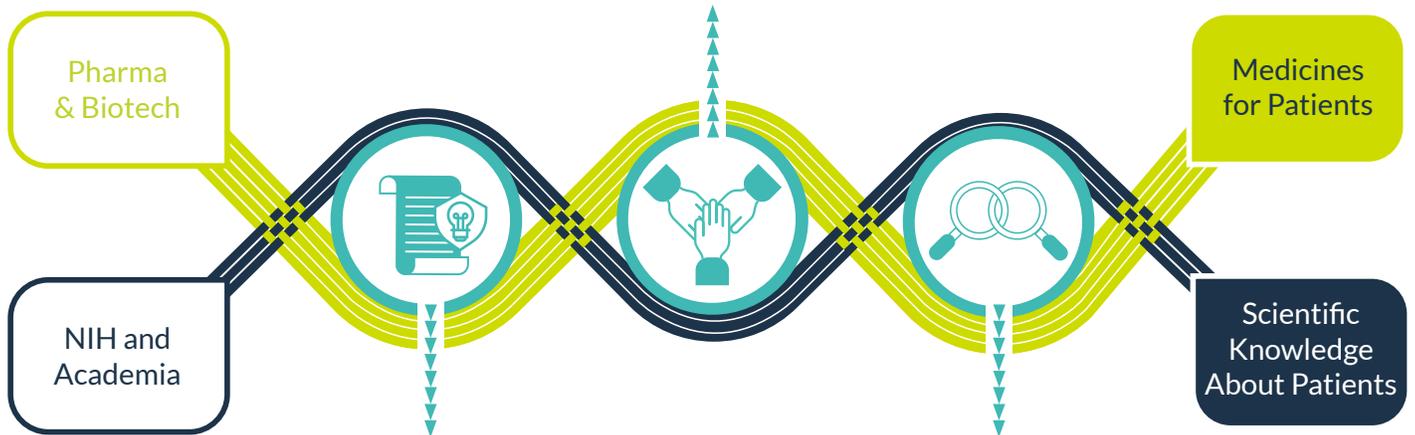
Sources: Merck²⁵; NIIMBL²⁶; Massachusetts Institute of Technology²⁷; Gilead²⁸

Public-Private Collaboration Fuels the US Biopharmaceutical Ecosystem

In recent years, stakeholders across the biopharmaceutical research ecosystem have shifted to non-asset-based, precompetitive partnership models to leverage their strengths in creative ways, create efficiencies, and tackle scientific and technological challenges.

EXCHANGE OF SCIENTIFIC KNOWLEDGE

Private industry, academic, and government scientists all work to understand the function of molecular compounds and cells or little-understood disease processes. This information is shared in peer-reviewed publications, scientific meetings, patents, and licensing of intellectual property, to be expanded upon and to fuel ideas for new medicines.



PATENTS & LICENSES

Patents allow researchers to protect and license their inventions for further development and potential commercialization, enabling the US biomedical R&D ecosystem to lead the world in biopharmaceutical progress.

RESEARCH COLLABORATION

Though industry, academic, and government scientists are encouraged to collaborate on research questions, the biopharmaceutical industry's ability to take the necessary risks is required to build on basic science research and advance it into safe and effective treatments.

Source: PhRMA²⁹

Working Together to Fight COVID-19

“Our industry will continue to work with health care partners across the world to do everything we can to beat this virus as quickly and as safely as possible. We will also make sure these potential treatments and vaccines are affordable and accessible to patients.”

PhRMA Open Letter to the Millions Affected by COVID-19³⁰

DIAGNOSTICS

It is essential to know who has been infected.

- ▶ Companies are working in partnership with governments and diagnostic companies on new and existing screening programs.

EXISTING MEDICINES

Medicines approved for other diseases may have some benefit for patients with COVID-19.

- ▶ Researchers are testing antivirals, antibiotics, and other medicines that have the potential to reduce the burden of COVID-19.

NEW TREATMENTS

Various drugs are in development, with some entering human trials.

- ▶ Researchers are working on new antiviral medications and antibody-based drugs to interfere with the way the virus infects cells or to bolster the immune system.

Sources: PhRMA^{30,31}

VACCINES

A vaccine would provide a preventive approach to beating COVID-19.

- ▶ A variety of biopharmaceutical companies are taking different approaches to finding a vaccine. More “shots on goal” will significantly increase the chances of success.

MANUFACTURING

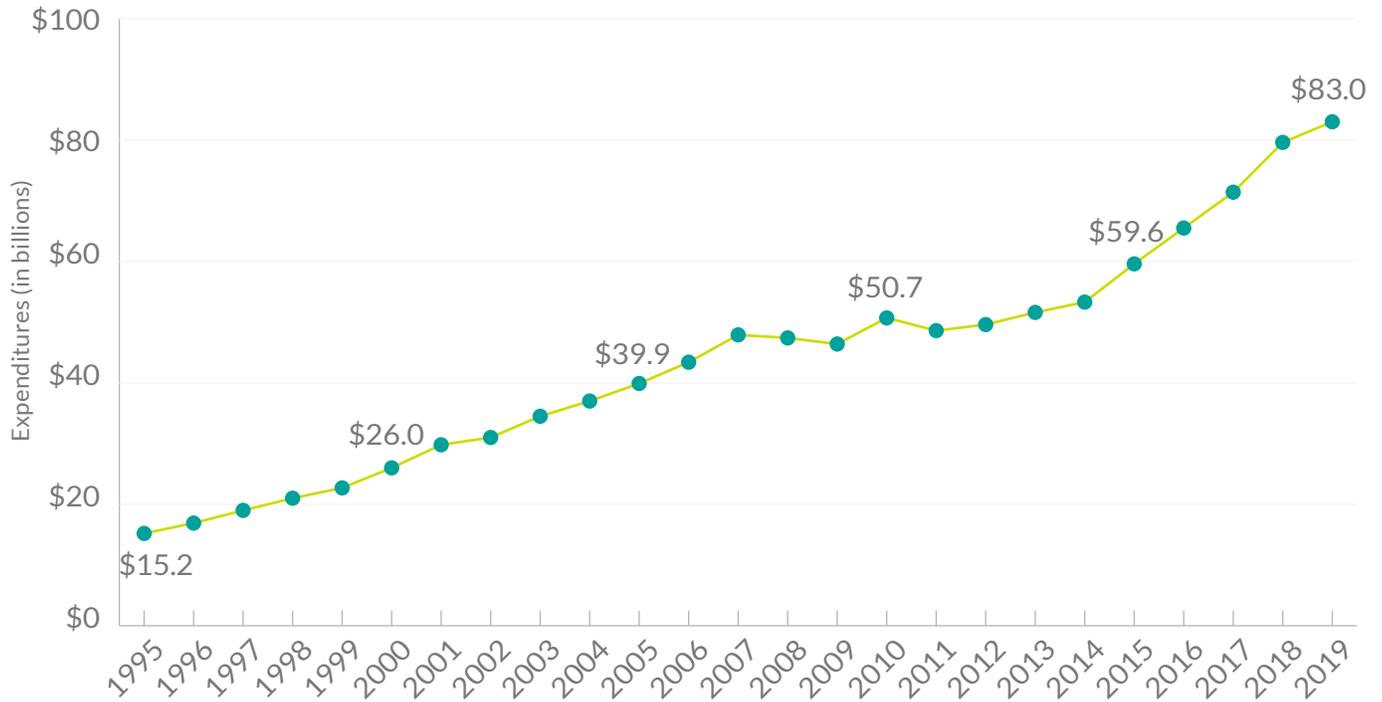
Biopharmaceutical companies are committed to manufacturing these medicines and making them available to those who need them.

- ▶ Biopharmaceutical companies are planning and building manufacturing capacity without assurance medicine and vaccine candidates will ultimately be successful, to ensure that if one is, distribution can occur rapidly.
- ▶ America’s biopharmaceutical companies are ensuring that solutions can be made available quickly to everyone who needs them.



PhRMA Member Company R&D Investment

PhRMA Member Company R&D Expenditures, 1995-2019



Source: PhRMA³²

The Costs of Drug Development Have More Than Doubled Over the Last 30 Years

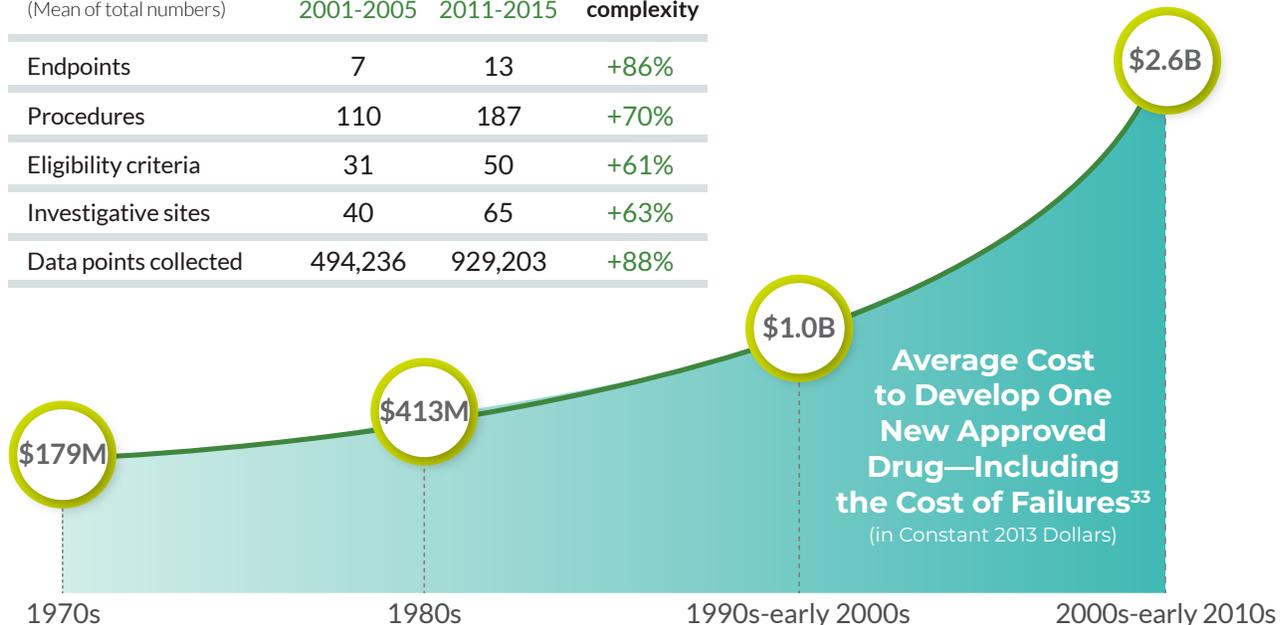
Many factors are driving up the costs of biopharmaceutical R&D, including increased clinical trial complexity, larger clinical trial sizes, more data sources to integrate, greater focus on targeting chronic and degenerative diseases, and higher failure rates for drugs tested in earlier-phase clinical studies.³³



A growing number of pharmaceutical and biotechnology companies . . . have taken steps to optimize their protocol designs in order to improve feasibility, ease site and subject participation burden, . . . and gather more meaningful clinical data.”

Ken Getz, MBA, Tufts Center for the Study of Drug Development, and Rafael Campo, Medidata Solutions³⁴

Typical Phase III protocol ³⁵ (Mean of total numbers)	2001-2005	2011-2015	Increase in complexity
Endpoints	7	13	+86%
Procedures	110	187	+70%
Eligibility criteria	31	50	+61%
Investigative sites	40	65	+63%
Data points collected	494,236	929,203	+88%



Sources: DiMasi JA et al³³; Getz KA et al^{34,35}

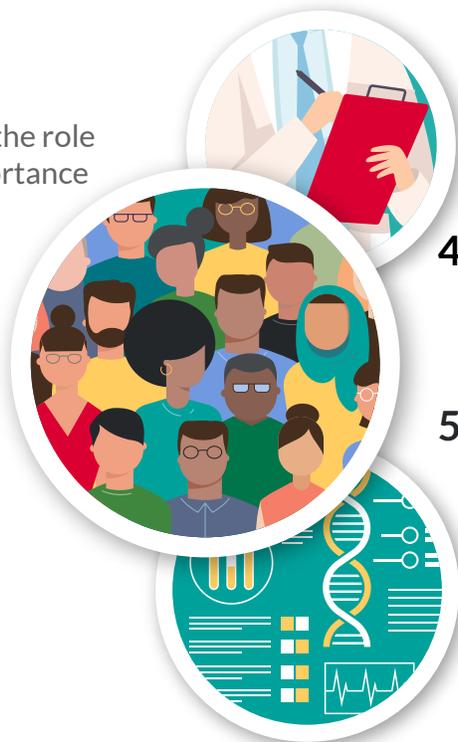
The Biopharmaceutical Industry Is Committed to Increasing Diversity in Clinical Trials

Achieving health equity starts with increased diversity in clinical trials.

The biopharmaceutical industry has long been committed to diversity in clinical trials to help ensure that the trial population is representative of the patients who will use the medicine and ensure that the results are generalizable.

Industry is committed to:

- 1 | Enhancing education about the role of clinical trials and the importance of diverse participation
- 2 | Increasing clinical trial awareness and considering ways to lower participation burden, particularly in diverse communities
- 3 | Broadening eligibility criteria, when scientifically and clinically appropriate, to increase diversity
- 4 | Leveraging real-world data to understand clinical effects in diverse patient populations
- 5 | Adopting patient-centric approaches, including diverse participant perspectives, in designing clinical trials

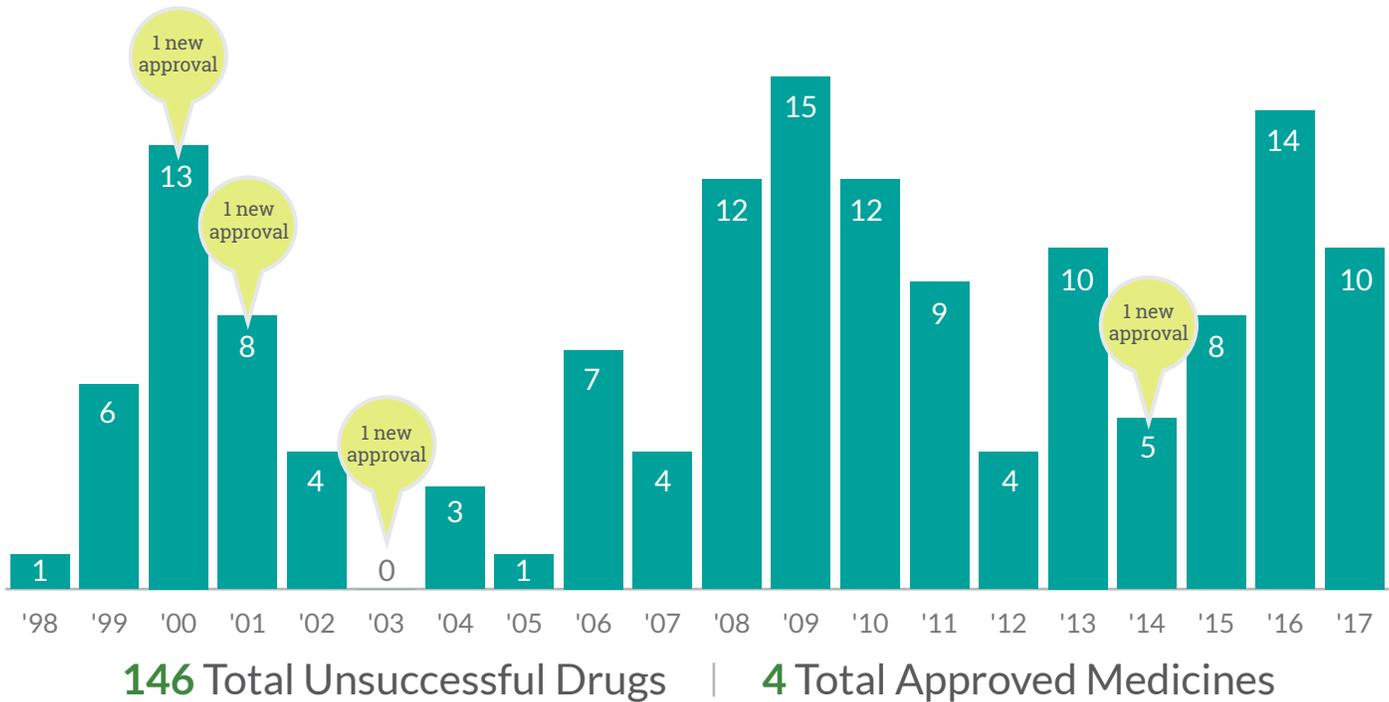


Source: PhRMA³⁶

Setbacks in Alzheimer's Disease Research Provide Stepping Stones for Future Innovation

Since 1998, 146 medicines in development for the treatment of Alzheimer's disease have not made it through clinical trials, with only 4 gaining FDA approval. These setbacks highlight the complexity of the R&D process. Though disappointing, they provide important knowledge to fuel future research.

Unsuccessful Investigational Drugs for Alzheimer's Disease, 1998-2017



Source: PhRMA³⁷

Cancer Researchers Build on Knowledge Gained From Setbacks to Inform Future Advances

The latest cancer advancements take, on average, 1.5 years longer than the development of other medicines and are built on “failures” that inform future research.



The scientific process is thoughtful, deliberate, and sometimes slow, but each advance, while helping patients, now also points toward new research questions and unexplored opportunities.”

Clifford A. Hudis, MD, FACP³⁸
Chief Executive Officer, American Society of Clinical Oncology
Chief, Breast Medicine Service, Memorial Sloan Kettering Cancer Center
Professor, Weill Cornell Medical College



MELANOMA*

158 unsuccessful attempts
12 new drugs



BRAIN CANCER*

122 unsuccessful attempts
3 new drugs



LUNG CANCER*

268 unsuccessful attempts
32 new drugs

*Setbacks and advances from 1998 to 2019

Sources: Patel JD et al³⁸; PhRMA³⁹

Pediatric Clinical Research: Overcoming Challenges

The Best Pharmaceuticals for Children Act (BPCA) and Pediatric Research Equity Act (PREA) work together to foster pediatric drug development, creating a balanced approach that generates important safety and efficacy information on the use of medicines in children. This enables biopharmaceutical companies to make significant investments in pediatric drug research.



BPCA/PREA Success

Since 1998,
nearly 770 pediatric labeling changes⁴⁰

Since 2002,
more than 250 drugs have been
granted exclusivity under BPCA⁴¹

Since 2007,
more than 680 pediatric studies
have been completed^{42,43}

Before 1997

>80%

of medicines used to
treat children did not
have pediatric dosing
information⁴⁴



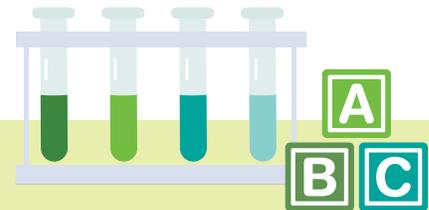
By 2012

That percentage had
been reduced to nearly

50%⁴⁵ ↓

Unique Challenges in PEDIATRIC RESEARCH⁴⁶

- Small patient populations
- Distinct dosage and formulation requirements
- Unique ethical, scientific, and medical considerations
- Difficult to enroll patients in trials



Sources: FDA⁴⁰⁻⁴⁵; ACS⁴⁶

Ensuring New Treatments Meet the Needs of Patients

As important stakeholders in the drug development process, patients, family members, caregivers, and health care providers can offer unique and valuable perspectives on the disease, daily struggles, and available treatment options. These perspectives can inform evaluation of a medicine's benefits and risks, inform areas where new treatments are needed, and inform how best to communicate this information.

WHO CAN PROVIDE PATIENT PERSPECTIVES?



Patients



Health Care Providers



Family Members and Other Caregivers

BENEFITS OF PATIENT PERSPECTIVES

RESEARCHERS:

facilitate enrollment in clinical trials, minimize patient burden in clinical trial participation, and increase the amount of patient-reported information collected and submitted to the FDA.

NEW MEDICINES:

reflect patient preferences and inform benefit and risk decisions for the use of new medicines.

THE FDA:

collects and utilizes robust and meaningful patient and caregiver input to inform drug development and support regulatory decision-making.

Source: FDA⁴⁷

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3

MARKET DYNAMICS

The Economics of Medicine
Development and the Market Forces
That Shape Spending on Medicines



Competition is a hallmark of the US prescription medicines market. Negotiating power is concentrated among a few pharmacy benefit managers (PBMs), which forces new and existing medicines to compete for coverage and increases the likelihood of excluding medicines from coverage altogether. The built-in cost containment of the prescription medicine lifecycle remains unique in health care, where new medicines eventually lead to lower-cost generics and biosimilars that bring long-term value to patients and the health care system.

Ongoing investment in research and development (R&D) depends on the commercial success of a few products that must make up for all the rest, including those that never reach the market. Average returns on R&D investments have been declining. Accounting for uncertainty and risk, biopharmaceutical profits are in the middle range among all industries.

A rapidly evolving market increasingly links payment to results, affecting how medicines are prescribed. Value- and market-based arrangements show promise for improving outcomes and reducing costs.

Illustrative Pharmaceutical Lifecycle

New pharmaceutical medicines typically face competition after a relatively short time on the market, first from brand competitors, and eventually from generics.



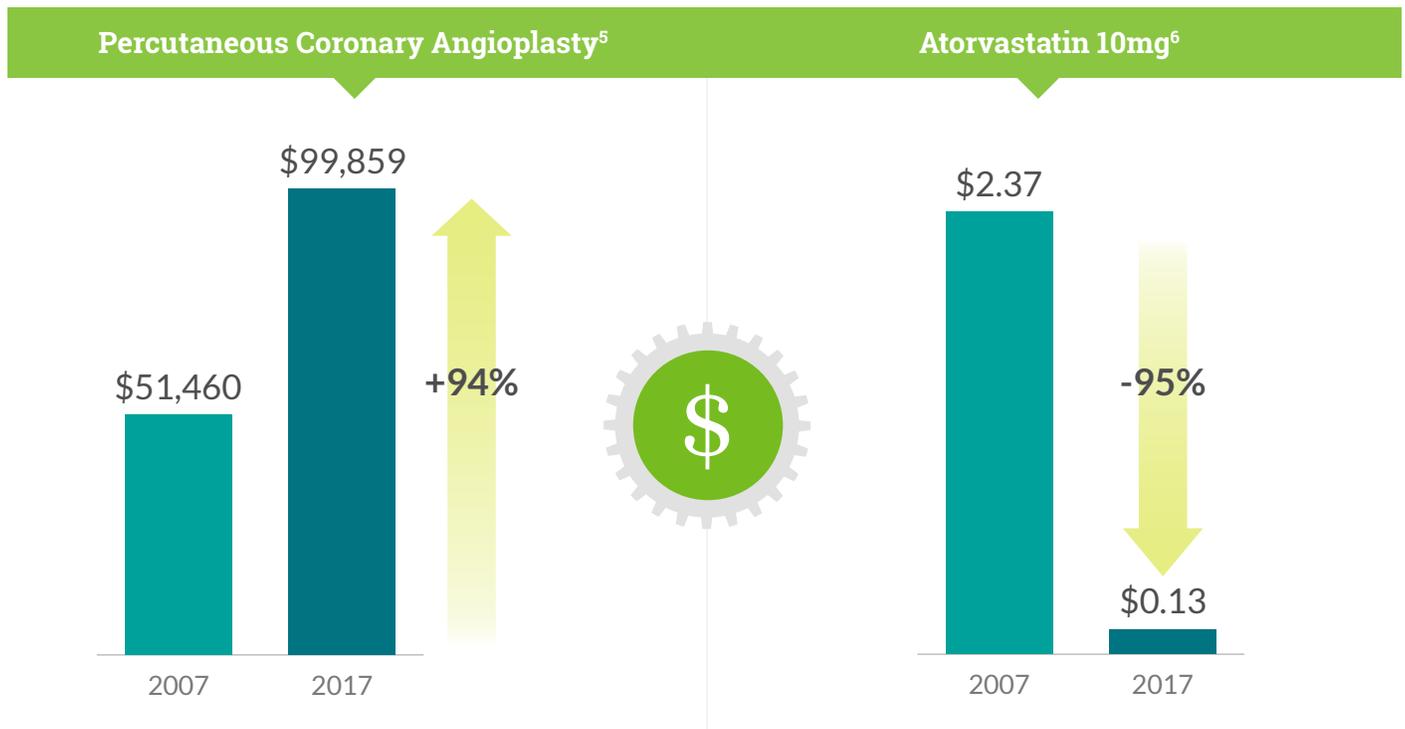
*Brand medicines limited to small molecule drugs. Brand medicine market share typically declines rapidly after generic entry.

†For brand medicines with more than \$250 million in annual sales in 2008 dollars, which account for 92% of sales of the brand medicines analyzed

Sources: PhRMA¹; DiMasi JA et al^{2,3}; Grabowski H et al⁴

Medicines Offer Built-in Cost Containment, Which Is Unique in Health Care

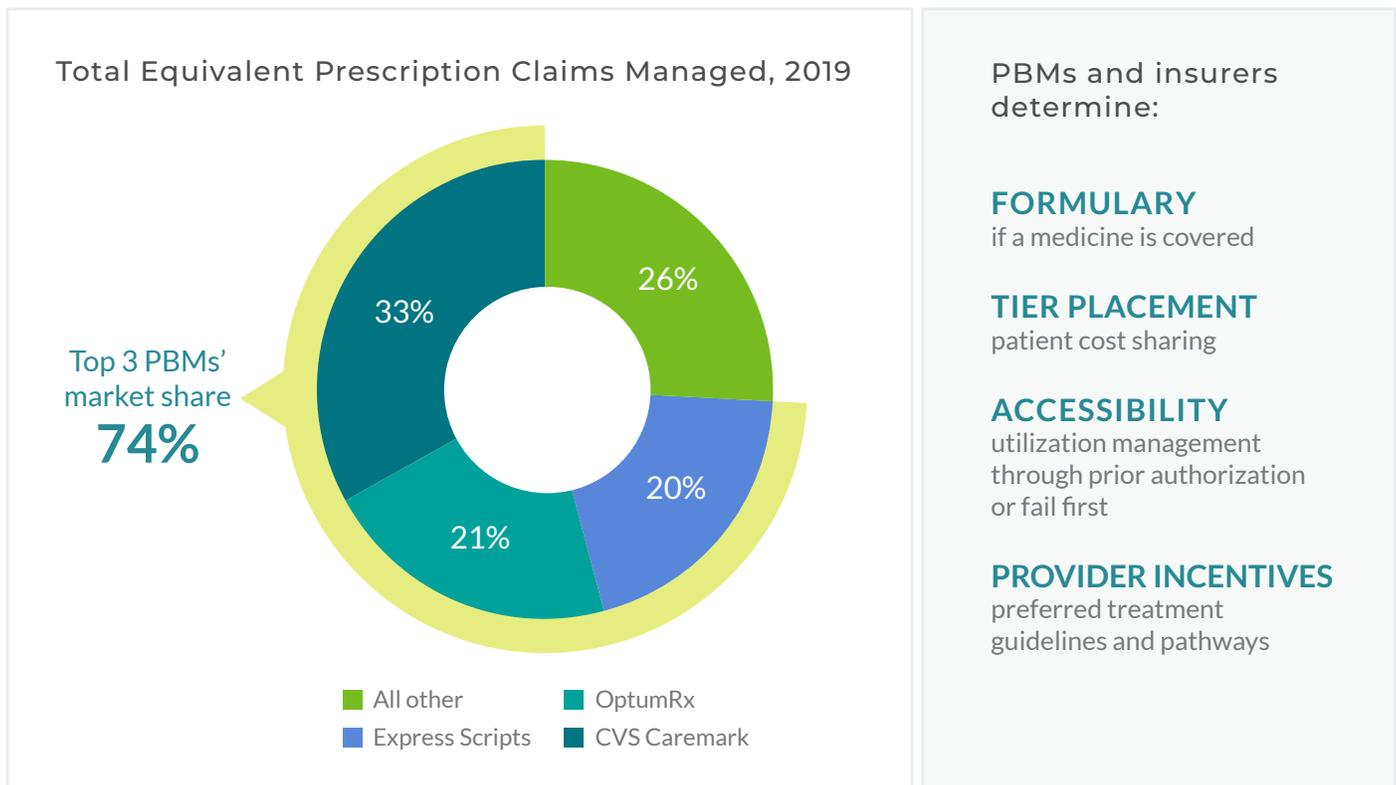
The price of a medicine commonly used to prevent cardiovascular disease dropped 95% between 2007 and 2017, while the average charge for a surgical procedure to treat it increased 94% over the same period.



Sources: Xcenda analysis of HCUP hospital charge data⁵; IQVIA⁶

Powerful Purchasers Negotiate on Behalf of Payers

Negotiating power is increasingly concentrated among fewer pharmacy benefit managers (PBMs), each purchasing medicines for more people than the populations of entire European countries.

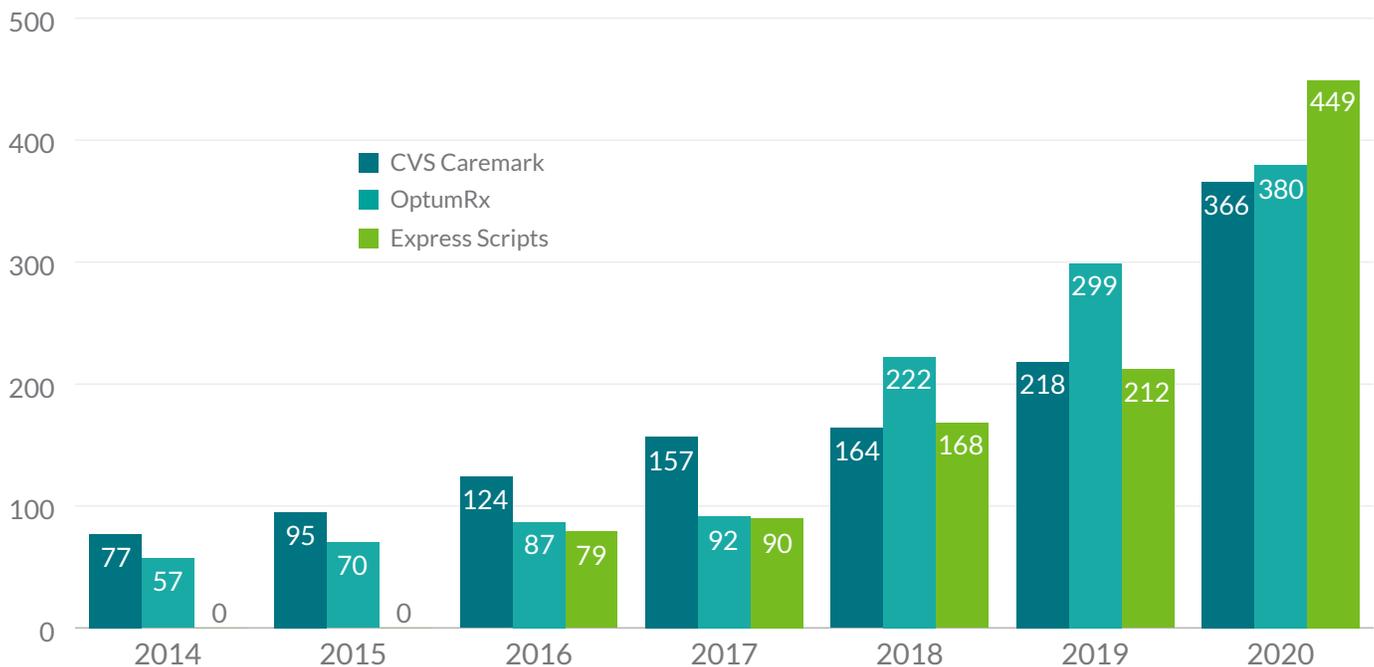


Source: Drug Channels Institute⁷

Number of Brand Medicines Excluded From PBM Formularies Has Increased Over Time

When a medicine is excluded from a pharmacy benefit manager's (PBM's) formulary, patients cannot access it through their insurance and instead pay the list price. This can interrupt the continuity of a patient's treatment as well as their doctor's ability to make prescribing decisions that best meet the patient's needs.⁸

Number of Medicines Excluded From 1 or More Formularies, by Year and PBM⁹



Sources: Tufts CSDD⁸; Xcenda⁹

Case Study in Manufacturer-Payer Negotiations: Hepatitis C Medicines

Leveraging increased competition in the hepatitis C market, payers negotiated deep discounts off list prices for new medicines with manufacturers, reducing prices below those in many Western European countries.¹⁰ Accounting for these discounts, prices today are nearly 80% lower than list prices at the launch of the class.¹¹

WHAT PAYERS CLAIMED WOULD HAPPEN



What they have done with this particular drug will break the country. . . . It will make pharmacy benefits no longer sustainable. Companies just aren't going to be able to handle paying for this drug.”

Express Scripts, April 2014¹²

This pricing, which Gilead attempts to justify as the cost of medical advancement, will have a tsunami effect across our entire health care system.”

America's Health Insurance Plans, July 2014¹³

WHAT ACTUALLY HAPPENED



The price is sufficiently low that we can go to our clients and say that they can treat every patient with hepatitis C.”

Express Scripts, January 2015¹⁴

We are receiving market-leading rates from both companies. Neither company wanted to be left off the formulary.”

Prime Therapeutics, January 2015¹⁵

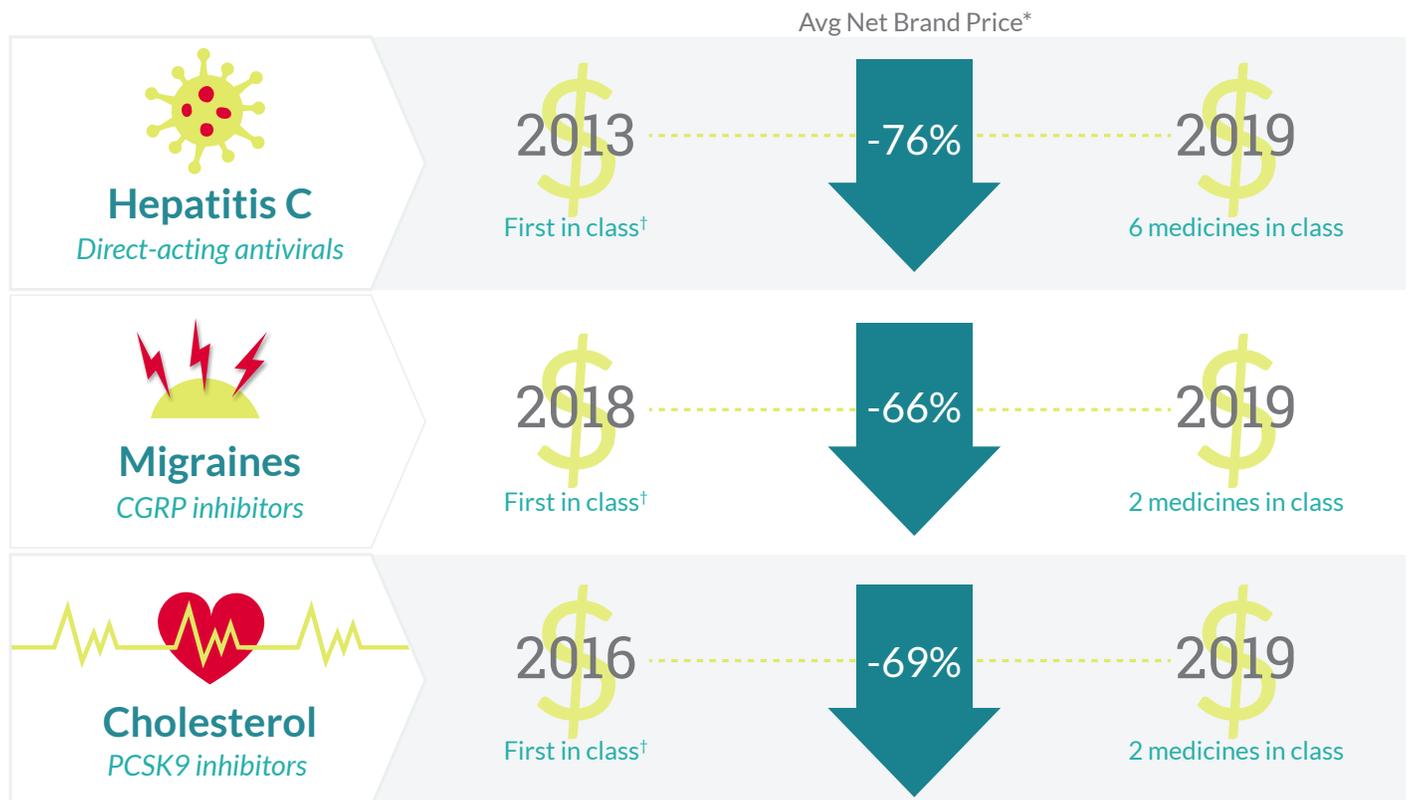
Competitive market forces and hard-nosed bargaining make ‘tremendously effective’ new hepatitis C medicines not just more accessible to ailing patients—but also offer good value to the U.S. health care system.”

The New York Times Editorial Board, September 2015¹⁶

Sources: LaMattina J¹⁰; SSR Health¹¹; Cortez MF¹²; Ignagni K¹³; Silverman E¹⁴; Langreth R¹⁵; New York Times Editorial Board¹⁶

Brand-to-Brand Competition Drives Savings in US Market-Based System

Payers leverage purchasing power and competition among brand medicines to negotiate substantial discounts on medicines.



*Percent change indicates difference between list price (WAC) at launch of first medicine in class and average sales-weighted net price in medicine class as of Q4 2019.

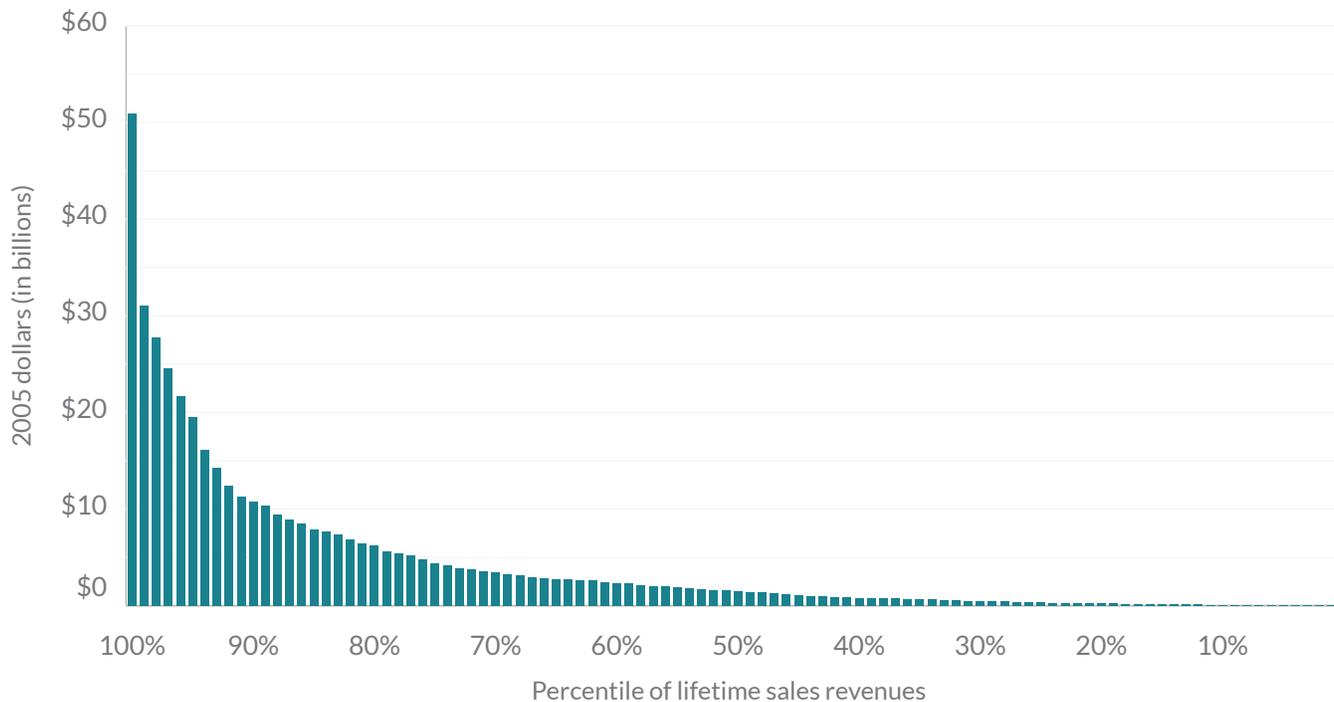
†Indicates launch year of the first medicine in this pharmacologic class.

Source: PhRMA analysis of SSR Health data¹⁷

Few Approved Medicines Are Commercially Successful

Ongoing investment in R&D depends on the commercial success of a few products that must make up for all the rest, including those that never reach the market.

Present Value of Lifetime Sales of Medicines* Introduced, 1991-2009



*A "medicine" is defined as a novel active substance (ie, a molecular or biologic entity or combination product in which at least one element had not been previously approved by the FDA). Sales are global sales, net of rebates and discounts.

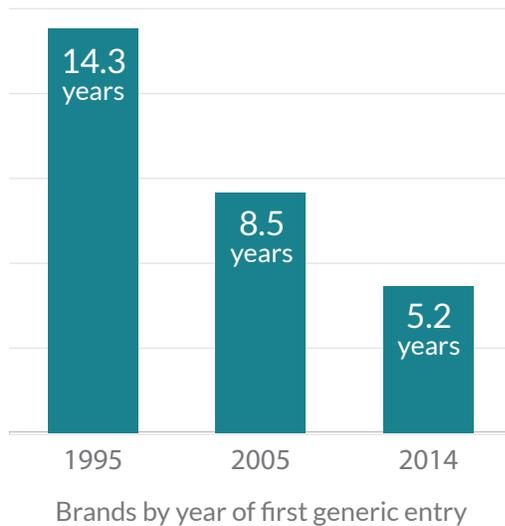
Source: Berndt ER et al¹⁸

Earlier and More Frequent Patent Challenges by Generic Companies

As early as 4 years after brand launch, a generic company may file a Paragraph IV certification with the FDA to challenge patents associated with the brand medicine, often allowing generic market entry before the patent expiration date.

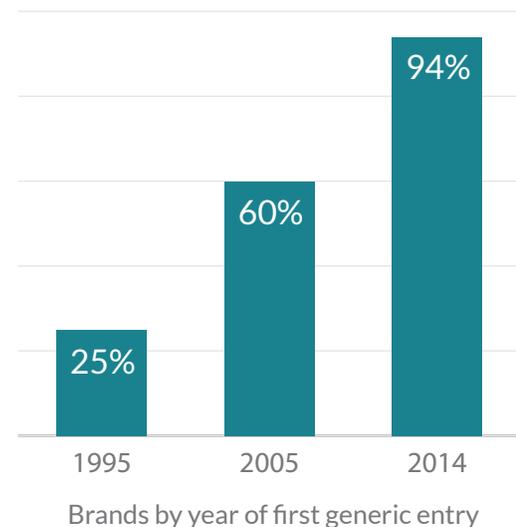
Patent challenges are occurring earlier...

Average Time From Brand Launch to Paragraph IV Patent Challenge*



...and are more common

Share of Brand Products Experiencing at Least 1 Paragraph IV Patent Challenge Prior to Generic Entry*



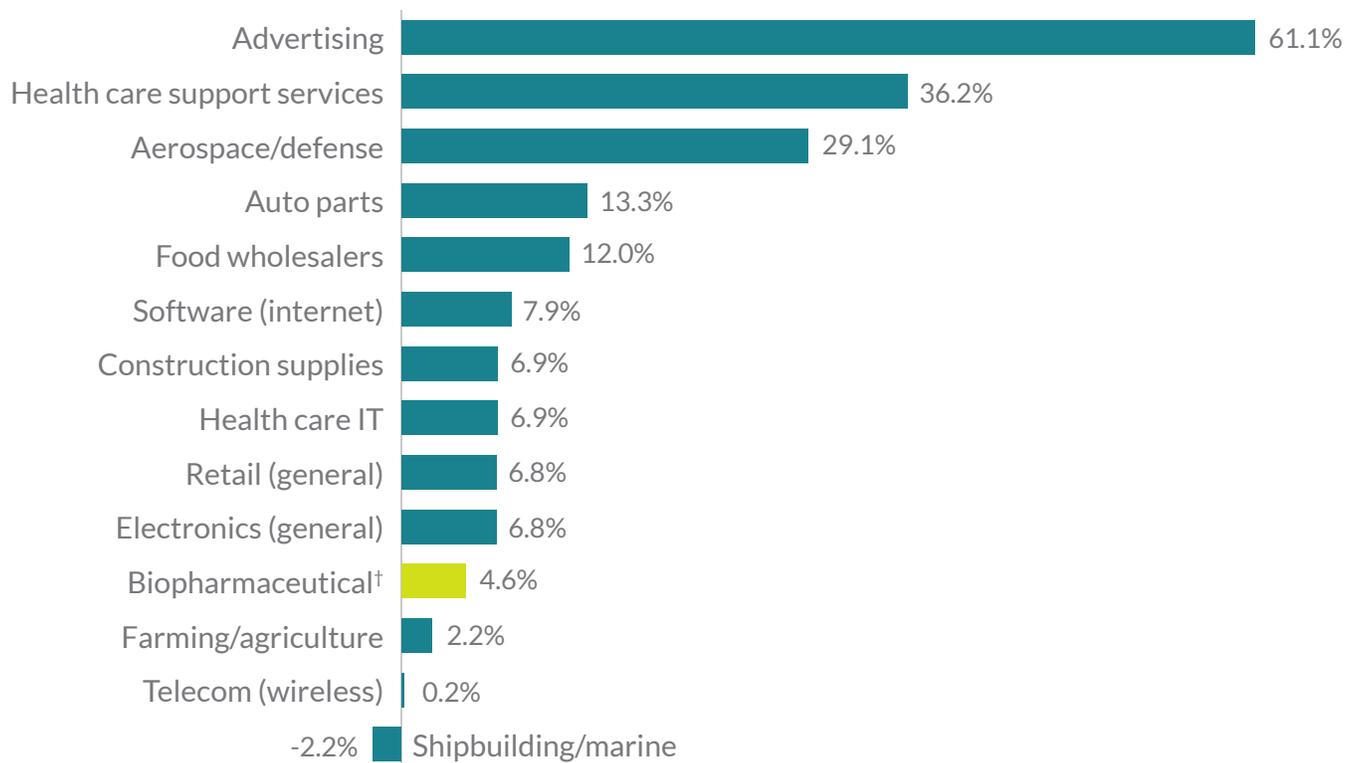
*All numbers are 3-year moving averages for brand medicines with more than \$250 million in annual sales in 2008 dollars, which account for 92% of sales of the brand medicines analyzed.

Source: Grabowski H et al¹⁹

Biopharmaceutical Profits Are in Line With Those of Other Industries

Accounting for the significant risk and capital investments required to develop medicines, biopharmaceutical industry profits are average among industries.

Average Economic Profit for Selected Industries, 2017-2019*



*Economic profits are accounting profits minus capital expenses.

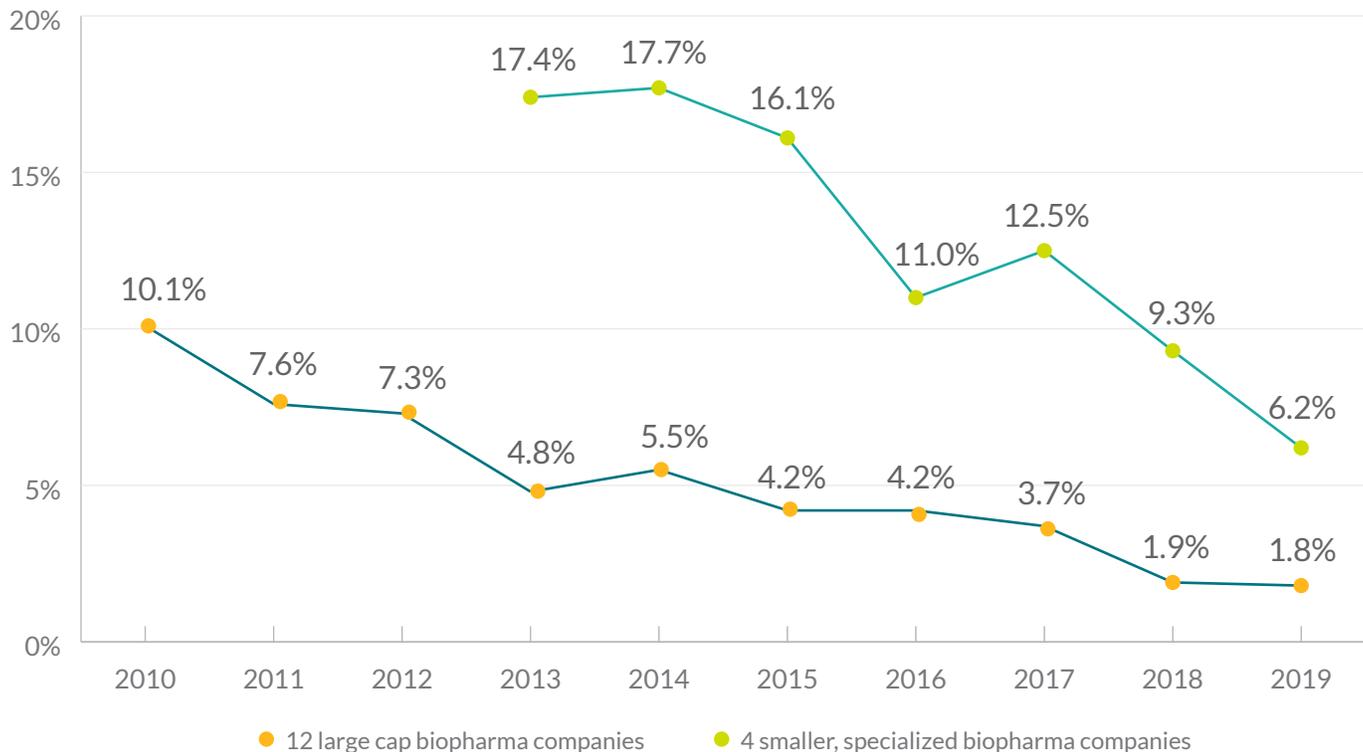
†Represents the weighted average of pharmaceuticals (8.2%) and biotechnology (2.2%), which are listed as separate industries in the source data.

Source: Adapted from Bates White²⁰

Increasingly Complex Science and Challenging Markets Have Led to Diminishing Returns on Research Investments

Despite headlines about large revenues from new medicine launches, biopharmaceutical companies have faced declining financial returns on their R&D investments.

Projected Internal Rate of Return for R&D Investments, 2010-2019

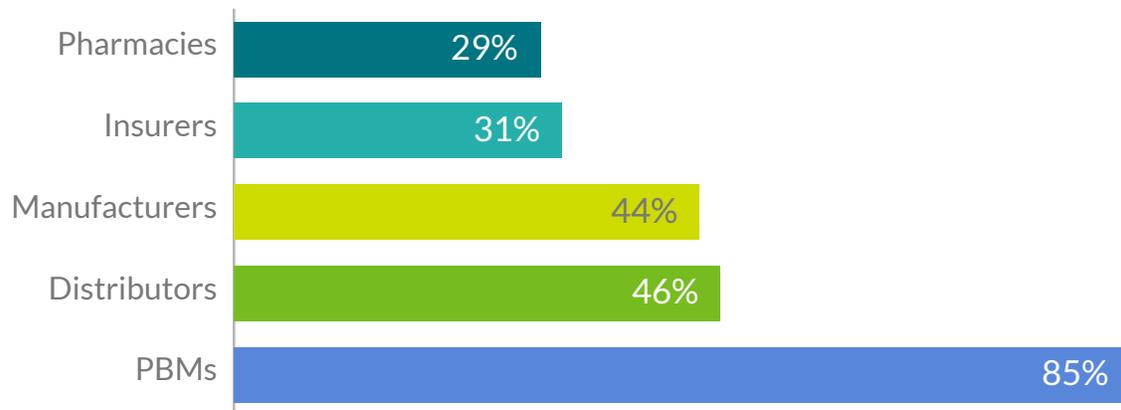


Source: Deloitte²¹

PBM Profit Margins Are Well Above Others in the Medicine Distribution and Supply Chain

Pharmacy benefit managers (PBMs) do not take possession of the medicines they manage, keeping their spending on fixed assets and other expenses very low. Their resulting profits are higher than manufacturers' profits, despite bearing very little risk.

Share of Gross Profit Converted to EBITDA, 2016-2017*



*Calculated as EBITDA (earnings before interest, taxes, depreciation, and amortization) margin divided by gross margin



Analysts at Bernstein tried to get a better picture of how profitable these [supply chain] companies are by excluding the cost of the drugs that are included in their revenue. . . . By this analysis, pharmacy-benefit managers are exceptionally profitable.”

Charley Grant, *Wall Street Journal*²⁴

Accounting Treatment of R&D Overstates Biopharmaceutical Profits



Correctly accounting for R&D as a long-lived investment tends to reduce substantially, if not to eliminate altogether, the inference that pharmaceutical companies are on average achieving supranormal profit returns.”

Frederic Scherer, AEI-Brookings Joint Center for Regulatory Studies²⁵



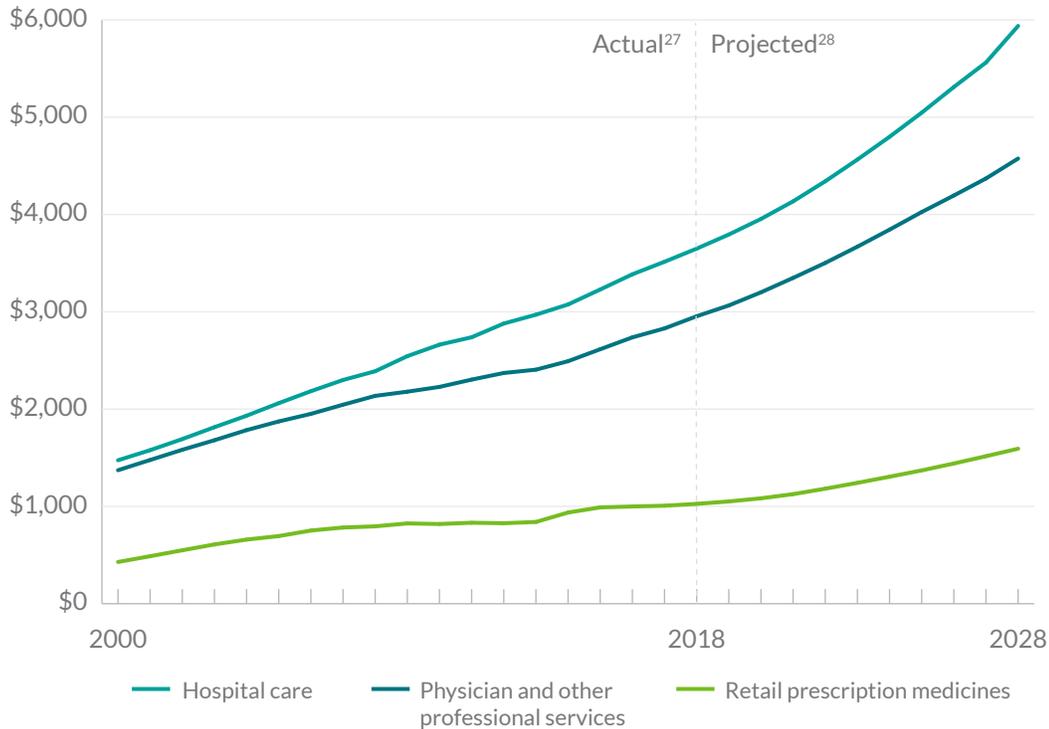
[T]he standard accounting measure of profits overstates true returns to R&D-intensive industries, such as pharmaceuticals, and makes it difficult to meaningfully compare profit levels among industries. Accounting measures treat most R&D spending (except for capital equipment) as a deductible business expense rather than as a capitalized investment. But the intangible assets that research and development generate—such as accumulated knowledge, new research capabilities, and patents—increase the value of a company’s asset base. Not accounting for that value overstates a firm’s true return on its assets.”

Congressional Budget Office²⁶

Sources: Scherer FM²⁵; CBO²⁶

Hospitals Continue to Drive Health Care Spending in the United States

Average US Health Care Spending per Person, 2000-2028



3x
more is spent on hospitals than on retail prescription medicines.

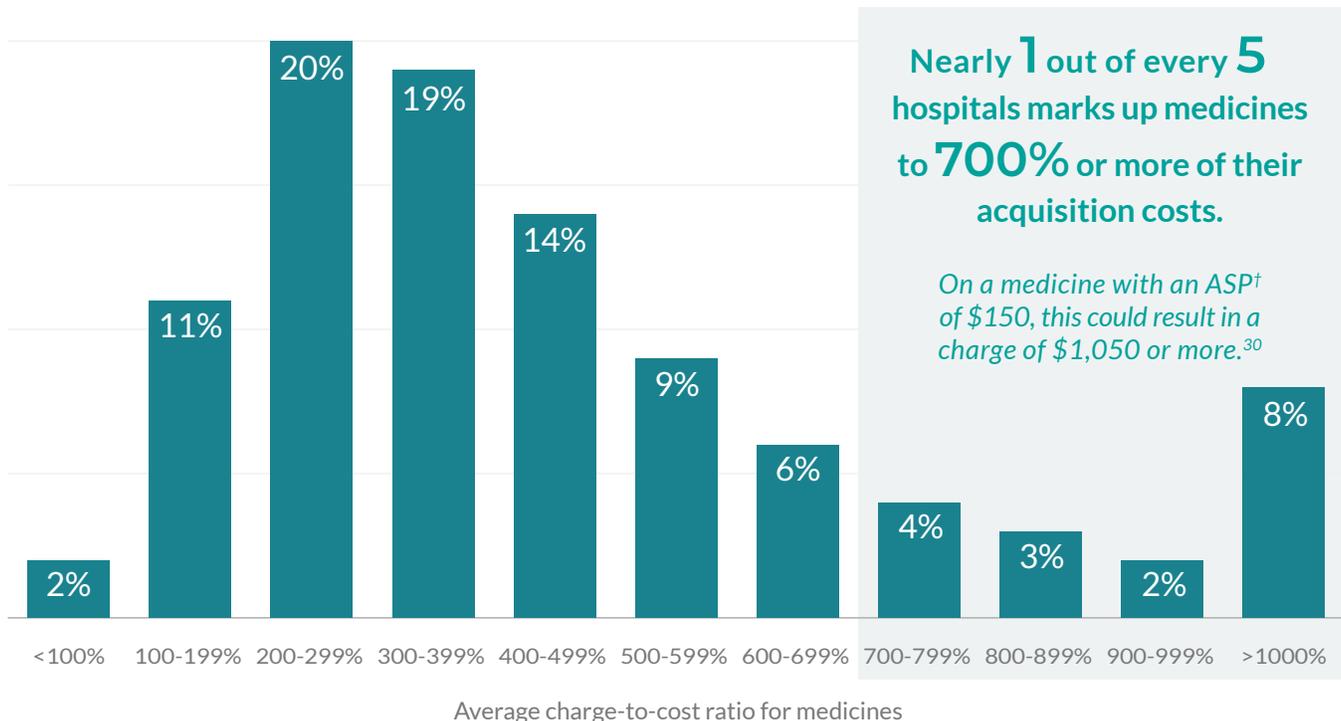
This trend is projected to continue through **2028.**

Sources: CMS^{27,28}

Hospitals Mark Up Medicines in the Outpatient Setting, Driving Up Costs to Patients and the Health System

Hospitals mark up medicine prices by nearly 500% on average. The amount hospitals receive after negotiations with commercial payers is, on average, more than 250% what they paid to acquire the medicine.²⁹

Percentage of Hospitals by Average Level of Markup for Medicines*



*Percentages in chart may not add up to 100% due to rounding.

†ASP: Average sales price

Sources: The Moran Company^{29,30}

Medicines Are Not the Major Driver of Hospital Spending Increases

Increased spending on medicines accounted for just 10% of total hospital spending growth between 2015 and 2017.³¹⁻³³

WHAT HOSPITALS CLAIMED

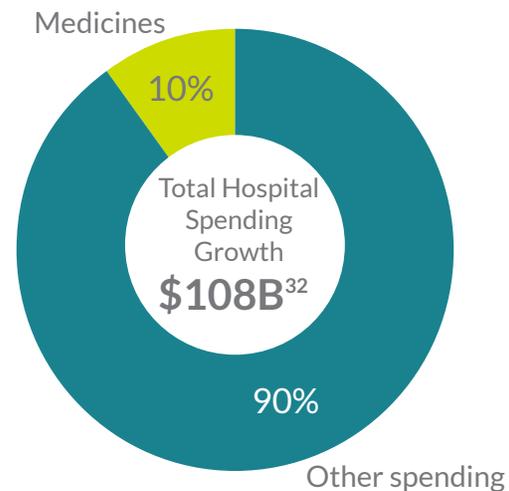


[W]e are in the midst of a prescription drug spending crisis that threatens patient access to care and hospitals' and health systems' ability to provide the highest quality of care."

**American Hospital Association,
January 2019³⁴**

WHAT ACTUALLY HAPPENED

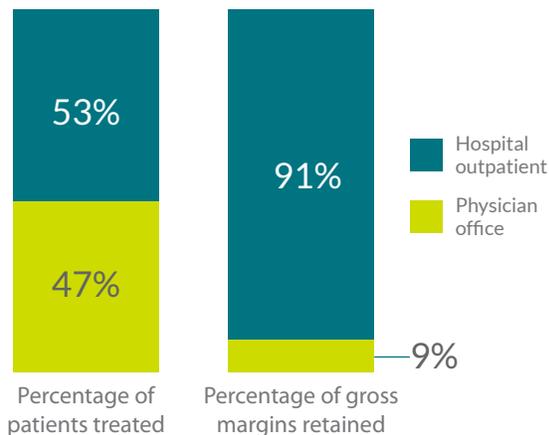
Source of Hospital Spending Growth, 2015-2017



Sources: NORC at the University of Chicago³¹; Martin AB et al³²; American Hospital Association^{33,34}

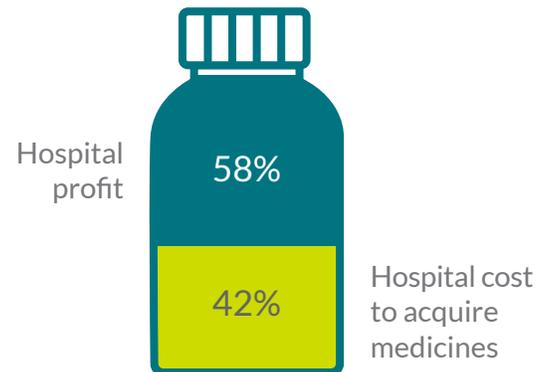
Hospitals Retain Significant Profits From Commercial Payers for Provider-Administered Medicines

Hospitals and Physicians Treat Similar Numbers of Patients but Hospitals Keep a Larger Share of Profit Margin



Hospitals collect 91% of the margin while serving 53% of patients receiving provider-administered medicines.³⁵

Hospitals Can Benefit More than Innovative Manufacturers Under the Current System



Hospitals retain \$58 for every \$100 reimbursed on provider-administered medicines in the hospital outpatient setting, while \$42 is shared between manufacturers and pharmaceutical supply chain entities.³⁶

Sources: Partnership for Health Analytic Research LLC³⁵; Yu NL et al³⁶

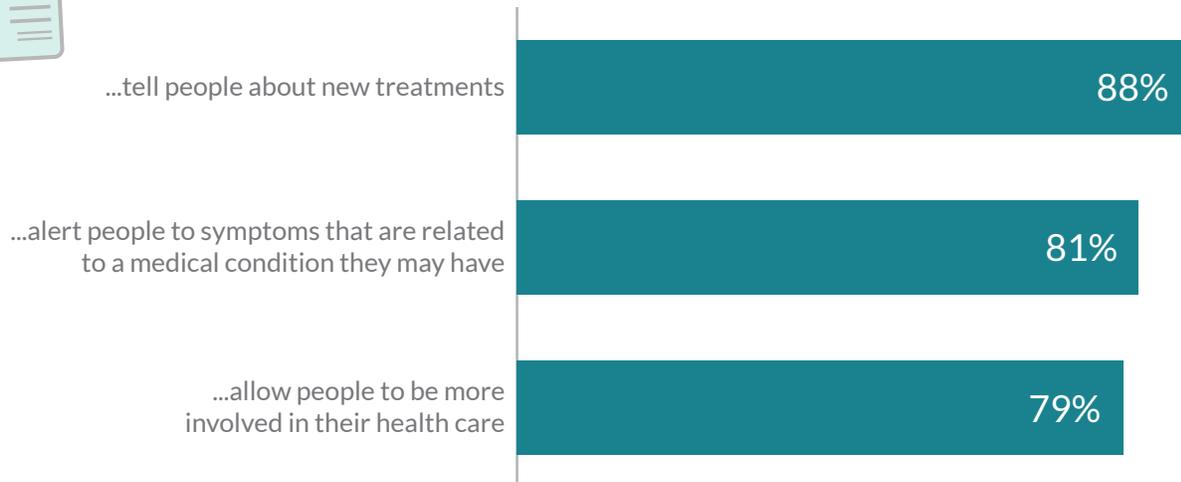
Direct-to-Consumer Advertising Increases Awareness of Conditions and Treatments

A survey of consumers demonstrated the positive contribution of direct-to-consumer (DTC) advertising to patients' knowledge.

How strongly do you agree or disagree with each statement?
Percentage who **AGREE** with each statement
(Survey of 1,564 consumers, April 2017)



DTC ads

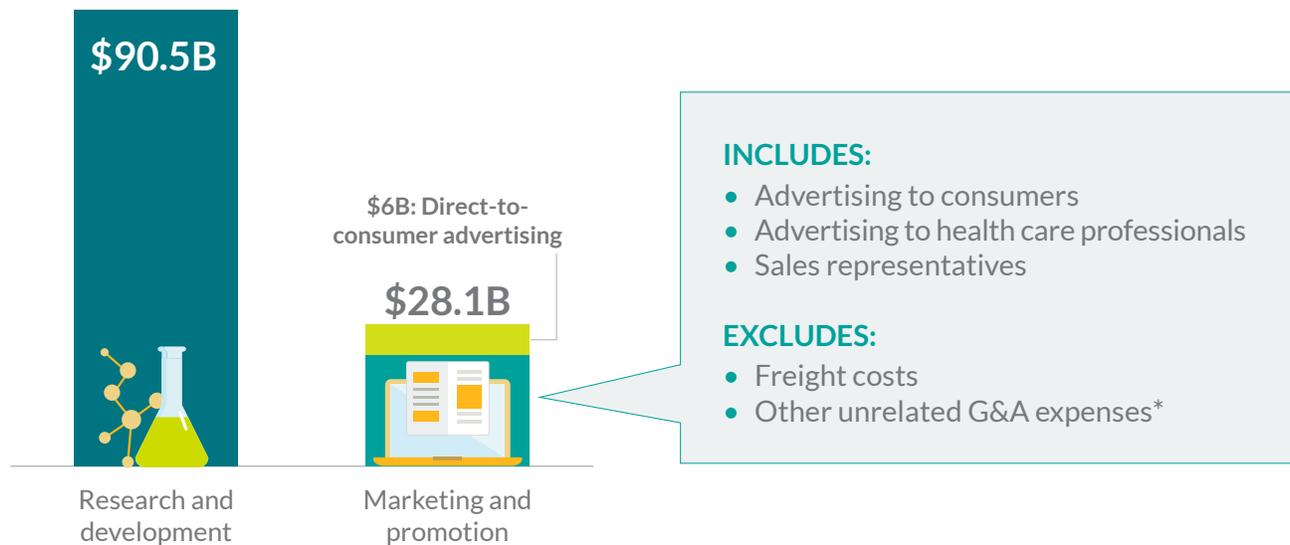


Source: Princeton Survey Research Associates International³⁷

Biopharmaceutical Company Marketing and Promotion Spending in Context

Inflated estimates of marketing and promotion spending has created the false impression that the biopharmaceutical industry spends more on marketing than on R&D. More precise estimates show the opposite to be true.

Select US Biopharmaceutical Industry Expenses, 2016



*Indicates general and administrative (G&A) expenses unrelated to marketing and promotion, such as finance and office staffs, rent, utilities, and supplies. Some have inaccurately used sales and G&A expenses as a proxy for industry marketing and promotion expenses.

Sources: Schwartz LM et al³⁸; Research!America³⁹

Innovative Contracts Deliver Results for Patients

Innovative contracts have the potential to benefit patients and the health care system by improving patient outcomes, reducing medical costs, and reducing the costs of medicines.

Outcomes-based contracts are associated with **28% lower** patient copayments.⁴⁰

One PBM saved **\$4.3B** through value-based programs in 2019.⁴¹

Patients saved nearly **\$800K** out of pocket through innovative contracting arrangements on cholesterol-lowering medication.⁴²



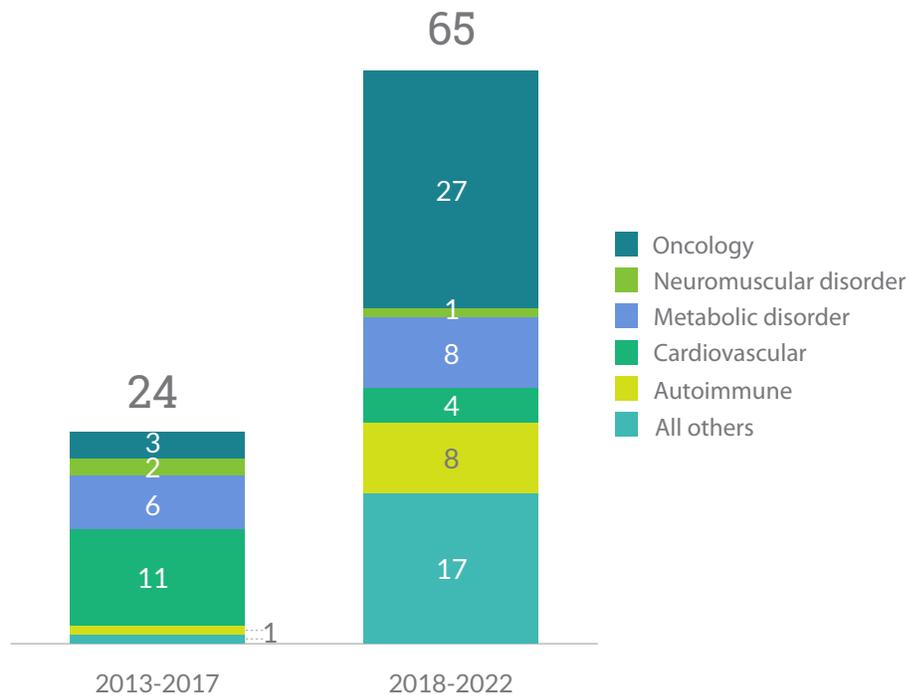
“We’ve been able to get the best of both worlds. The insurer gets competitive guaranteed discounts on prescriptions, and the manufacturer is aligned and accountable when something doesn’t work.”

Chris Bradbury, Cigna⁴²

Innovative Market-Based Arrangements That Link Payment for Medicines to Outcomes Are on the Rise

In 2019, 59% of payers executed at least one outcomes-based contract. This is more than double compared to 2017 when only 24% of payers executed at least one outcomes-based contract.⁴³

New US Publicly Announced Outcomes-Based Contracts and Projected Future Increases⁴⁴



Sources: Bulter S et al⁴³; IQVIA Institute⁴⁴

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4

COST SHARING TRENDS

Evaluating the Impact of Insurance Benefit Design on Patients

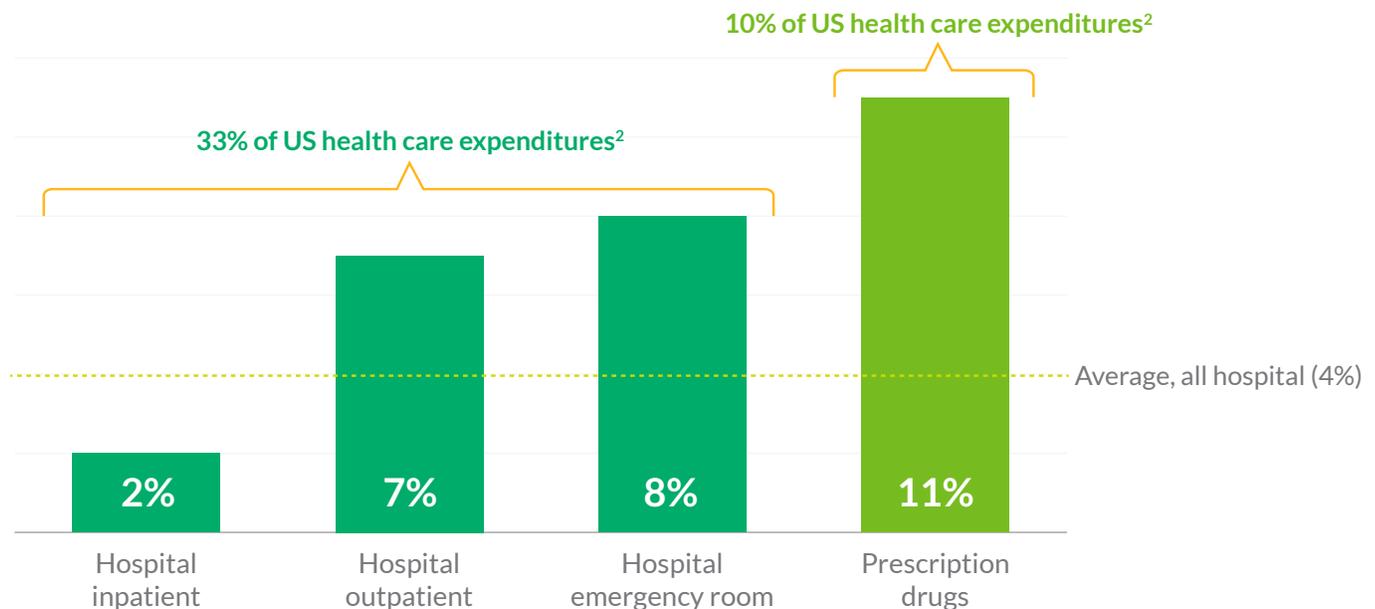


Insurers are increasingly using high deductibles, coinsurance, and multiple cost sharing tiers, which push more costs to some patients. Out-of-pocket spending for prescription medicines can represent a disproportionate share of total health care costs borne directly by patients, especially those who are low income or chronically ill. High cost sharing for medicines may limit patients' access to needed treatments, reduce adherence, and lead to poor health outcomes. Manufacturer cost sharing assistance can help patients afford their medicines and lower abandonment rates.

Insurance Covers a Lower Share of Prescription Costs Than Hospital Care Costs

On average, patients pay out of pocket 11% of their prescription drug costs compared with 4% of costs for hospital care. Meanwhile, hospital care as a percentage of US health care expenditures is significantly larger than outlays on medicines.

Average Share of Health Care Costs Patients Pay Out of Pocket, All Ages¹

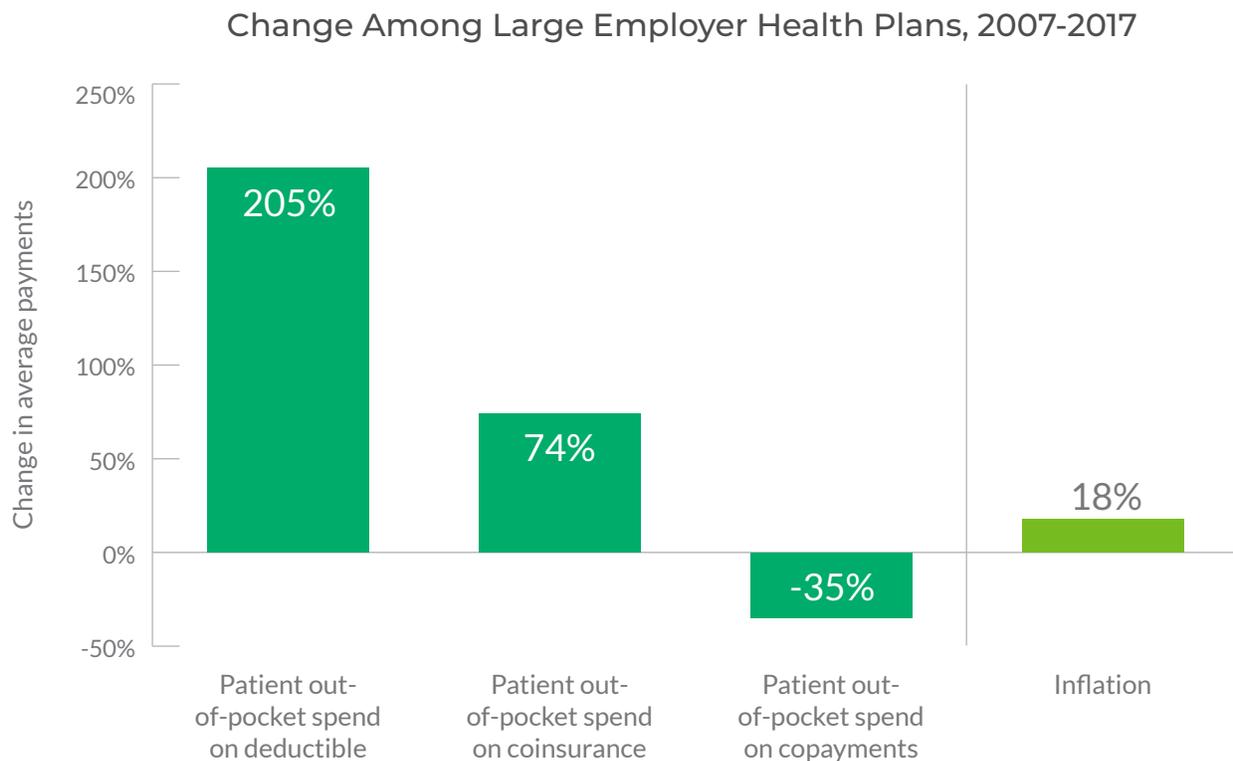


Analysis includes individuals with any source of health care coverage, public or private; this includes individuals who had health coverage without coverage for prescription drugs, which can be expected to account for less than 2% of those with health coverage. Prescription drug spending includes spending on brand and generic drugs, pharmacy, and distribution costs for retail prescriptions. Note: Prescription drug out-of-pocket costs are based on gross medicine price, not the net price after rebates.

Sources: Avalere Health analysis of Medical Expenditure Panel Survey, 2017¹; CMS²

Patient Spending Rises as Plans Use More Deductibles and Coinsurance

Trends in health plan design—toward higher deductibles and coinsurance, and decreased use of copayments—have shifted costs to patients at a higher rate than inflation.

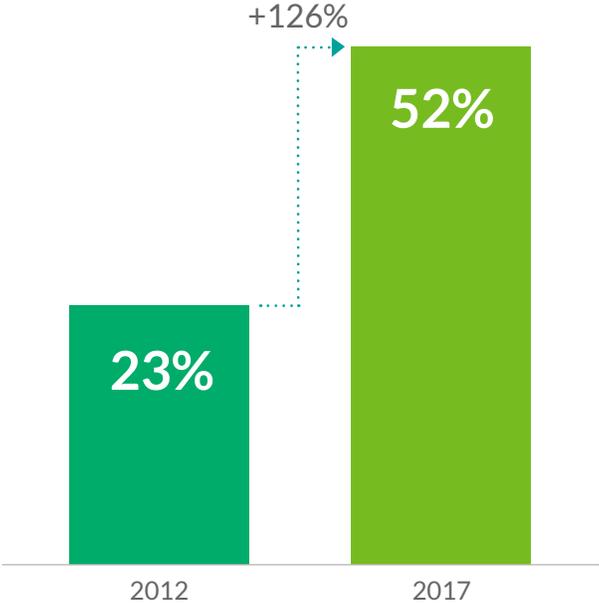


Source: Peterson Center on Healthcare and Kaiser Family Foundation³

Share of Employer-Sponsored Health Plans With a Prescription Drug Deductible Is Increasing

The percentage of employer-sponsored plans requiring deductibles for pharmacy benefits continues to increase.

Percentage of Plans With Deductibles for Prescription Drugs



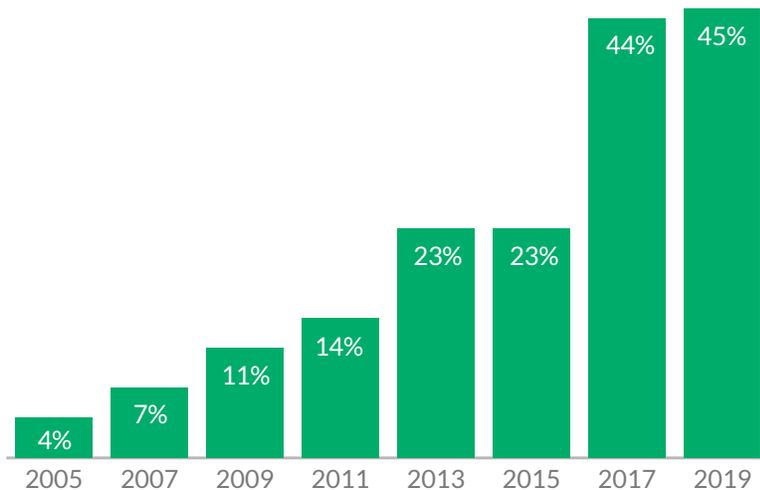
Source: PwC⁴

Plans Increasingly Subject Certain Medicines to Higher Cost Sharing

Increased use of 4 or more tiers by plans means that more patients are subject to what is commonly higher cost sharing on the specialty tier. Medicines on the specialty tier are also more likely to be subject to coinsurance than products placed on lower cost sharing tiers.⁵

The use of 4 or more cost sharing tiers is becoming more common in employer plans.

Share of Workers in Plans With 4 or More Tiers⁶⁻⁸



For fourth tier^{8*}



Average coinsurance

29%

Average copay

\$123

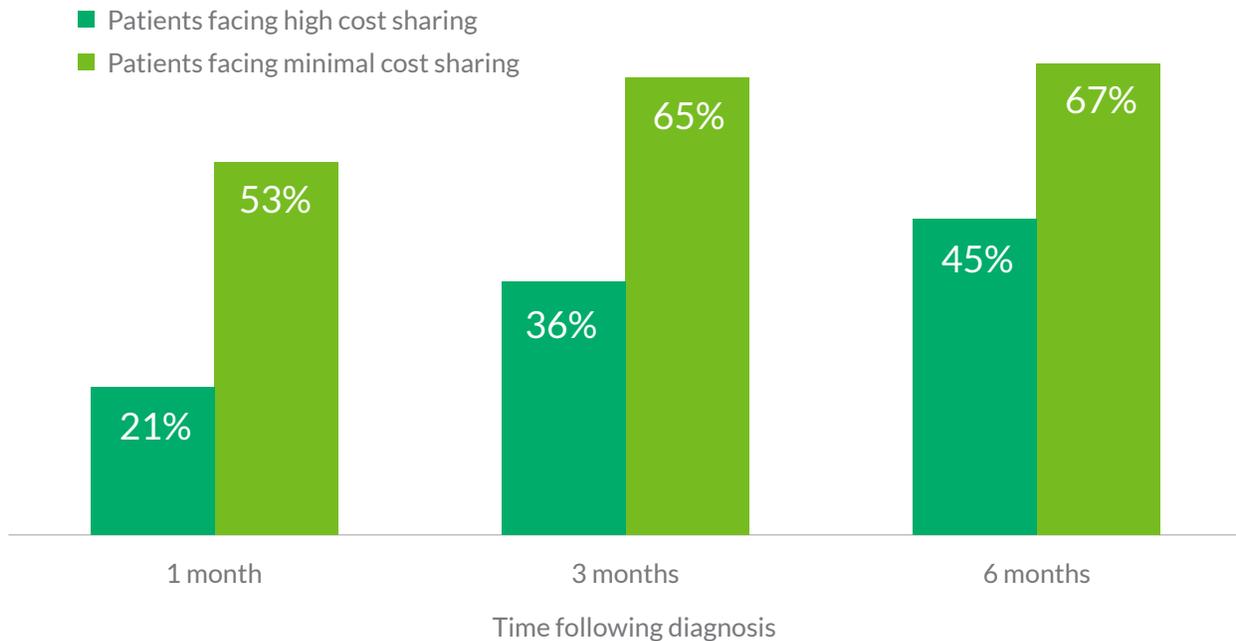
*53% of plans with coinsurance for the fourth tier have a maximum amount.

Sources: Kaiser Family Foundation⁵⁻⁸

Patients Facing High Cost Sharing Commonly Do Not Initiate Treatment

Patients with chronic myeloid leukemia facing high out-of-pocket costs for medicines on a specialty tier are less likely to initiate drug therapy than patients receiving a cost sharing subsidy, and these patients take twice as long to initiate treatment.

Percentage of Patients With Chronic Myeloid Leukemia Initiating Treatment

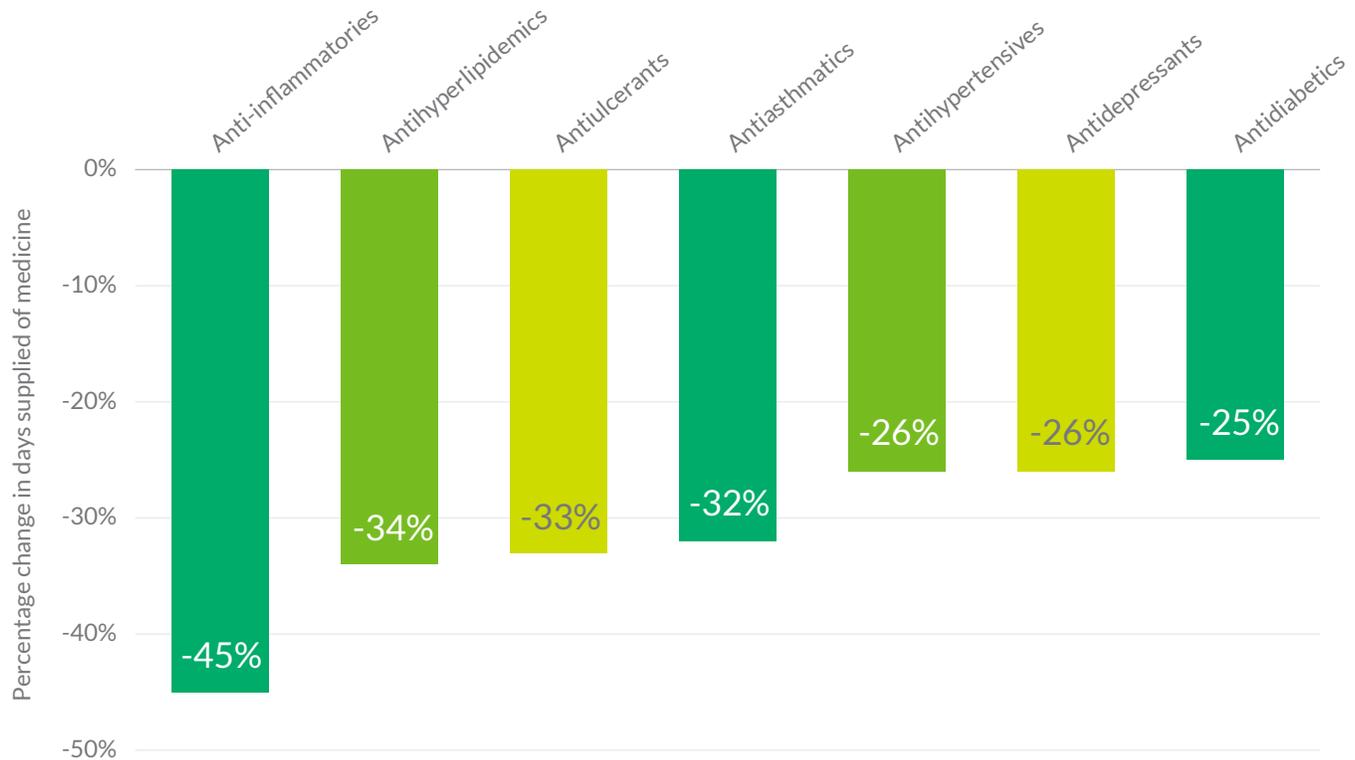


Source: Doshi JA et al⁹

High Cost Sharing Reduces Adherence

RAND researchers found that doubling copays reduced patients' adherence to prescribed medicines by 25%-45% and increased emergency room visits and hospitalizations.

Percentage Change in Adherence From Doubling Medicine Copays, by Drug Class

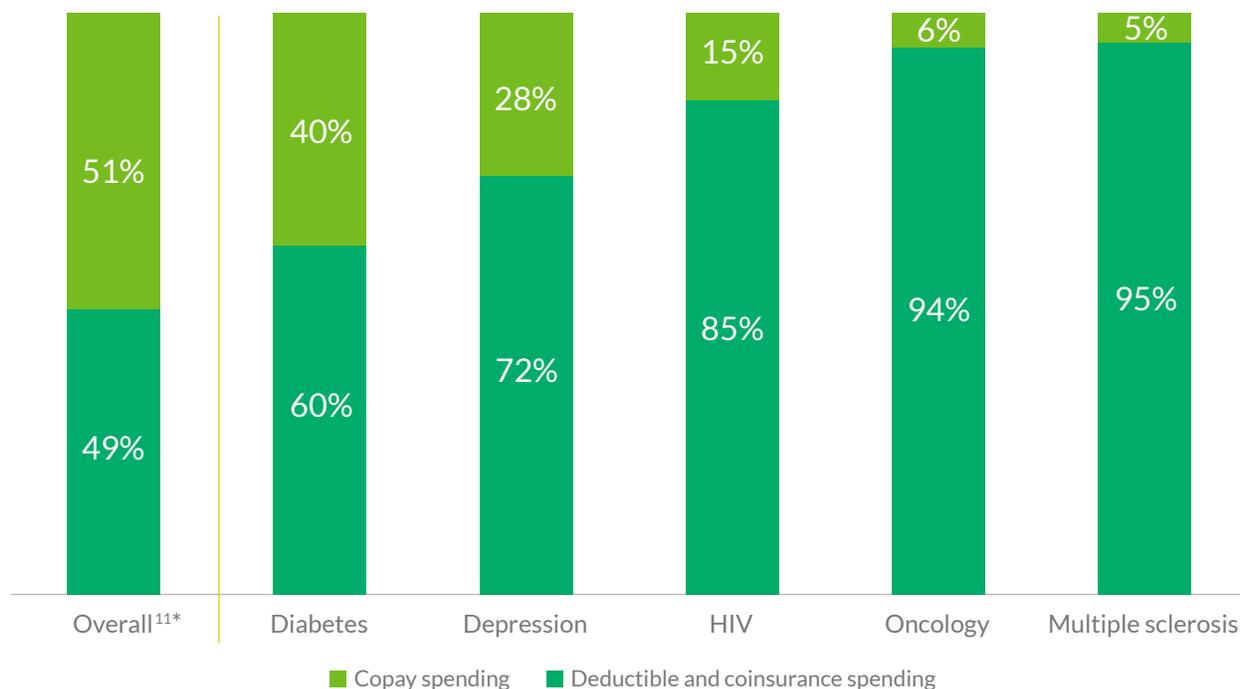


Source: Goldman DP et al¹⁰

Cost Sharing Is Typically Based on the List Price When Patients Pay for Brand Drugs With Coinsurance or While in the Deductible

Despite manufacturers' rebates and discounts negotiated by health plans, nearly half of commercially insured patients' out-of-pocket spending for brand medicines is based on the medicine's undiscounted list price.¹¹

Share of Final Out-of-Pocket Spending on Brand Medicines by Type of Cost Sharing Across Therapeutic Classes, 2019 (Commercial Market)¹²



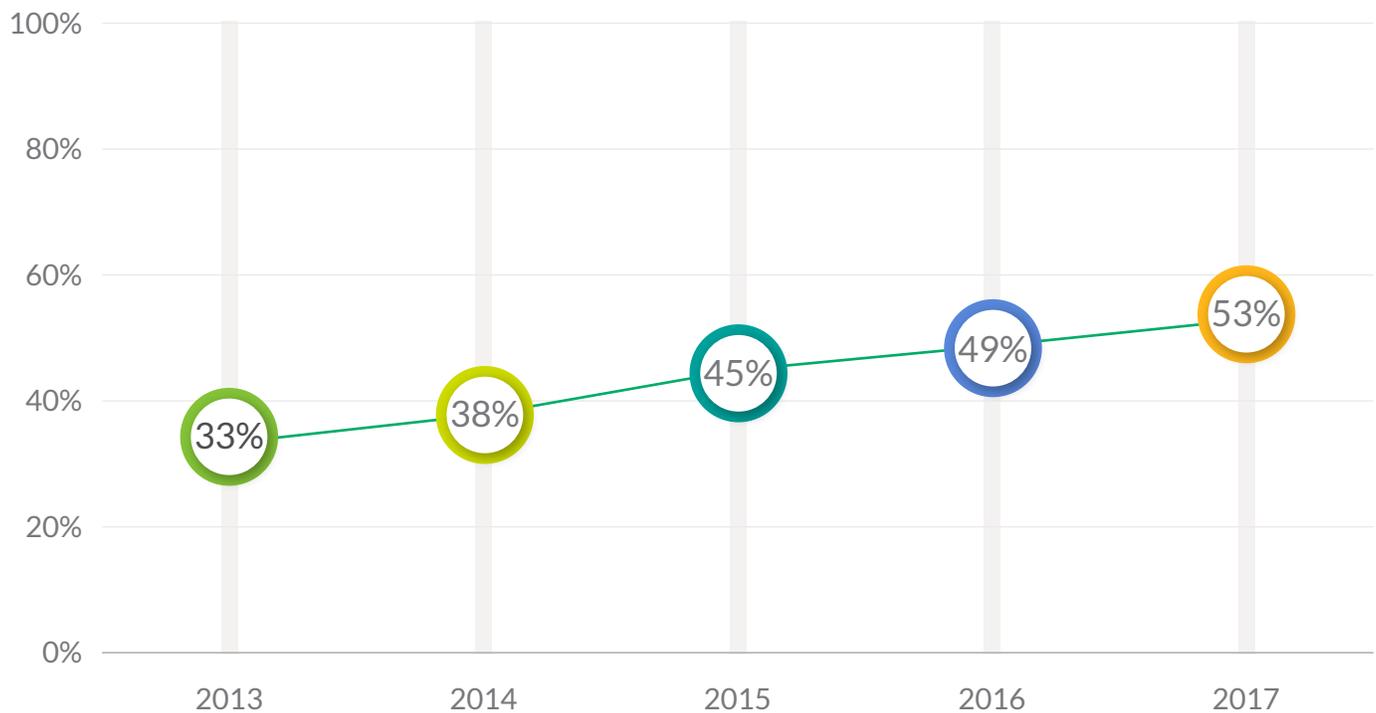
*"Overall" is based on IQVIA Longitudinal Access and Adjudication Data (sample claims data, 2019) and is not limited to the therapeutic areas displayed.

Sources: IQVIA Institute¹¹; IQVIA analysis for PhRMA¹²

Cost Exposure for Brand Medicines Is Becoming More Prevalent Over Time

In 2017, 7% of claims for brand medicines had cost sharing of \$125 or more, and these claims now represent more than half of total patient cost exposure.

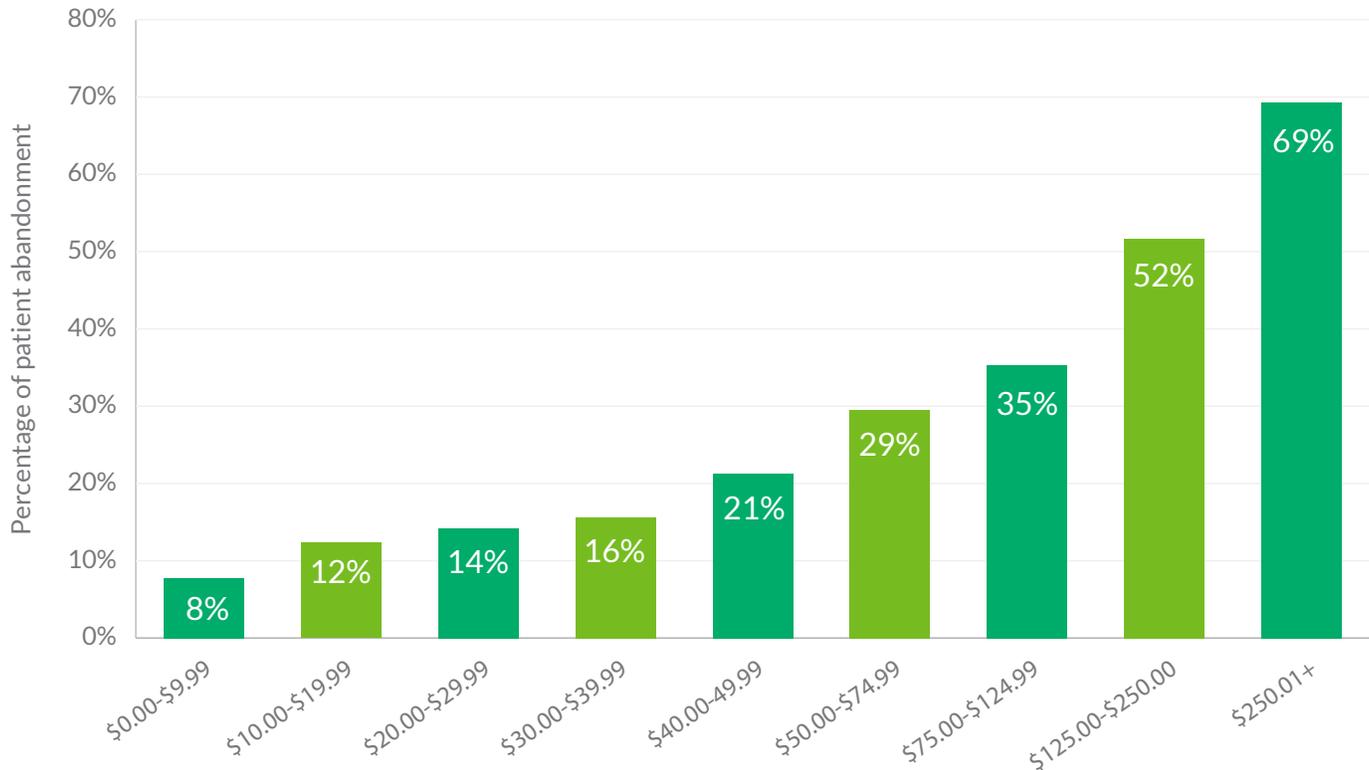
Share of Total Patient Cost Exposure Accounted for by \$125+ Claims
(Commercial Claims; Brands; 2013-2017)



Source: IQVIA¹³

As Cost Sharing Rises, Patients Are More Likely to Abandon Their New Medicines

New Patient Abandonment by Final Out-of-Pocket Cohort*
(Commercial Claims; PayCo® Brands; 2017)



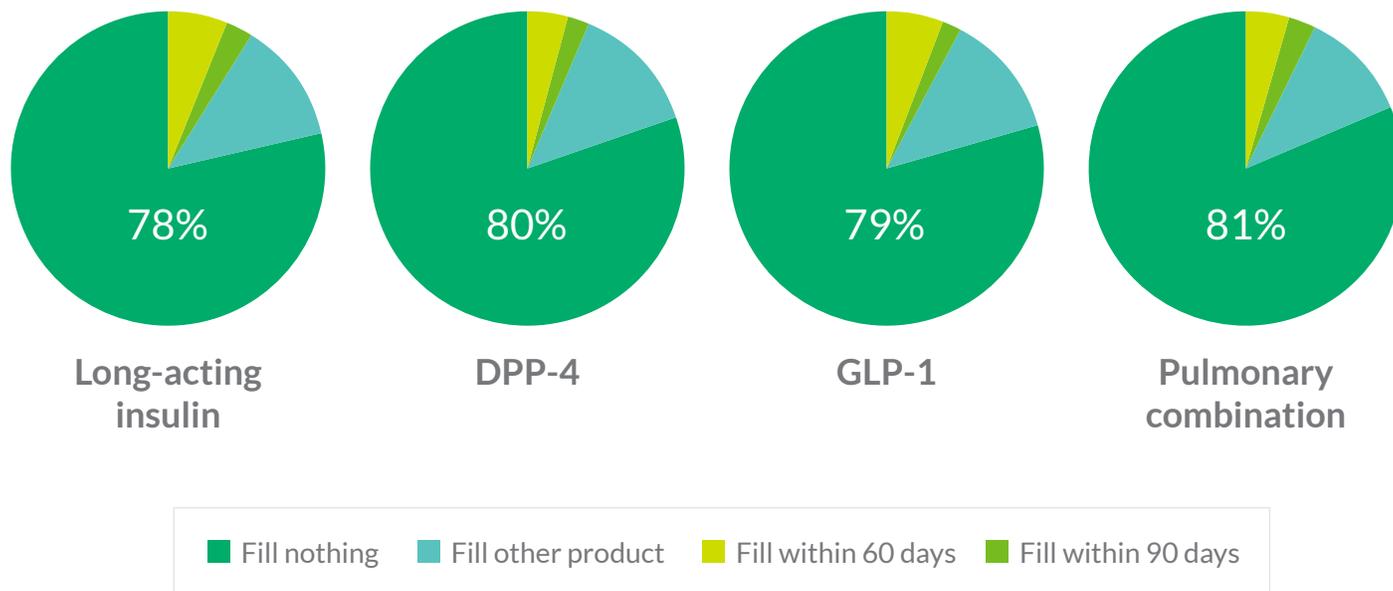
*Sample is limited to new patient approvals across top brands, which span over 25 traditional and specialty therapeutic areas.

Source: IQVIA¹⁴

Patients Who Abandon Prescriptions Often Do Not Initiate Another Therapy

Most patients who abandon a brand drug do not fill another drug prescription within 90 days, indicating that they may not be receiving any treatment for their condition.

New Patient Abandonment, Subsequent Fill (Brands, 2014)

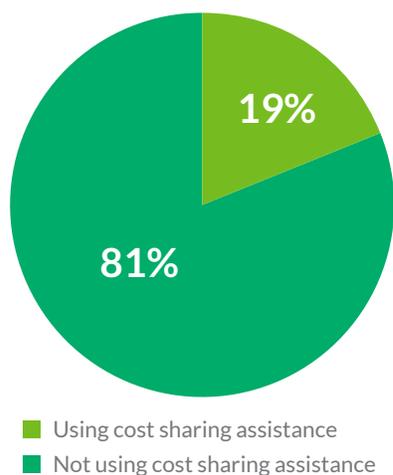


Source: IMS Institute for Healthcare Informatics¹⁵

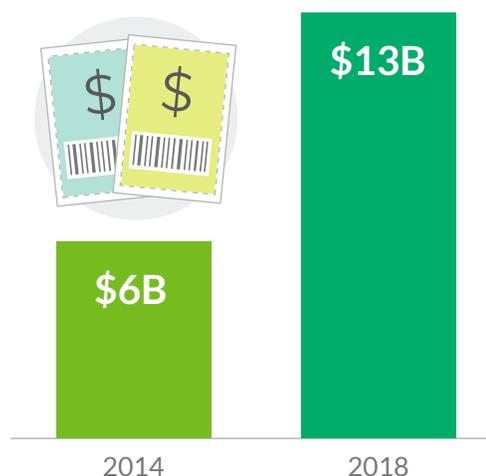
Manufacturer Cost Sharing Assistance Is an Important Source of Financial Help for Commercially Insured Patients

Manufacturer cost sharing assistance helps commercially insured patients who otherwise might struggle to afford their out-of-pocket costs. Manufacturer cost sharing assistance—like other third-party financial assistance—helps patients pay the full out-of-pocket costs of their prescribed and covered medicines at the pharmacy, and this assistance does not undermine plans' benefit design or utilization management techniques.

Percentage of Commercially Insured Patients Using Manufacturer Cost Sharing Assistance for Brand Drugs, 2018¹⁶



Manufacturer Cost Sharing Assistance Helps Commercially Insured Patients Pay Out-of-Pocket Costs¹⁶

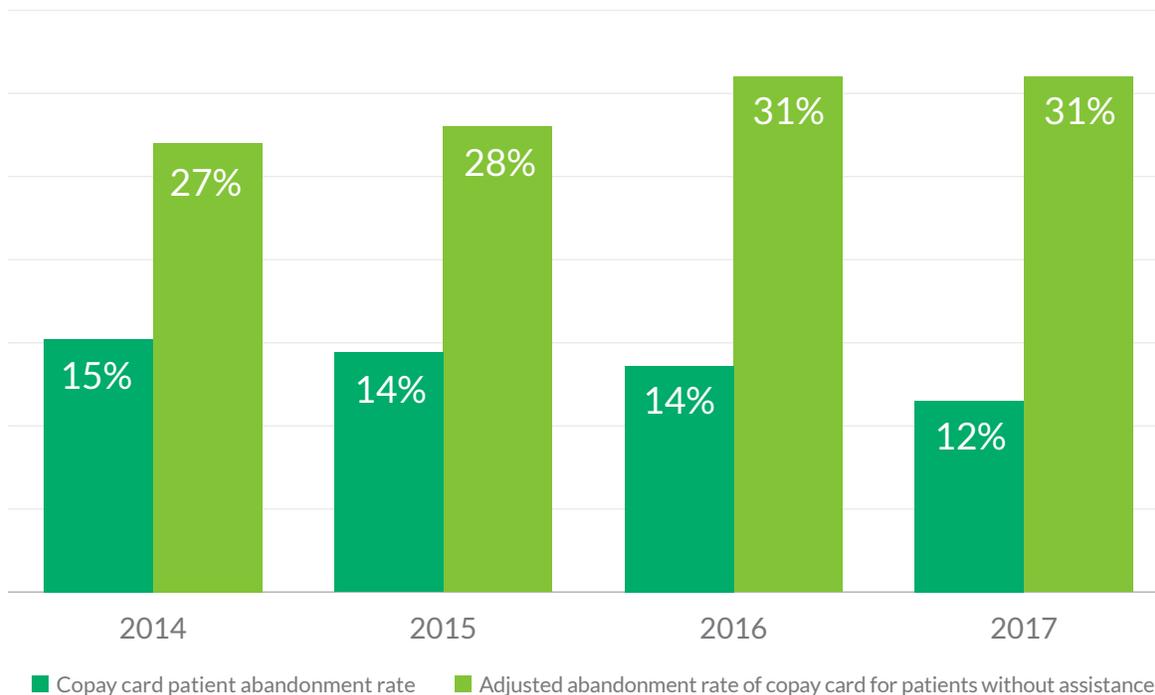


Source: IQVIA Institute¹⁶

Manufacturer Cost Sharing Assistance Can Help Offset Patient Abandonment

Patient abandonment rates increase with out-of-pocket costs, but manufacturer cost sharing assistance, like copay cards, helps patients pay their out-of-pocket costs, which lowers abandonment rates.

New Patient Abandonment Trend Comparing Current and Adjusted Copay Card for Patients (Commercial Claims; PayCo® Brands)

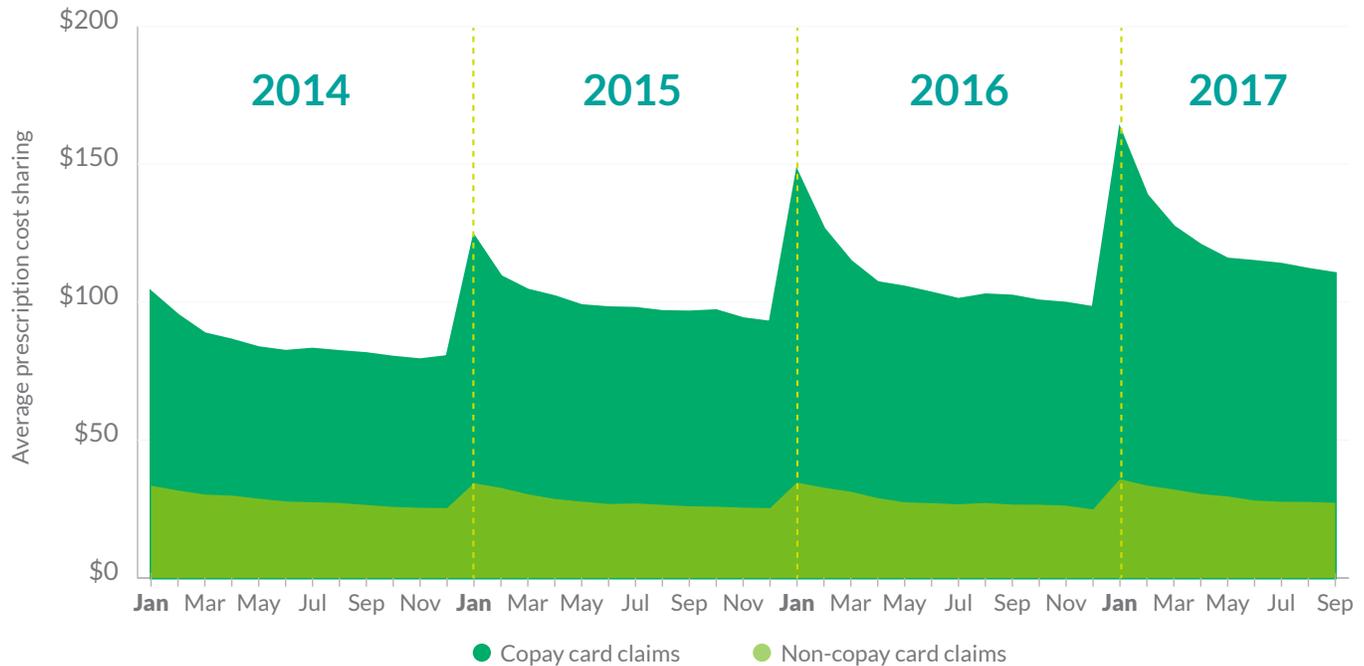


Source: IQVIA¹⁷

Without Cost Sharing Assistance, Patients Would Pay Higher Average Out-of-Pocket Costs per Prescription

Each January, patients in the commercial market with deductibles face steep increases in out-of-pocket costs for brand drugs.

Average Primary Cost Exposure, 2014-2017*
(Commercial Copay Card Claims; All Brands)



*Averages are calculated among paid claims where a copay card is used as the secondary payer and normalized to 30 days.

Source: IQVIA¹⁸

Manufacturer Cost Sharing Assistance Can Help Patients Pay Their Out-of-Pocket Costs



In 2017, just **0.4%** of commercial claims were filled with a coupon for a **brand medicine** that had a generic equivalent.

Programs that do not count manufacturer cost sharing assistance toward a patient's deductible or out-of-pocket maximum hurt the sickest patients, leaving them vulnerable to unexpected out-of-pocket costs as high as **several thousands of dollars** to continue taking their medicine.



Source: IQVIA¹⁹

Accumulator Adjustment Programs May Increase Medication Nonadherence

Accumulator adjustment programs (AAPs), operated by health plans, exclude the value of manufacturer cost sharing assistance from patients' deductibles and out-of-pocket maximums. This can result in increased patient out-of-pocket costs and nonadherence.

Impact of AAP Implementation on High-Deductible Health Plan (HDHP) Enrollees With Health Savings Accounts Taking Specialty Autoimmune Medicines²⁰

HDHP ENROLLEES HAD:



233

fewer autoimmune drug fills per 1,000 patients



20%

higher treatment discontinuation



12%

fewer prescription days covered

Pharmaceutical Company Assistance Helps Patients With Needed Financial Support

Despite more Americans having insurance, many are facing high cost sharing that puts their ability to stay on a needed therapy at risk. Because of this, biopharmaceutical companies provide patient assistance in a variety of ways.

Building off the work of the Partnership for Prescription Assistance, PhRMA built the

Medicine Assistance Tool (MAT)

in 2019 to provide patients, caregivers, and providers with a streamlined point of access for information that can help them make more informed health care decisions.

MAT INCLUDES:

- A search engine to connect patients with **medicine-specific financial assistance programs**
- Resources to **help patients navigate** their insurance coverage
- Links to websites **providing cost information** referenced in PhRMA member company direct-to-consumer television advertising



Source: PhRMA²¹

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5

SPENDING ON MEDICINES

Understanding Medicine
Costs in Context

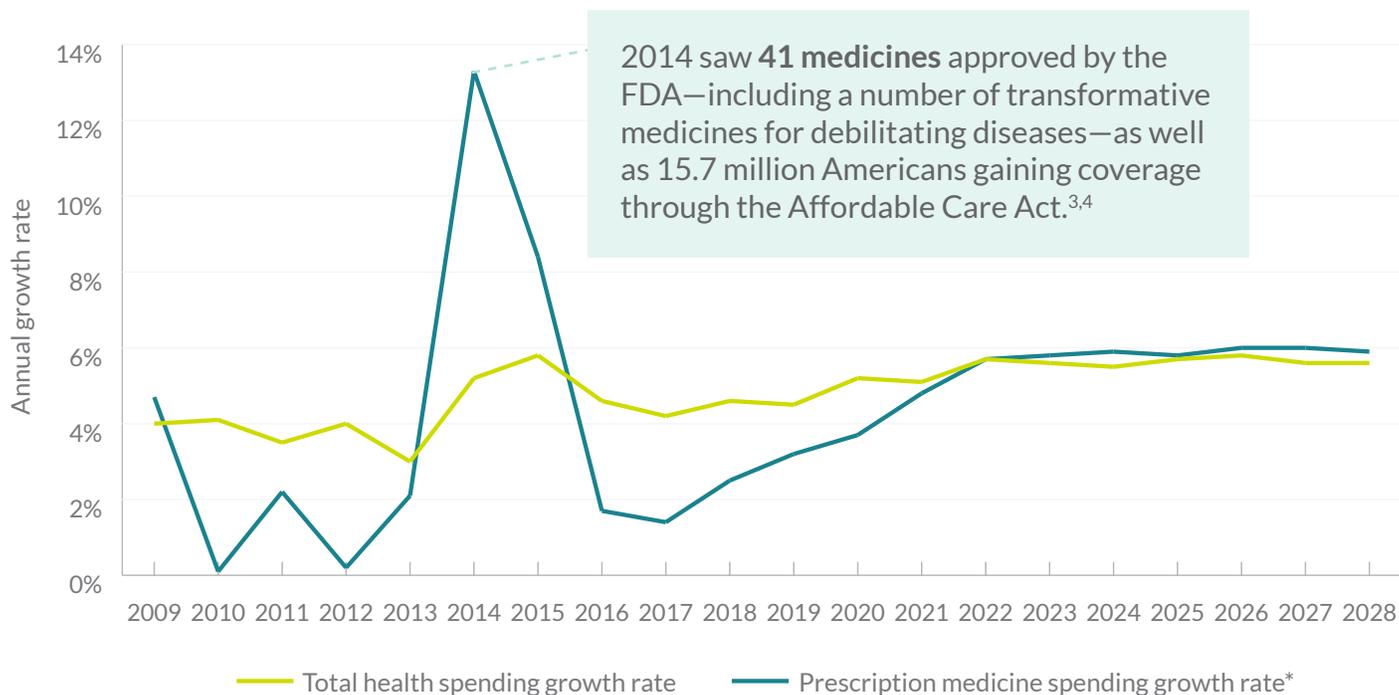


Prescription medicines represent a small share of national health spending, and government estimates project that medicines will remain a stable share of health spending through the next decade. In 7 of the past 10 years, spending on retail prescription medicines grew more slowly than total health care spending and is projected to grow just 3% to 6% annually over the next decade, in line with total health care spending. Rebates, discounts, and fees paid by brand manufacturers to the government, private payers, and supply chain entities increased to \$175 billion in 2019. Brand medicine net price growth, which reflects these rebates and discounts, has been in line with or below inflation for the past 5 years.

In 7 of the Last 10 Years, Retail Prescription Medicine Costs Grew More Slowly Than Total Health Care Costs

Government actuaries project prescription medicine spending growth to remain between 3% and 6% annually through 2028, in line with overall health care spending growth.¹

Prescription Medicine Spending Growth, 2009-2028²

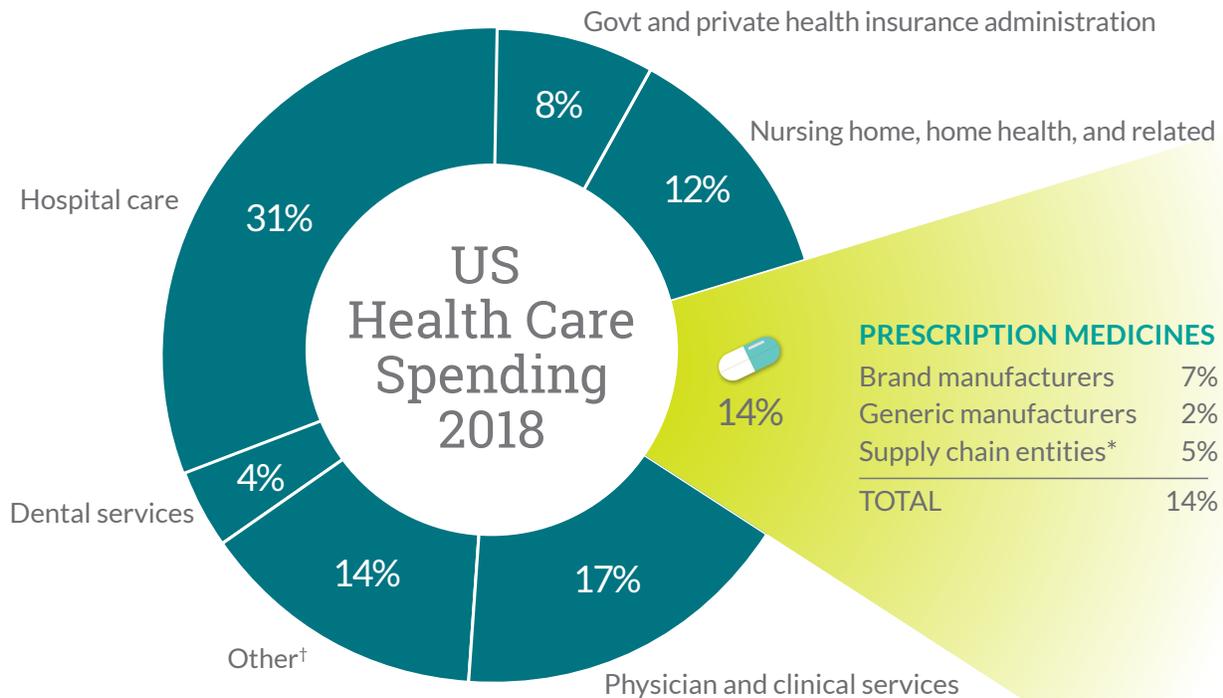


*Total net retail sales including brand medicines and generics

Sources: PhRMA analysis of CMS data^{1,2}; RAND Corporation³; FDA⁴

Spending on All Prescription Medicines Is a Small Share of Total US Health Care Spending

Prescription medicines, whether picked up at a pharmacy or administered in a physician’s office or hospital, account for about 14% of total annual health care spending. Half of this total goes to brand manufacturers, with the rest going to generic manufacturers and the supply chain.



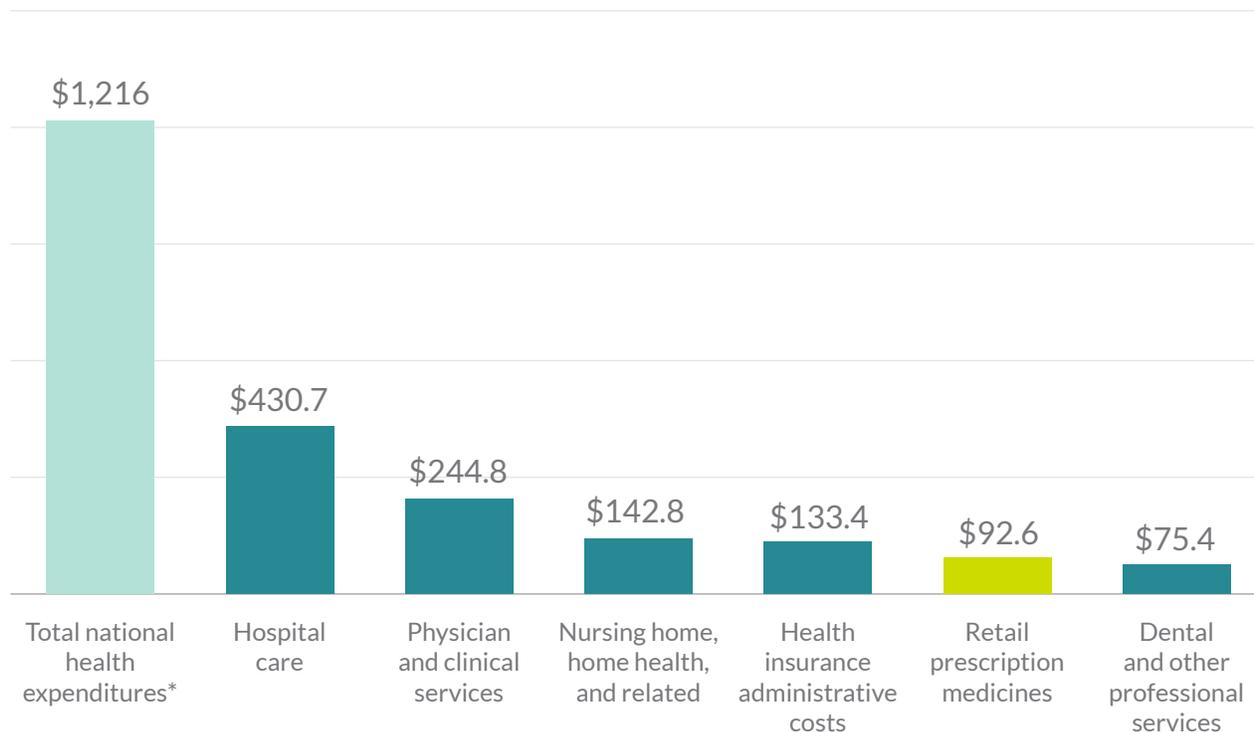
*Supply chain entities include wholesalers, pharmacies, pharmacy benefit managers (PBMs), hospitals, and other health care providers.

†Other includes expenditures for Other Professional Services, Nondurable Medical Products, Durable Medical Equipment, Public Health Activity, Research, Structures, and Equipment.

Sources: PhRMA analysis of CMS data⁵; Altarum Institute⁶; Berkeley Research Group⁷

Retail Prescription Medicine Spending Contributed Less Than One-Tenth of Total Health Care Spending Growth in the Past Decade

Cumulative Spending Growth Over 10 Years (in Billions), 2010-2019

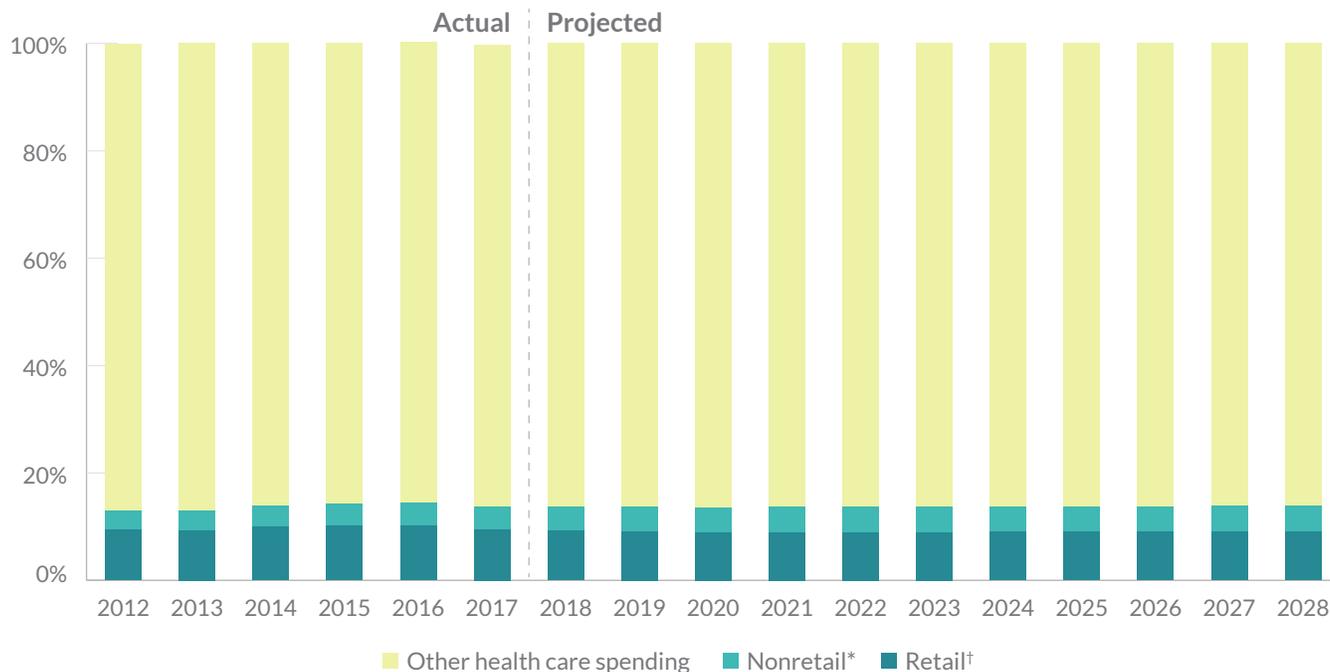


*Listed categories do not sum to Total National Health Expenditures. Not all categories are shown.

Sources: PhRMA analysis of CMS data^{8,9}

Prescription Medicines Are Expected to Account for a Stable Share of Total Health Care Expenditures Through the Next Decade

US Health Care Expenditures Attributable to Retail and Nonretail Prescription Medicines, 2012-2028



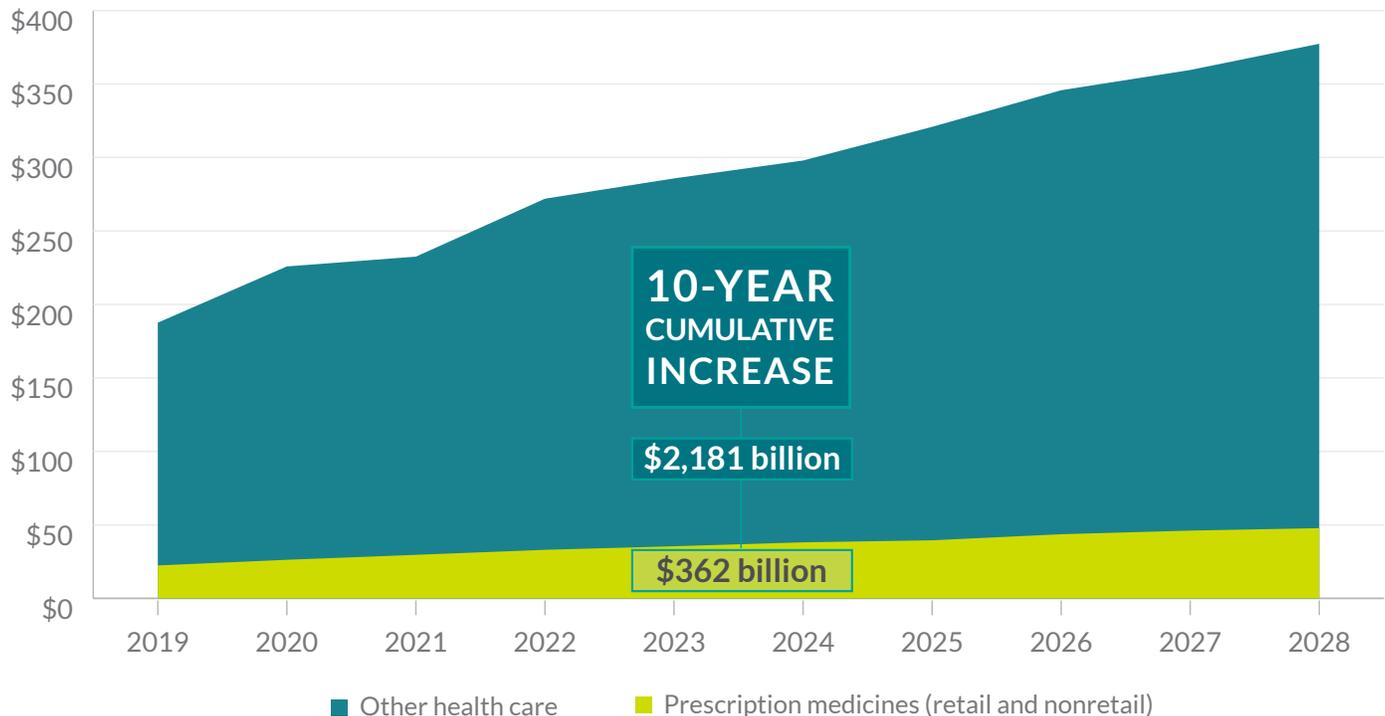
*Nonretail prescription medicines are those purchased through physicians' offices, clinics, and hospitals and are typically administered to the patient by the provider.

†Retail prescription medicines are those filled at retail pharmacies or through mail service.

Source: Altarum Institute¹⁰

Cumulative Spending Growth for Other Health Care Will Be More Than 6 Times That of Medicines Through the Next Decade

Projected Cumulative Growth in Spending (in Billions), 2019-2028

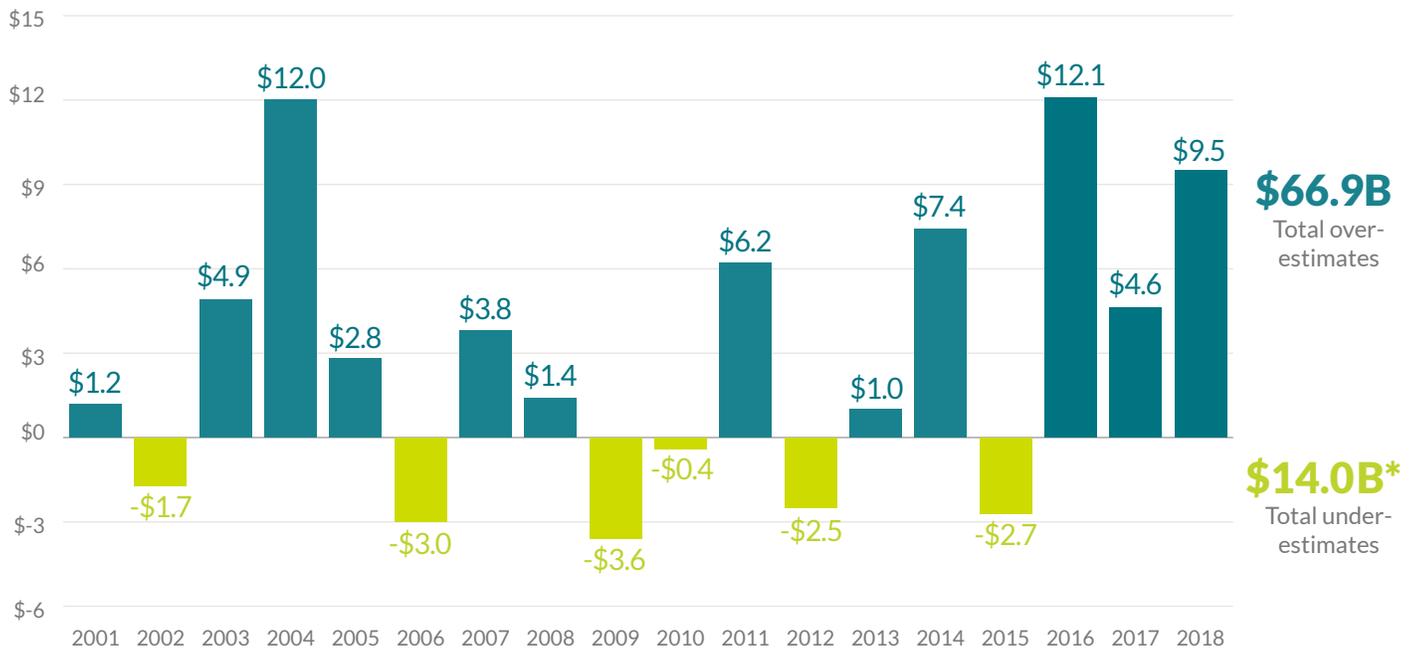


Sources: PhRMA analysis of CMS data¹¹; PhRMA analysis of Altarum Institute data¹²

Medicine Spending Projections Often Overestimate Actual Spending

Centers for Medicare & Medicaid Services (CMS) actuaries annually publish estimates of future retail prescription medicine spending. However, two-thirds of the time, projections made by CMS are overestimated by \$1 billion or more compared to actual spending amounts published just 1 year later.

Difference Between CMS Projected and Actual Retail Medicine Spending (in Billions), 2001-2018

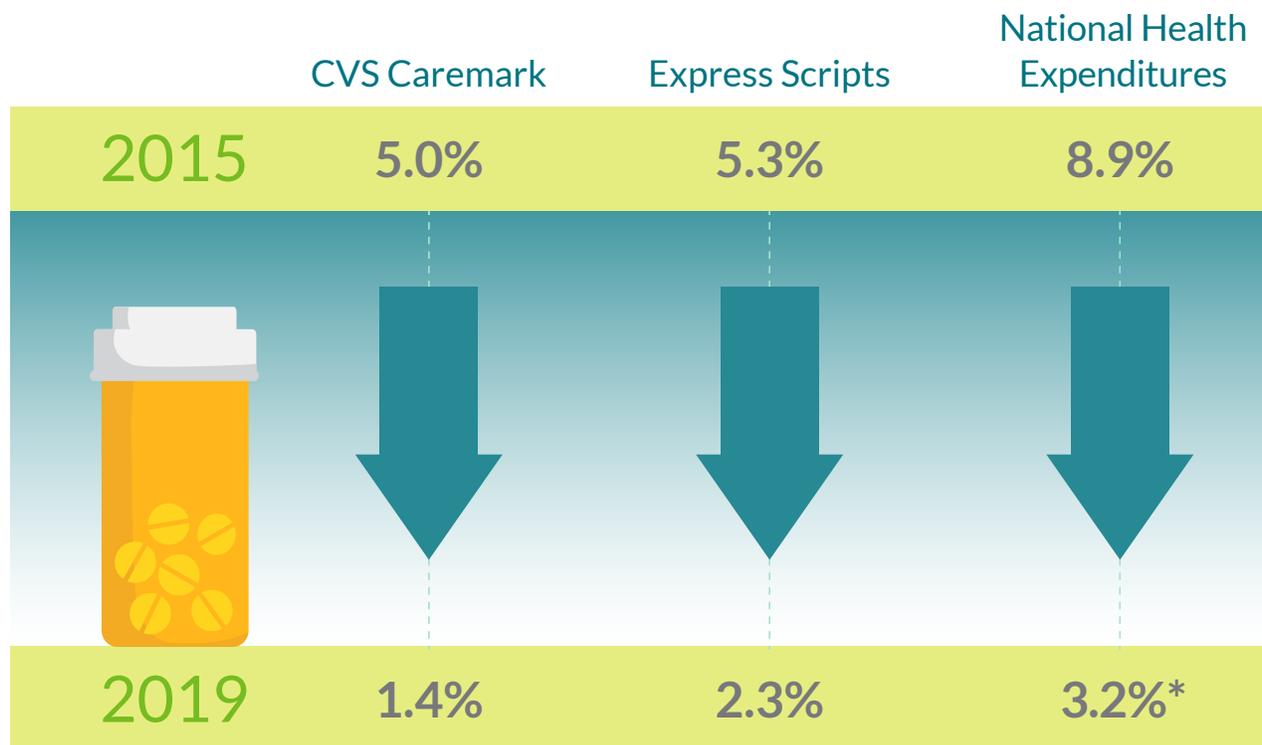


*Figures may not sum due to rounding.

Sources: PhRMA analysis of CMS data^{13,14}

Pharmacy Benefit Managers (PBMs) and Government Actuaries Report Slowing Growth in Medicine Spending

Annual Growth in Net Retail Prescription Medicine Spending



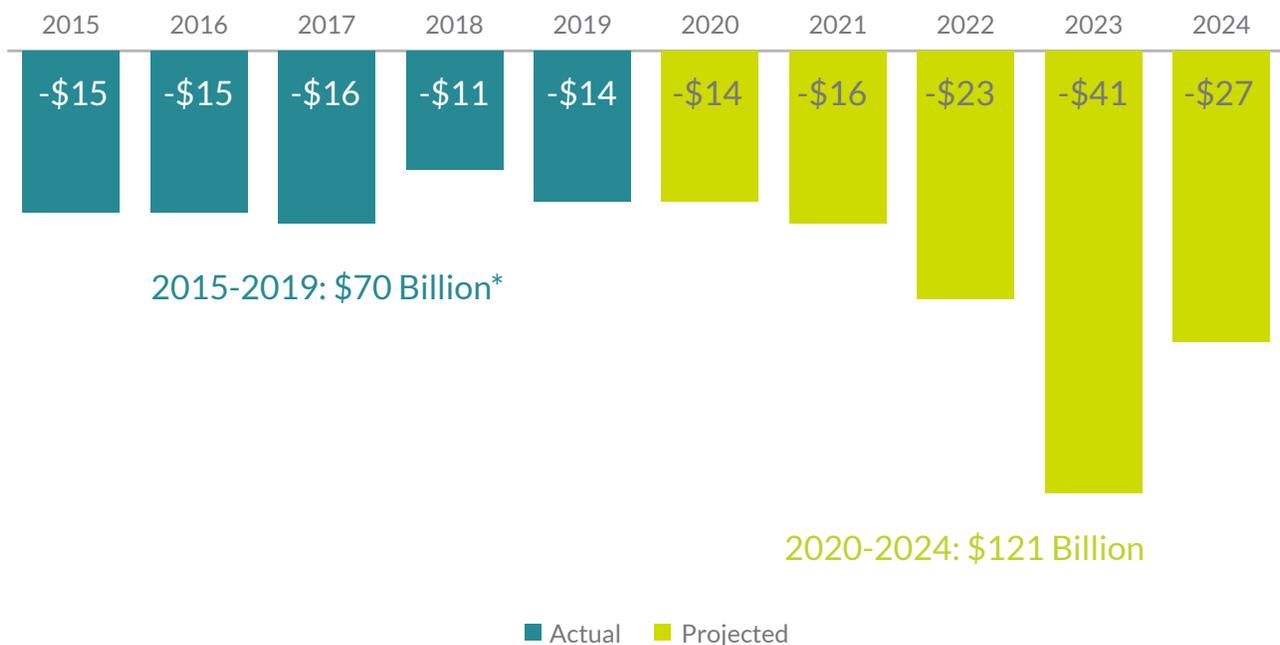
*Projected

Sources: CVS Health^{15,16}; Express Scripts^{17,18}; CMS^{19,20}

Competition From Generics and Biosimilars Is Expected to Reduce US Brand Sales by \$121 Billion From 2020 to 2024

The projected savings from new generics and biosimilars in the coming years are expected to surpass the large-scale savings observed in recent years.

Lower Brand Invoice Spending Due to Loss of Exclusivity (in Billions), 2015-2024



*Figures may not sum due to rounding.

Source: IQVIA Institute²¹

Nearly Half of Spending on Brand Medicines Went to the Supply Chain and Others in 2018

Although total brand medicine spending at the point of sale increased between 2013 and 2018, the share of spending retained by pharmaceutical manufacturers declined by 12.5 percentage points.

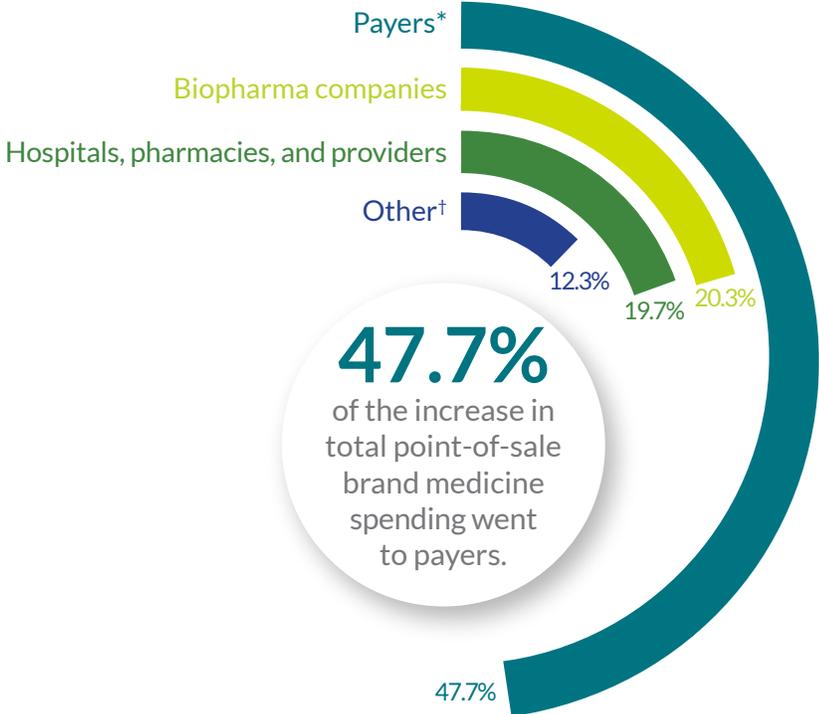
Percentage of Total Spending on Brand Medicines Retained by Manufacturers and Other Entities, 2013-2018



Source: Berkeley Research Group²²

More Than Three-Quarters of the Growth in Brand Medicine Spending Went to Payers, Hospitals, and Others—Not to Biopharmaceutical Companies

Share of Total Brand Spending Growth Received, 2015-2018



From 2013 to 2018, the amount of spending on brand medicines retained by **HOSPITALS, PHARMACIES, and PROVIDERS** doubled.

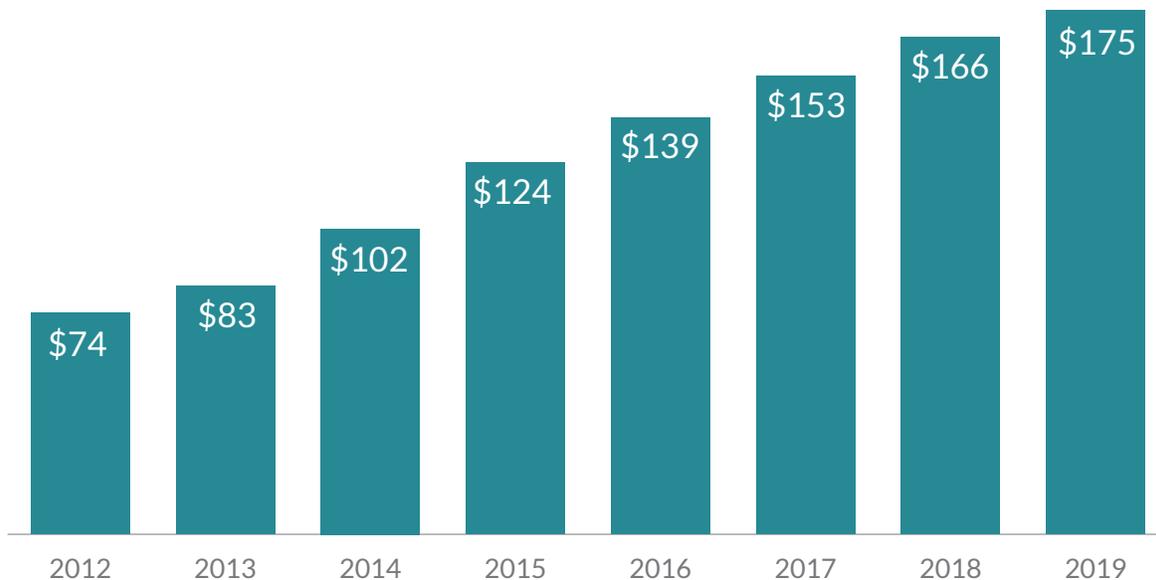
*Payers include health plans, pharmacy benefit managers (PBMs), the federal and state governments, and employer groups.
 †Other includes wholesaler margin, patient cost sharing assistance, excise fees, and group purchasing organization administrative fees.

Source: Berkeley Research Group²³

Manufacturers' Gross-to-Net Reductions Have More Than Doubled Since 2012

Rebates and discounts provided by manufacturers to government, private payers, pharmacy benefit managers (PBMs), and others totaled \$175 billion in 2019.

Total Value of Pharmaceutical Manufacturers' Gross-to-Net Brand Medicine Reductions (in Billions), 2012-2019



GROSS-TO-NET REDUCTIONS

are defined as “rebates, off-invoice discounts, copay assistance, price concessions, and other reductions like distribution fees, product returns, the 340B Drug Pricing Program, and more.”

Source: Drug Channels Institute²⁴

Patients Often Do Not Directly Benefit From Negotiated Rebates and Discounts Paid by Manufacturers

Prices paid by wholesalers, pharmacies, pharmacy benefit managers (PBMs), and health plan sponsors vary and are determined by negotiations between stakeholders, each with varying degrees of negotiating power.

Flow of Payment for a \$400 Insulin Prescription for a Patient in the Deductible Phase

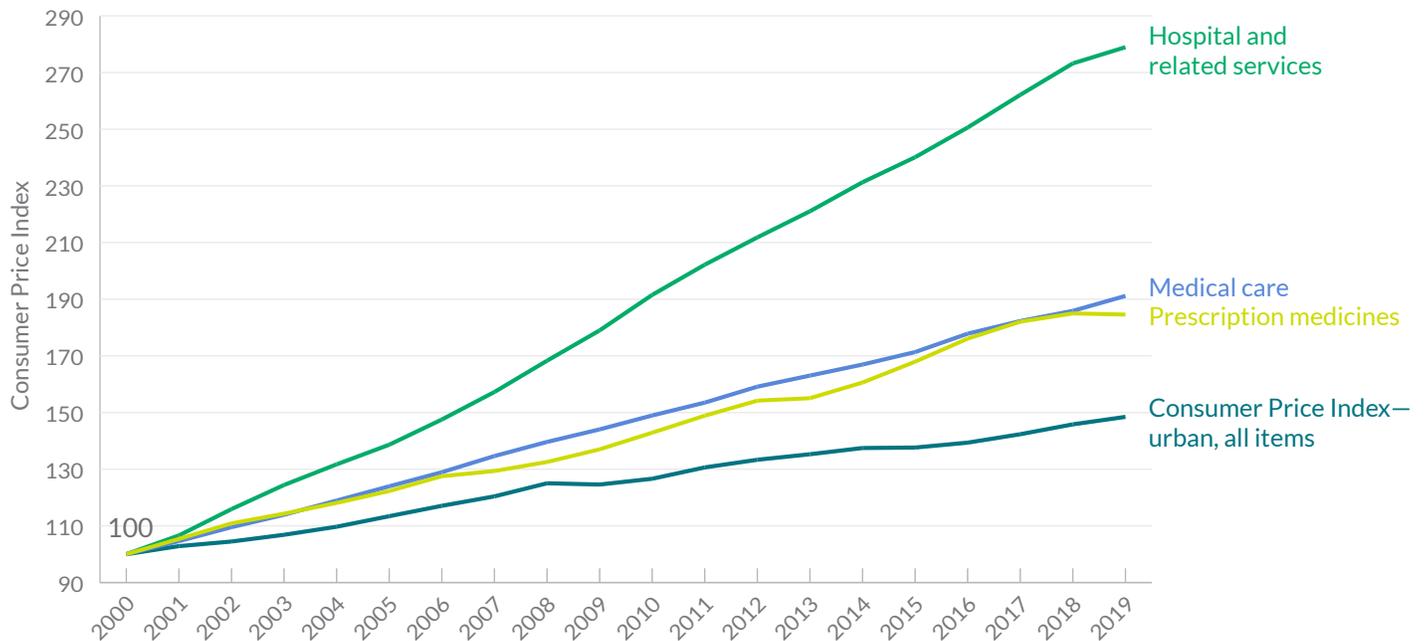


This graphic is illustrative of a hypothetical product with a wholesale acquisition cost (WAC) of \$400 and an average wholesale price (AWP) of \$480. It is not intended to represent every financial relationship in the marketplace. The payment amounts do not add up to \$400 due to markups and discounts along the supply chain.

Source: PhRMA²⁵

Growth in Prescription Medicine Prices Has Been in Line With Other Health Care Price Growth

Average Price Levels, Selected Goods and Services, 2000-2019

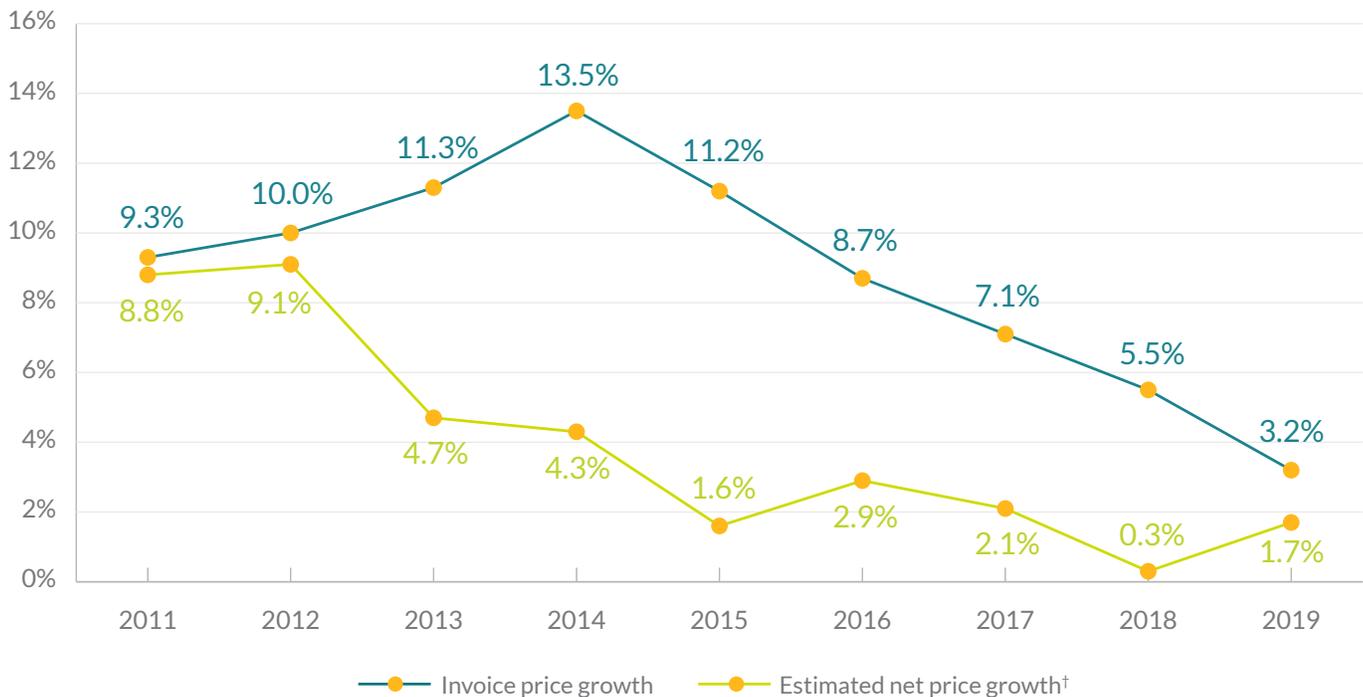


Source: PhRMA analysis of Bureau of Labor Statistics data²⁶

Net Price Growth of Medicines Is Lower Than List Price Growth and Has Been in Line With Inflation Over the Last 5 Years

Commonly reported invoice (or list) prices are higher than what payers ultimately pay for medicines.

Average Price Growth for Brand Medicines, 2011-2019*



*Includes protected brand medicines only (ie, brand medicines without generic or biosimilar versions available in the year indicated).

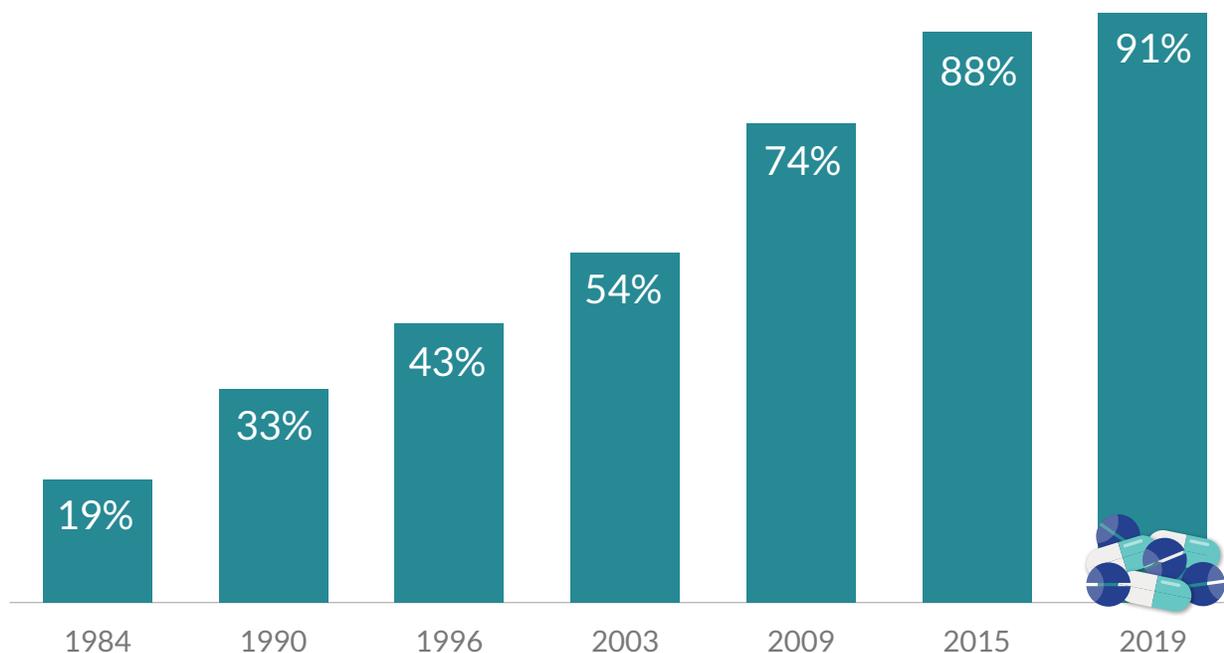
†Estimated net price growth reflects impact of off-invoice rebates and discounts provided by manufacturers.

Source: IQVIA Institute²⁷

Ninety-One Percent of All Medicines Dispensed in the United States Are Generics

Between 2010 and 2019, use of generics and biosimilars saved nearly \$2.2 trillion in US health care spending.²⁸

Generic Share of Prescriptions Filled, 1984-2019^{29,30*}

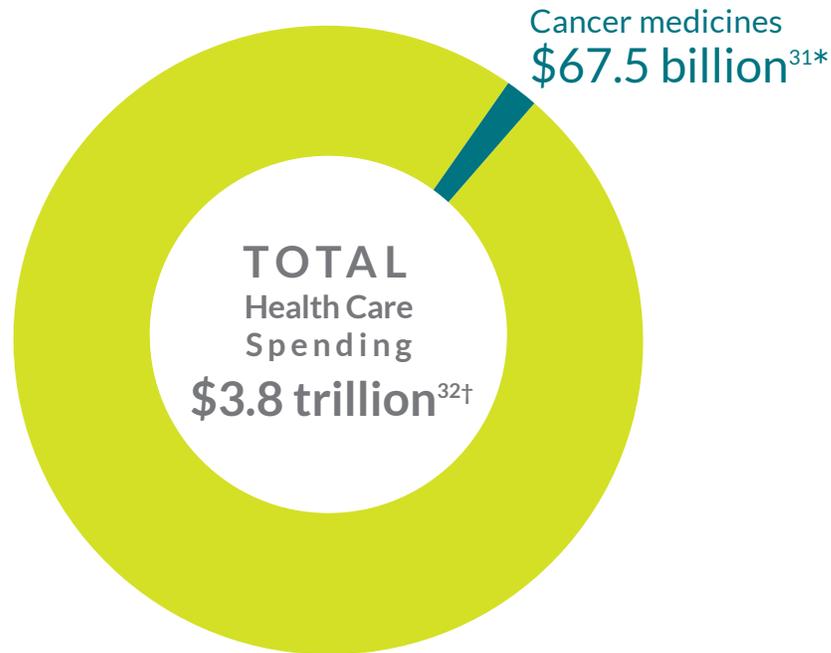


*Generic share includes generics and branded generics.

Sources: Association for Accessible Medicines²⁸; IQVIA Institute²⁹; Drug Channels Institute³⁰

Spending on Cancer Medicines Represents Less Than 2% of Overall Health Care Spending

Cancer Medicines as a Portion of Total US Health Care Spending, 2019



*Cancer medicine spending reflects invoice spending, which does not account for rebates and discounts.
†Projected

Sources: IQVIA Institute³¹; CMS³²

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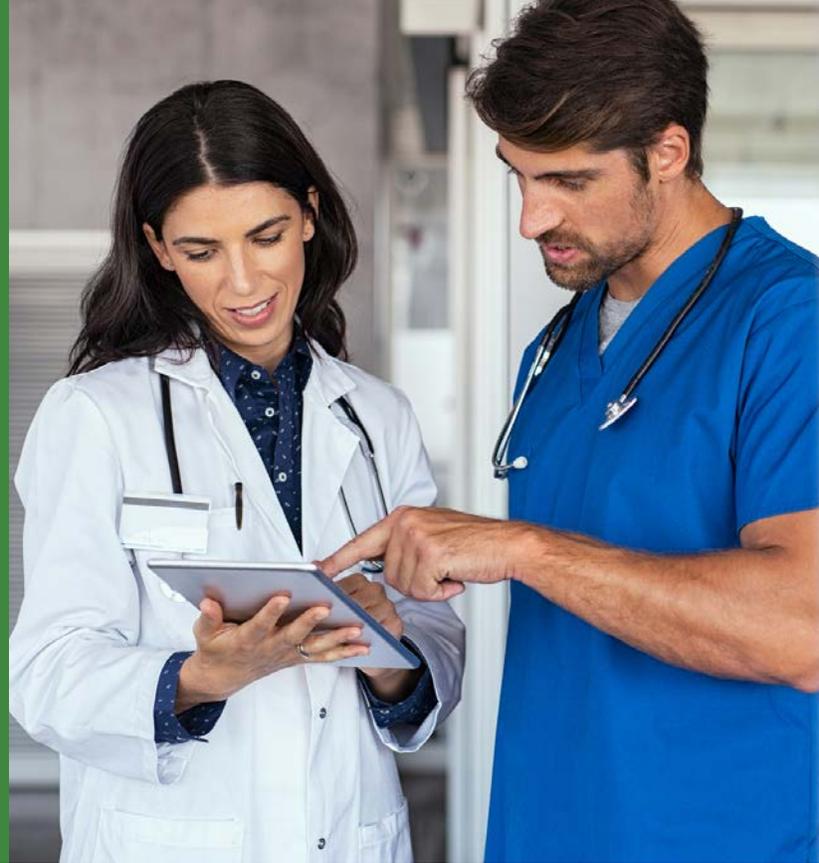
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6

OUTCOMES AND SAVINGS

Overcoming Gaps in Treatment,
Improving Outcomes, and
Reducing Costs Through
Better Use of Medicines



Undertreatment of complex and chronic conditions as well as suboptimal use of prescribed medicines are significant public health problems, costing the US economy hundreds of billions of dollars each year. Medicines help patients live healthier lives and reduce the need for costly health care services such as emergency department visits, hospital stays, surgeries, and long-term care. An ever-growing body of evidence demonstrates that improved use of prescribed medicines can result in better health outcomes, lower costs for other health care services, and increased worker productivity.

The Human and Economic Costs of Chronic Disease

More than 1 million lives could be saved annually through better treatment and prevention of chronic disease. It costs almost \$357 billion per year to treat Americans who have just one chronic condition.¹

MEDICARE HOSPITAL READMISSIONS



99% are beneficiaries with **two or more** chronic conditions²

ESTIMATED COST

\$42 trillion across all payer types³



Costs of **CHRONIC DISEASE**

AMERICANS

191 million have at least one
75 million have multiple³

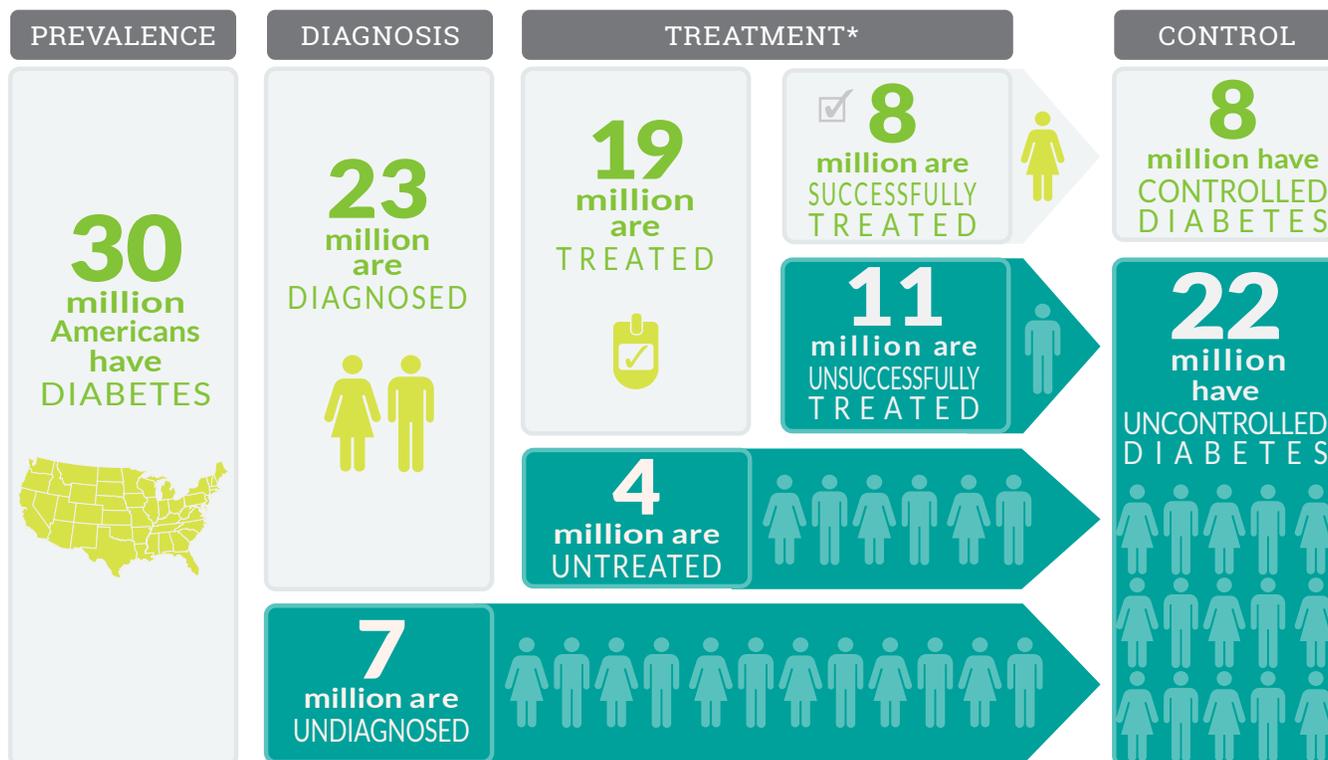


5% of the population accounts for
50% of US health care spending⁴

Sources: Bipartisan Policy Center¹; CMS²; IHS³; Agency for Healthcare Research and Quality⁴

Diabetes: An Example of Underdiagnosis and Undertreatment

Uncontrolled diabetes can lead to kidney failure, amputation, blindness, and stroke. Care for people with diagnosed diabetes accounts for 1 in 4 health care dollars in the United States.⁵



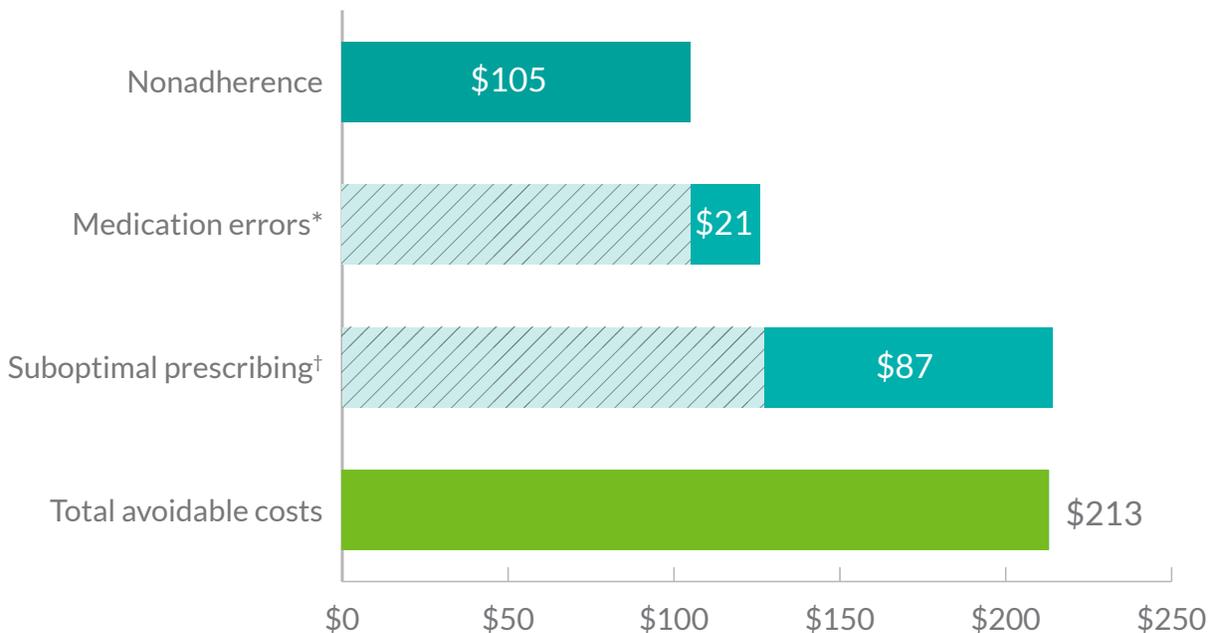
*Treatment includes blood sugar control (medicines, diet, and exercise) and testing to prevent complications.

Sources: American Diabetes Association⁵; IHS Life Sciences analysis of CDC data⁶

Potential Savings From Better Use of Medicines

Better use of medicines could eliminate up to \$213 billion in US health care costs annually, which represents 8% of the nation's health care spending.

Sources of Avoidable Annual US Health Care Costs, 2012 (in Billions)



*Category includes medication errors (\$20 billion) and mismanaged polypharmacy (\$1 billion).

†Category includes untimely medicine use (\$40 billion), inappropriate antibiotic use (\$35 billion), and suboptimal generic use (\$12 billion).

Source: IMS Institute for Healthcare Informatics⁷

Lowering Cost Sharing for Seniors at the Pharmacy Counter Can Generate Medicare Savings

Sharing a portion of negotiated manufacturer rebates directly with patients could improve medicine adherence and result in savings for seniors and Medicare in Part D.

BENEFITS OF SHARING NEGOTIATED REBATES:



Lower beneficiary out-of-pocket spend by **\$350 per year**



Save Medicare nearly **\$1,000 per year** for every senior taking diabetes medicine



Reduce total health care spending by approximately **\$20B over 10 years**

Source: IHS Markit⁸

Better Use of Medicines Yields Significant Health Gains by Avoiding the Need for Other Medical Services

Due to a growing body of evidence, in 2012 the Congressional Budget Office (CBO) began recognizing reductions in other medical expenditures associated with an increased use of medicines in Medicare.



Pharmaceuticals have the effect of improving or maintaining an individual's health . . . adhering to a drug regimen for a chronic condition such as diabetes or high blood pressure may prevent complications . . . taking the medication may also avert hospital admissions and thus **reduce the use of medical services** [bold added].”

CBO⁹



Since the CBO announcement, the evidence has continued to develop, broadening the potential for cost offsets in the health care system.

CHRONIC DISEASES

Medicare savings due to better use of medicines may be **3 to 6 times greater** than estimated by the CBO for seniors with common chronic conditions, including heart failure, diabetes, and hypertension.¹⁰



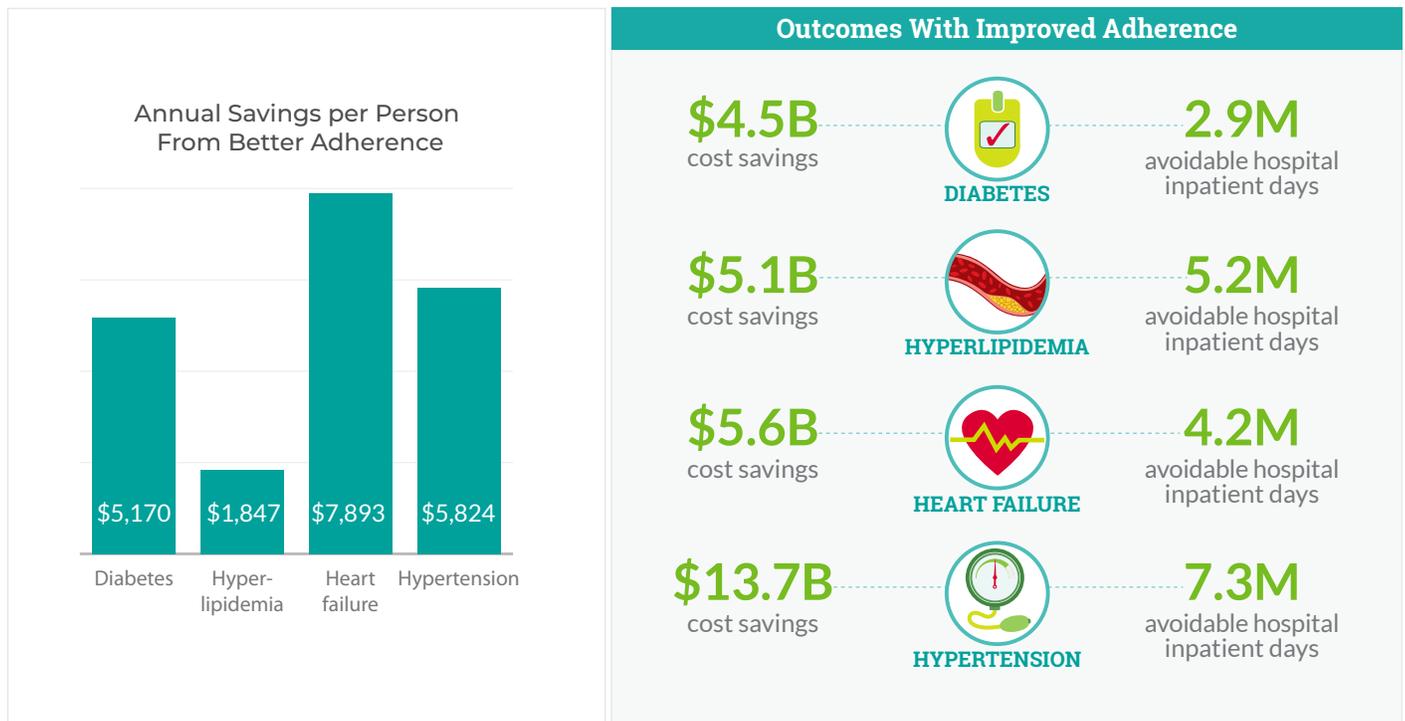
MEDICAID

Increased use of medicines is associated with **reductions** in Medicaid expenditures from avoided use of inpatient and outpatient services.^{11,12}

Sources: CBO⁹; Roebuck MC¹⁰; Roebuck MC et al^{11,12}

Improved Medicine Use Can Lead to Savings in Medicare

Between 20% and 40% of Medicare beneficiaries with common chronic diseases are not adherent to their medicines. Billions of dollars in cost savings from avoided hospital stays can result from improved adherence.

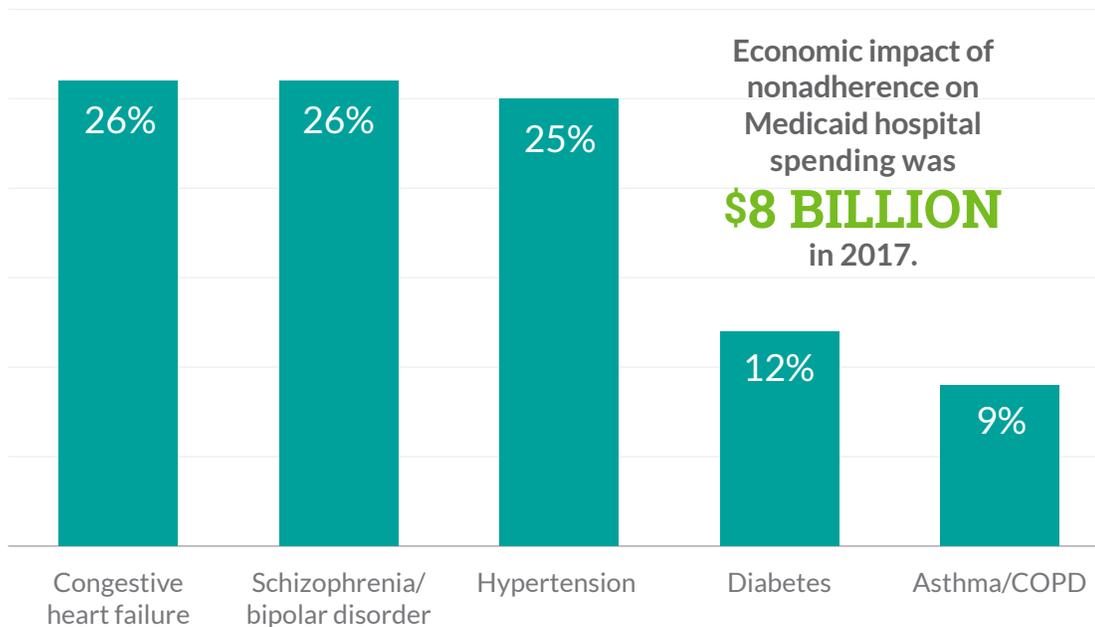


Source: Lloyd JT et al¹³

Better Adherence Generates Savings in Medicaid

Optimal adherence to medicines for a range of chronic conditions leads to reductions in hospitalizations for many patients enrolled in Medicaid.

Reductions in Hospitalizations Due to Medication Adherence*



*Results apply to Medicaid populations that are not blind or disabled.

Source: Roebuck MC et al¹⁴

Improving Access to Treatment Could Reduce the Clinical and Economic Impact of Addiction

The economic impact of the opioid crisis in the United States was estimated to be \$504 billion in 2015.¹⁵

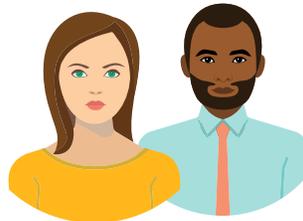
Medication-assisted treatment (MAT) is an evidence-based approach for the treatment of **opioid use disorder** that pairs behavioral therapy with medicines that block the effects of opioids and/or mitigate the symptoms of opioid withdrawal.

Doubling access to MAT in the commercial market alone over the next 15 years could¹⁶:

Prevent up to
6.1M
OVERDOSES



Save as many as
805K LIVES



Save the health care
system as much as
\$645B



Sources: Hagemeyer, NE¹⁵; IHS Markit¹⁶

Recent Studies Show Significant Value From Better Use of Medicines

Patients with a range of diseases could offset health care spending by exercising better adherence.



PARKINSON'S DISEASE

Health care savings of **up to \$6,300** in fewer than 2 years can be achieved among patients with Parkinson's who continually stay on therapy.¹⁷



MULTIPLE SCLEROSIS

Initiation of therapy is associated with **reductions of up to \$5,700** in medical costs, driven by decreased use of outpatient services and inpatient hospital stays.¹⁸



CYSTIC FIBROSIS

Among children with cystic fibrosis, poor medication adherence is associated with more hospitalizations and emergency department visits and an increase of **more than \$14,000** in same-year medical costs compared with children who are highly adherent.¹⁹



LUPUS

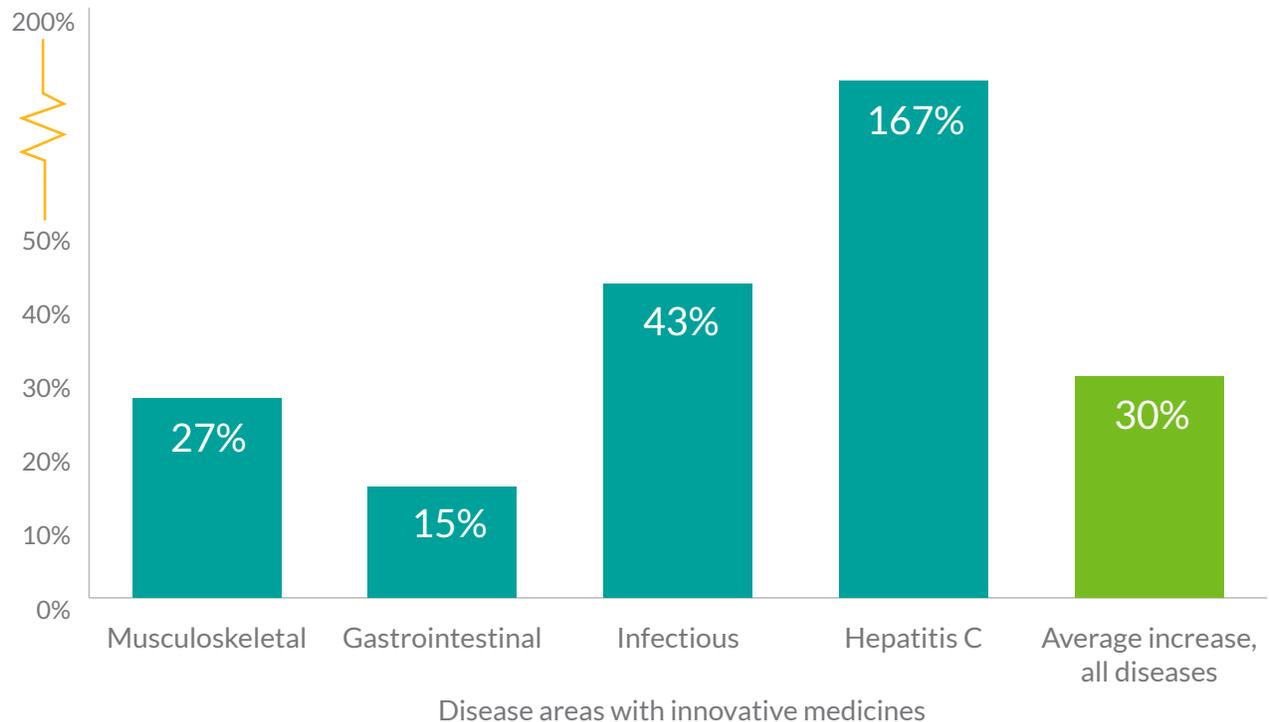
Nonadherence among children on Medicaid who are diagnosed with lupus is associated with a **55% increase** in emergency department use and a **nearly 40% increase** in hospitalizations.²⁰

Sources: Wei YJ et al¹⁷; Nicholas J et al¹⁸; Quittner AL et al¹⁹; Feldman CH et al²⁰

Innovative Medicines Improve Patients' Ability to Work

New drugs increase worker productivity by 4.8 million work days and add \$221 billion in wages per year.

Percent Increase in Worker Productivity Due to Innovative Medicines



Source: Chen AJ et al²¹

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FRESH PRODUCE

LOCALLY GROWN

100% ORGANIC

FARMER

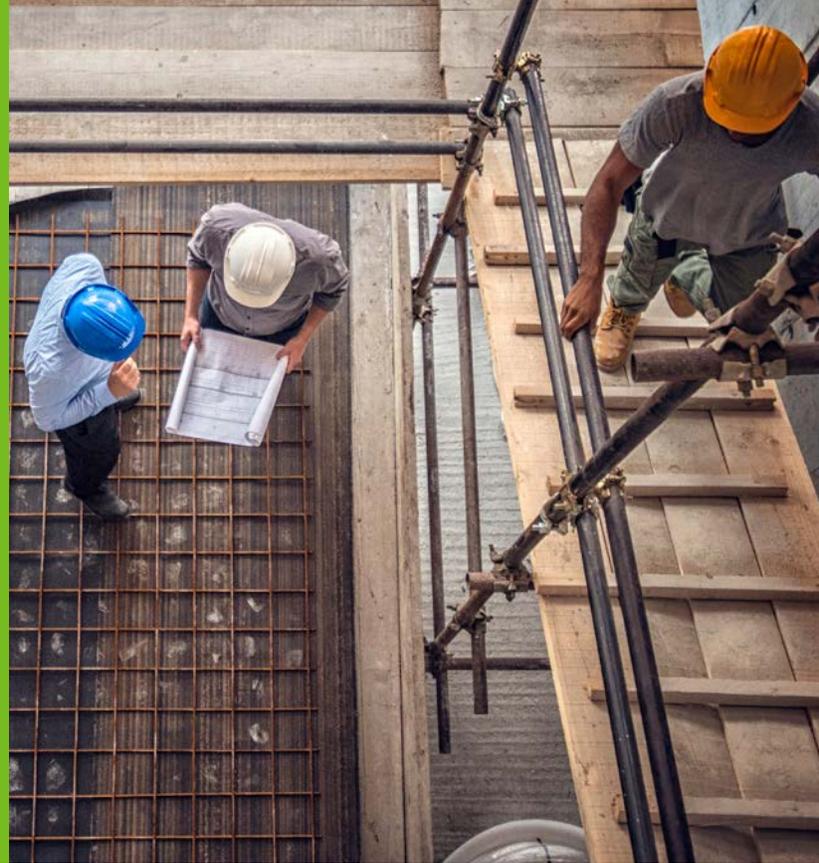
FRUITS & VEGETABLES
FRESH EGGS
MEATS & CHEESE
BREADS & PRESERVES

ORGANIC POTATO
\$4.25/LB

7

ECONOMIC IMPACT

Sustaining and Growing
State and Local Economies

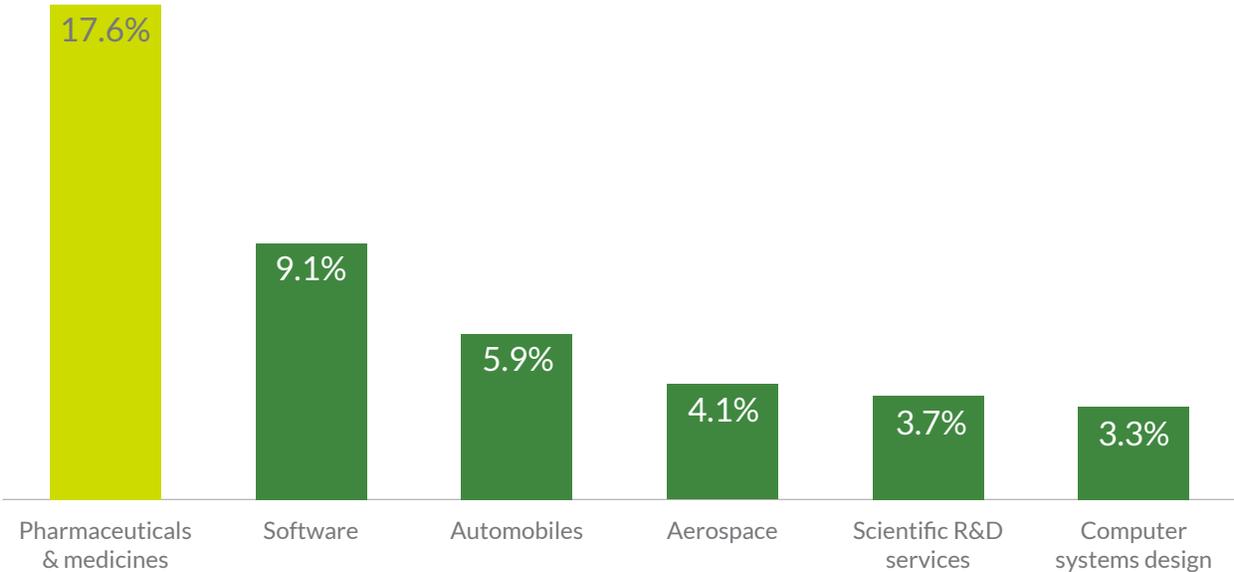


America's biopharmaceutical industry is the foundation for one of the country's most dynamic innovation and business ecosystems. The industry is among the most research and development (R&D)-intensive in the United States, accounting for 1 out of every 6 dollars spent on domestic R&D by US businesses. The industry's large-scale research and manufacturing footprint, along with its attendant supply chain, supports high-quality jobs in communities across the United States. More biopharmaceutical venture capital is invested in startups in the United States than anywhere else in the world, providing an ongoing source of highly skilled jobs aimed at making advances in biopharmaceutical science. However, US leadership in innovation is facing increasing challenges from emerging global competitors seeking to attract and grow a biopharmaceutical presence in their own countries.

The Biopharmaceutical Industry Is the Single Largest Funder of Business R&D in the US

The biopharmaceutical industry accounts for the single largest share of all self-funded R&D, representing 1 out of every 6 dollars (18%) spent on domestic R&D by US businesses. Furthermore, US industry is also the largest global funder of biopharmaceutical R&D, accounting for about half of all R&D investments worldwide.

Share of Total US Business R&D by Industry, 2017*



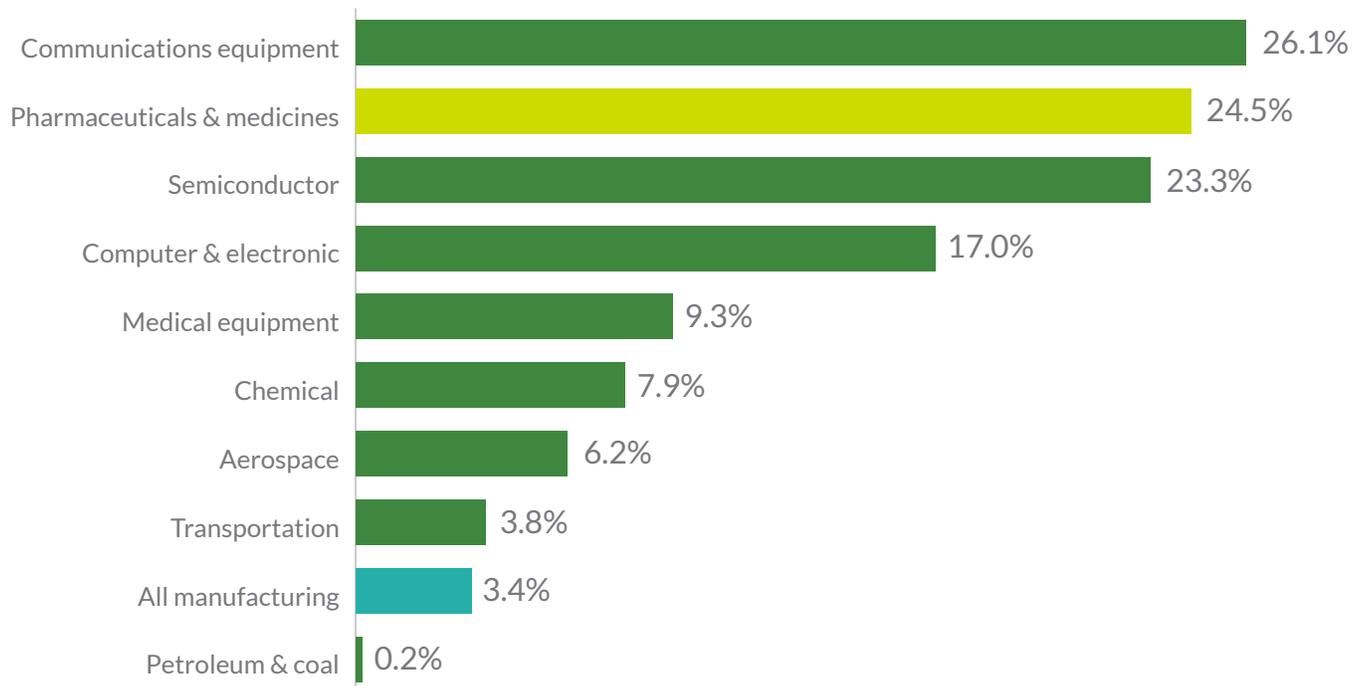
*The remaining 56.3% share of business R&D spending is conducted by other industries, including subsectors of the machinery sector, the computer and electronic products sector, and the electrical equipment, appliance, and components sector.

Source: NDP Analytics¹

The US Biopharmaceutical Sector Is Among the Biggest Investors in R&D Relative to Sales

Biopharmaceutical investments in R&D, as a percentage of sales, are more than 6 times the average for all manufacturing industries, making the sector one of the most R&D-intensive industries.

R&D as a Percentage of Sales by Industry, 2003-2017

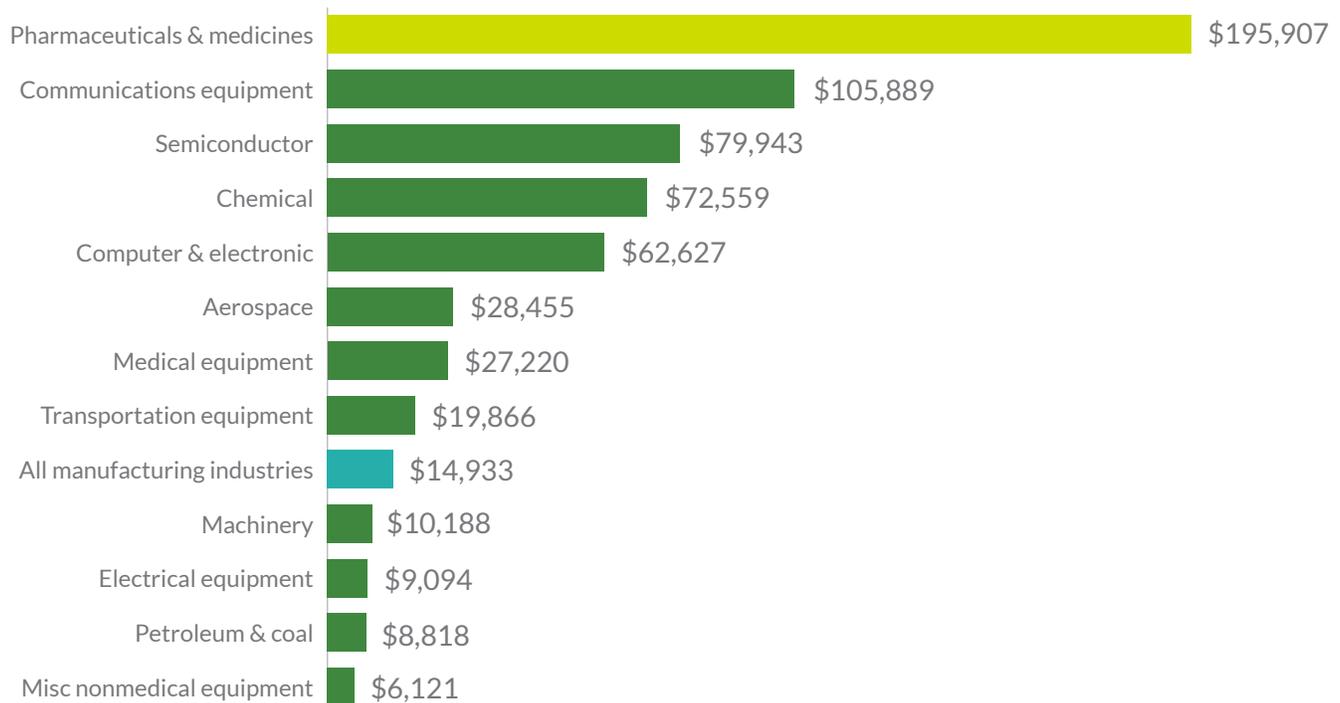


Source: NDP Analytics²

The Biopharmaceutical Industry Invests More R&D Dollars per Employee Than Any Other Industry

On a per employee basis, biopharmaceutical companies invest 13 times more in R&D than the average for manufacturing industries overall.

R&D Expenditures per Employee by Manufacturing Sector and Industry, 2003-2017

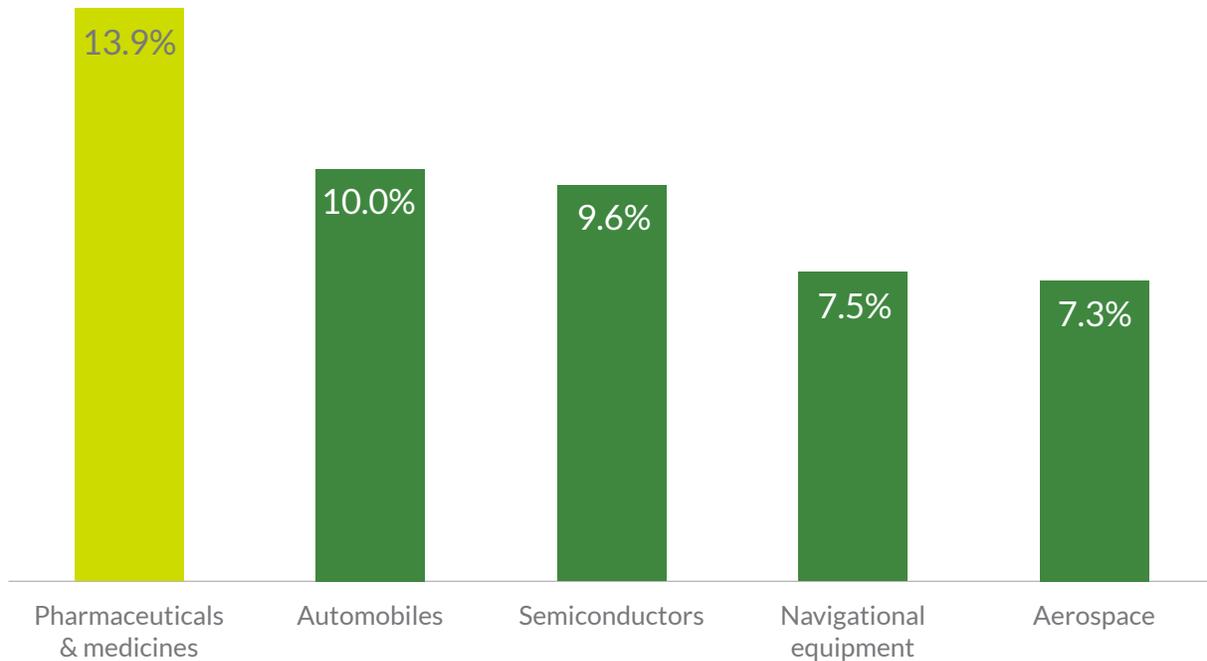


Source: NDP Analytics³

The Biopharmaceutical Industry Employs the Largest Share of All Manufacturing R&D Workers

One out of every 7 R&D workers in the nation's manufacturing industries is employed by the biopharmaceutical industry.

Selected Manufacturing Industries' Share of Total R&D Workers, 2017*



*All other manufacturing sectors account for the remaining 51.7% of the R&D workforce.

Source: NDP Analytics⁴

The Economic Reach of the US Biopharmaceutical Industry

Every biopharmaceutical sector job supports a total of 5 jobs across the economy.



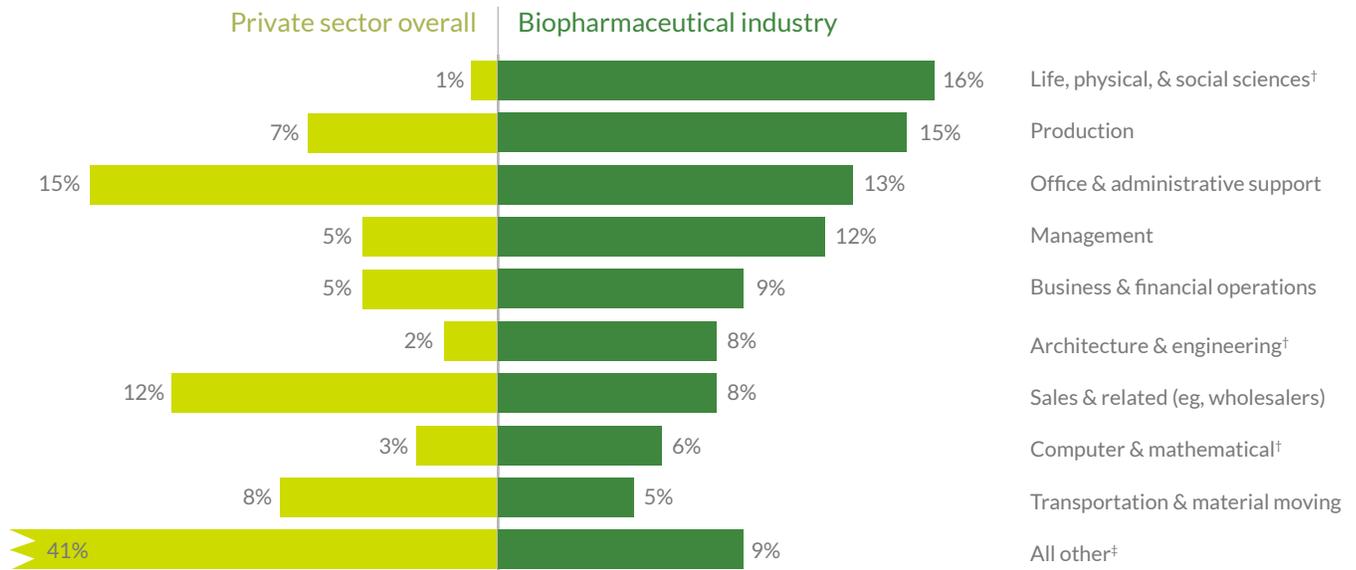
The biopharmaceutical industry supported more than 4 million jobs across the US economy in 2017.

Source: TEconomy Partners⁵

The US Biopharmaceutical Sector Produces High-Quality Jobs Across an Array of Fields

One-third of the jobs in the biopharmaceutical sector are in key STEM (science, technology, engineering, and mathematics) occupations, a far higher share than in the private sector as a whole.

Percentage of Jobs in Sector by Occupation, 2017*



*Column percentages may not add up to 100% due to rounding.

†Indicates a STEM occupation.

‡Other occupations include health care practitioners/techs (2.8% of biopharma industry jobs); installation/maintenance/repair (2.5%); arts/design/entertainment/sports/media (0.9%); building & grounds cleaning/maint (0.6%); legal (0.4%); health care support (0.4%); construction/extraction (0.3%); educ/training/library science (0.3%); protective services (0.2%); community/social services (0.2%); personal care & service (0.1%); farming/fishing/forestry (0.1%); and food prep/serving (0.1%).

Source: TEconomy Partners⁶

US Biopharmaceutical Exports Have Grown

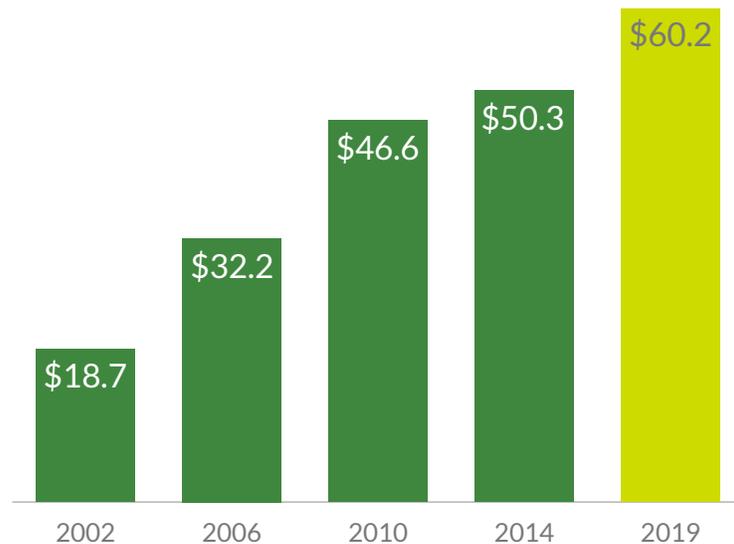
Biopharmaceutical exports have tripled since 2002, accounting for about 4% of all US exports in 2019.



Pharmaceuticals rank as one of the top exporting sectors for IP-intensive industries in the United States.”

International Trade Administration⁷

US Biopharmaceutical Goods Exports (in Billions)⁸

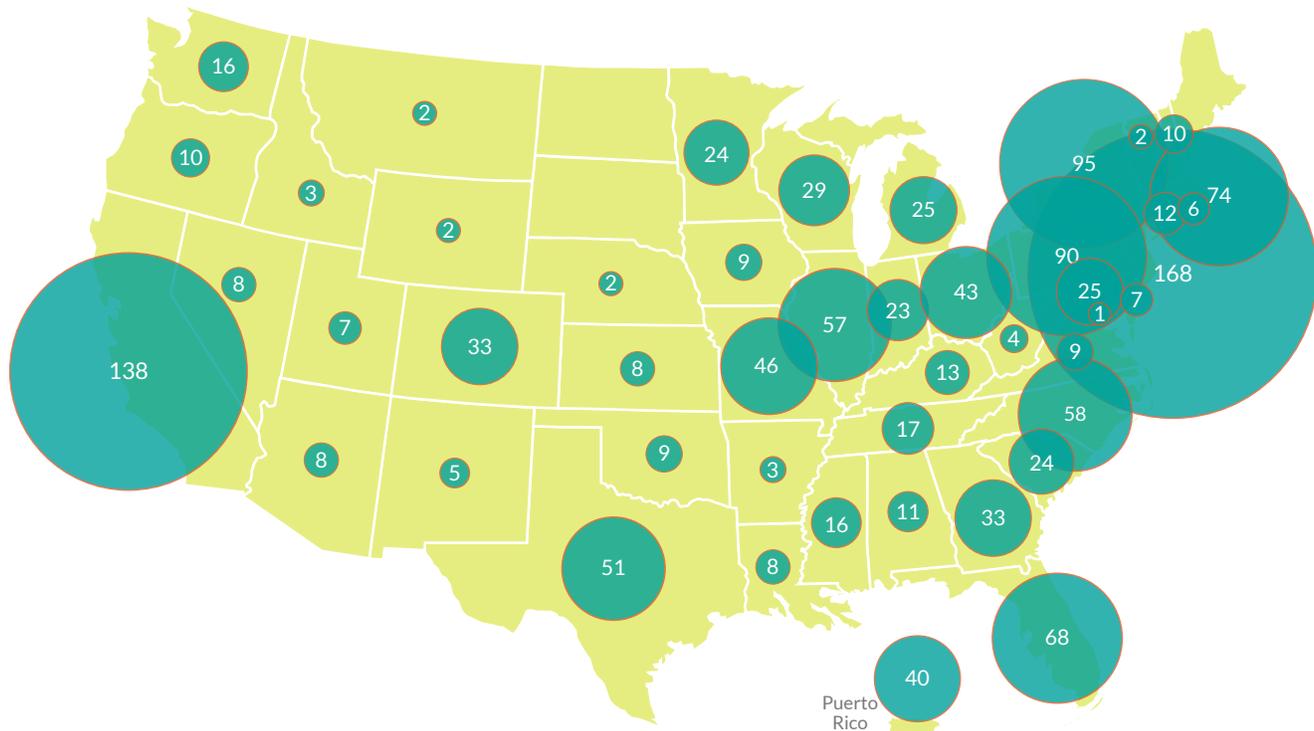


Sources: ITA⁷; PhRMA analysis of US Census Bureau data⁸

The Biopharmaceutical Sector's Extensive US Manufacturing Footprint

More than 1,300 manufacturing plants involved in the production of human-use medicines are located in 45 US states and Puerto Rico. Biopharmaceutical companies are building the plants to make cutting-edge therapies of the future in the United States.

Biopharmaceutical Manufacturing Facilities by State/Territory (January 2020)^{9,10}



Sources: NDP Analytics analysis of FDA DECRS data⁹; Hargreaves B¹⁰

Building a Pharmaceutical Manufacturing Supply Chain: A Complex and Lengthy Venture

Biopharmaceutical companies begin setting up the highly regulated manufacturing supply chain for a medicine years before the medicine is even approved for use by patients. Quality control and system resilience are built into every aspect of the process.

DEVELOP INITIAL MANUFACTURING PLANS

Planning stage addresses all aspects of operations:

- Raw materials sourcing
- Production
- Packaging
- Labeling
- Storage
- Distribution
- Tracking systems
- Quality control, testing, and compliance systems

SCALE UP MANUFACTURING PROCESS

Companies must go from test scale manufacturing used for clinical trials to commercial scale production, while ensuring strict quality controls.



It can cost **\$1 billion to \$2 billion** and take **5 to 10 years** to set up a new manufacturing facility, and even longer to onshore an entire manufacturing network.

1

BUILD THE SUPPLY CHAIN, INCLUDING QUALIFIED SUPPLIERS

Companies must ensure supply chains for all active and inactive ingredients. As a matter of course, manufacturers have business continuity plans to ensure resiliency in the event of disruption.

2

3

COMPLY WITH REGULATIONS AND SUBMIT FOR INSPECTIONS

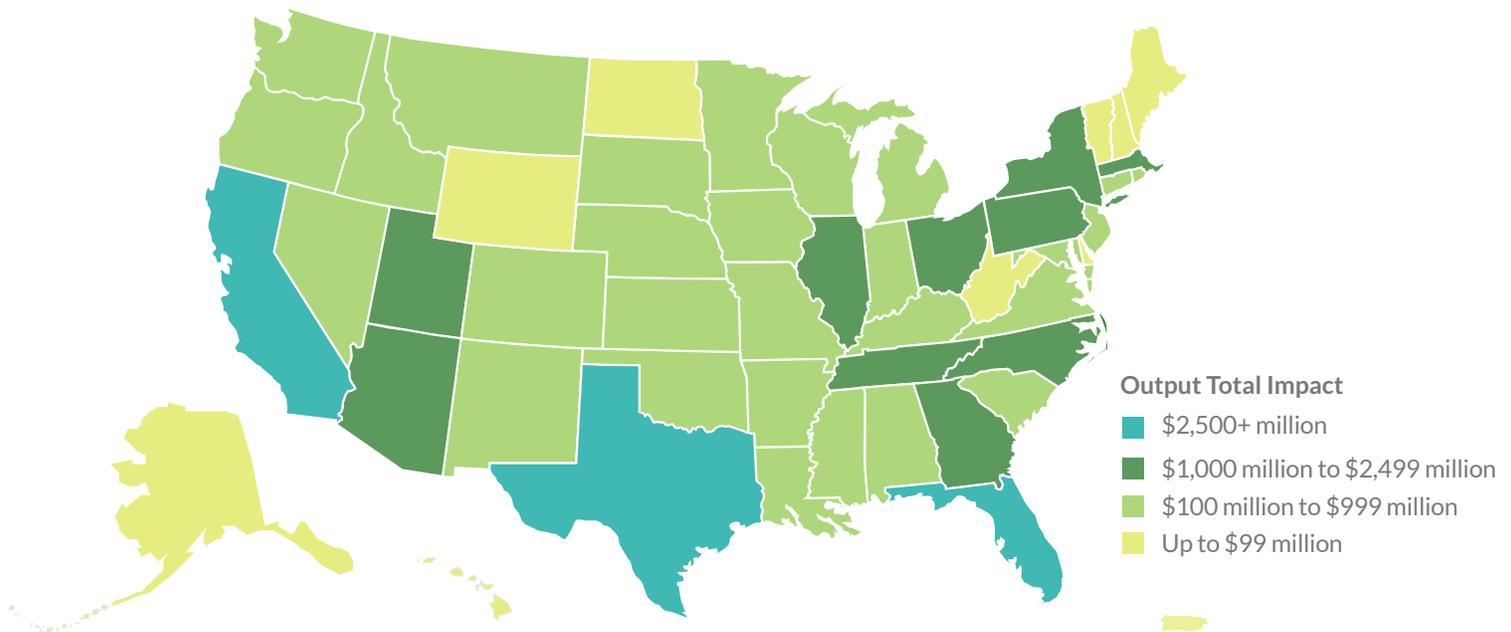
- The FDA reviews manufacturing process as part of drug application review.
- Facility registration with the FDA includes finished drug and active pharmaceutical ingredients sites.
- Pre-approval and surveillance inspections help ensure compliance.
- Laws and regulations on manufacturing and product security help ensure quality.

4

Industry-Sponsored Clinical Trials Contribute Significant Value Across the Nation

In 2017, the biopharmaceutical industry sponsored more than 4,500 clinical trials of medicines in the United States, involving 920,000 participants and supporting \$42 billion in economic activity across all 50 states, the District of Columbia, and Puerto Rico.*

Estimated Total Economic Impact of Industry-Sponsored Clinical Trials Activity Across the US, 2017



*Estimates reflect only those activities occurring at clinical trial sites and exclude more centralized cross-site functions such as coordination and data analysis. Also excluded are nonclinical R&D activities such as basic and preclinical research and the significant economic contribution from non-R&D activities of the industry such as manufacturing and distribution.

Source: TEconomy Partners¹²

States Are Increasingly Targeting the Biopharmaceutical Industry in Their Economic Development Plans

Recognizing the broad economic impact of the biopharmaceutical industry, states across the country are adopting a range of policies and programs to attract and grow the industry within their borders.

Common policies and programs that states are pursuing include:



Adopting comprehensive, targeted strategies for life science industry development



Accelerating innovation through entrepreneurial development programs



Building research capacity and infrastructure



Increasing the availability of financial capital for life science development



Building advanced manufacturing capabilities



Establishing economic incentives for life science innovation



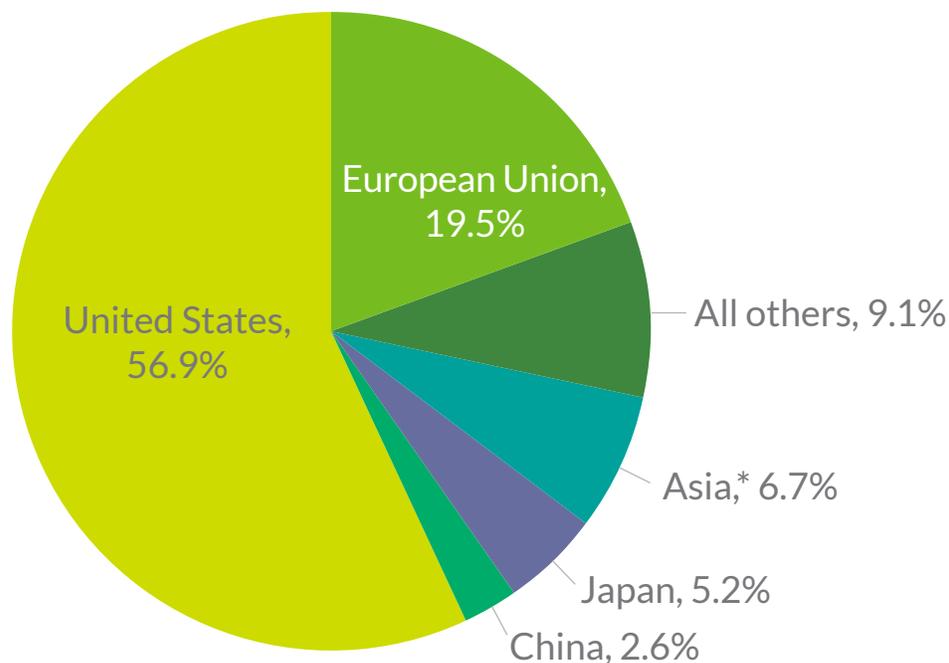
Advancing the STEM talent pipeline

Source: TEconomy Partners¹³

The United States Leads in Biopharmaceutical Intellectual Property

More than half of the intellectual property related to new medicines was created in the United States.

US Patents Granted in Pharmaceuticals by Region/Country of Inventor, 2016



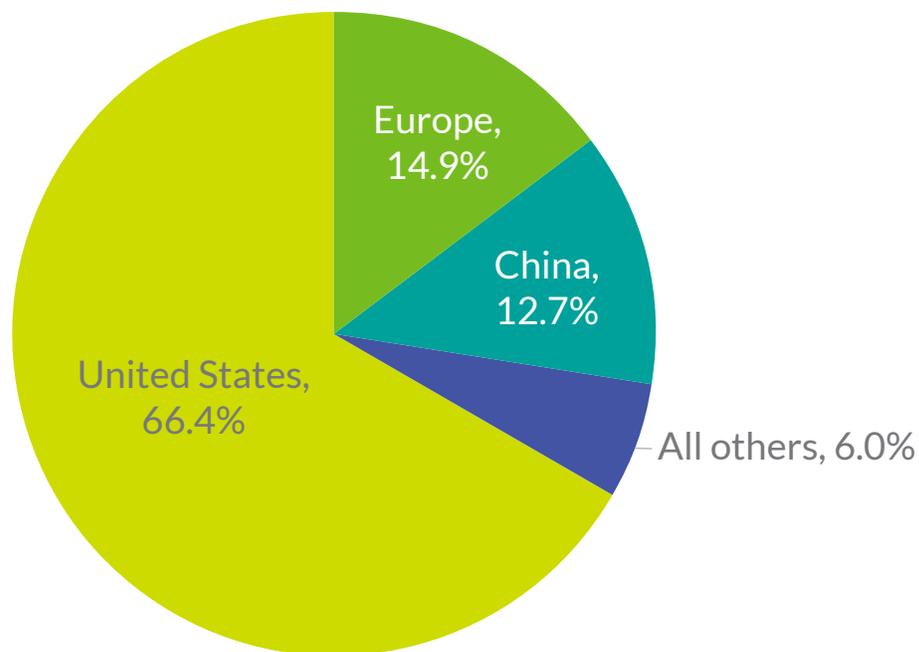
*Asia includes India, Malaysia, South Korea, and others.

Source: PhRMA analysis of National Science Foundation data¹⁴

The United States Leads in Biopharmaceutical Venture Capital Investment

Two-thirds of worldwide venture capital investment in biopharmaceutical startups are made in the United States.

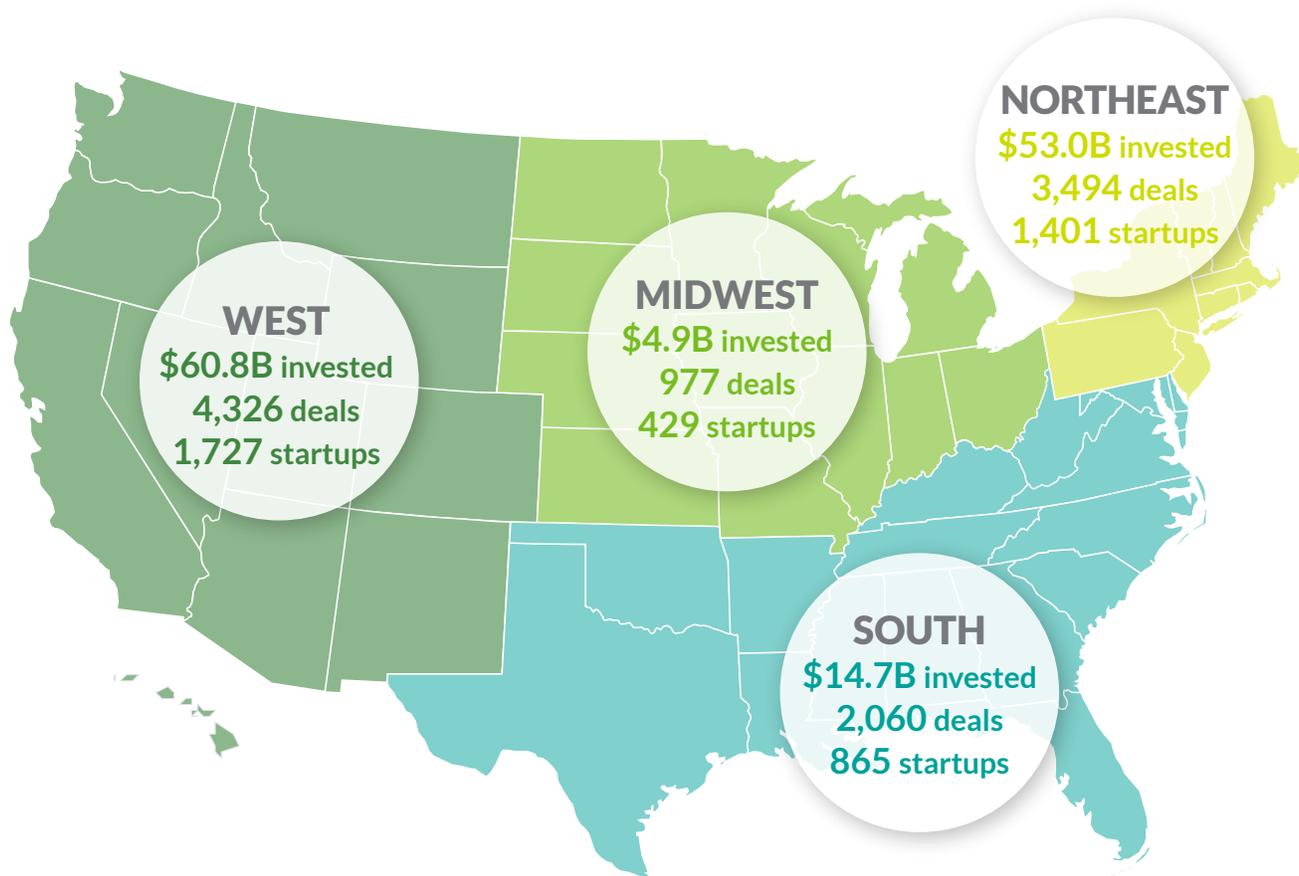
Biopharmaceutical Venture Capital Investment by Region/Country, 2019



Source: PhRMA analysis of PitchBook Venture Investment database¹⁵

Biopharmaceutical Venture Capital Provides Tremendous Resources for Startup Company Financing

Between 2000 and 2019, venture capitalists invested over \$133 billion in more than 10,000 deals helping scientists and entrepreneurs to start up over 4,400 biopharmaceutical companies across the United States.

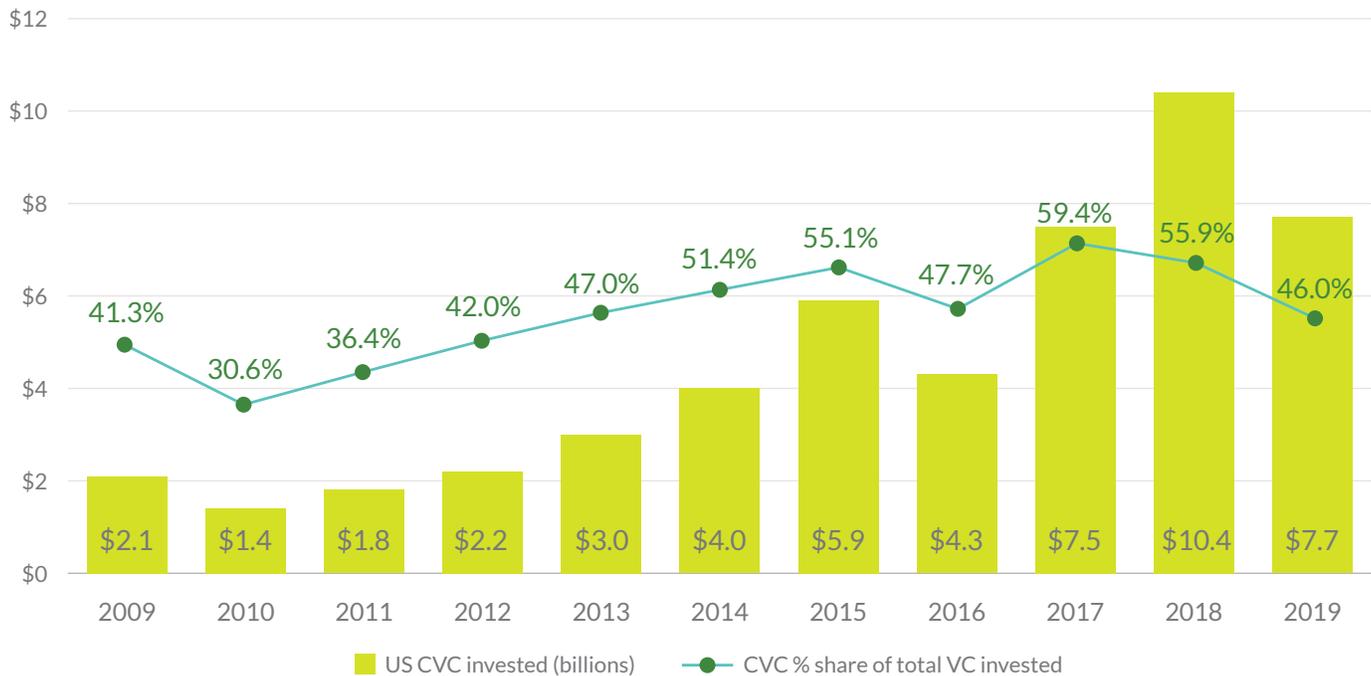


Source: PhRMA analysis of PitchBook Venture Investment database¹⁶

Biopharmaceutical Corporate Venture Capital Supports a Broader Ecosystem

Corporate venture capital (CVC) from biopharmaceutical companies and others plays an increasingly important role in financing emerging biopharmaceutical companies, now accounting for roughly half of venture capital (VC) investment in the sector.

US CVC Investment in Biopharmaceutical Startups, 2009-2019



Source: Q4 2019 PitchBook-NVCA Venture Monitor¹⁷

The Biopharmaceutical Industry Is Reducing Its Impact on the Environment

Biopharmaceutical companies are pioneers in green chemistry and are committed to finding creative and innovative ways, including the following, to reduce waste, conserve energy, and adopt other more environmentally friendly processes.

Implementing manufacturing methods that **replace many solvents** with safer alternatives

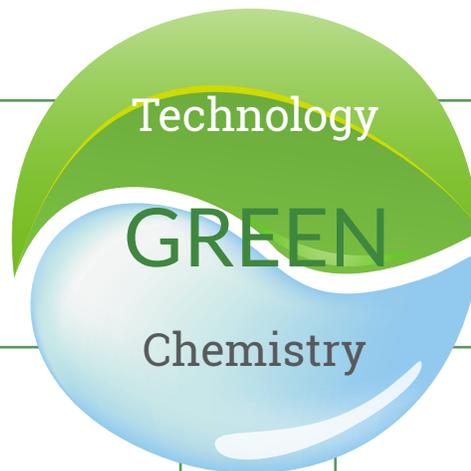
Adopting new manufacturing processes to **reduce emissions and energy use**

Constructing facilities that are **LEED-certified** (Leadership in Energy and Environmental Design)

Adapting **single-use production systems** to minimize environmental impact

Expanding use of **biocatalyzed processes**, which are shorter, produce less waste, and reduce environmental impact

Setting **limits on wastewater discharges** to reduce environmental impact of manufacturing discharges¹⁸

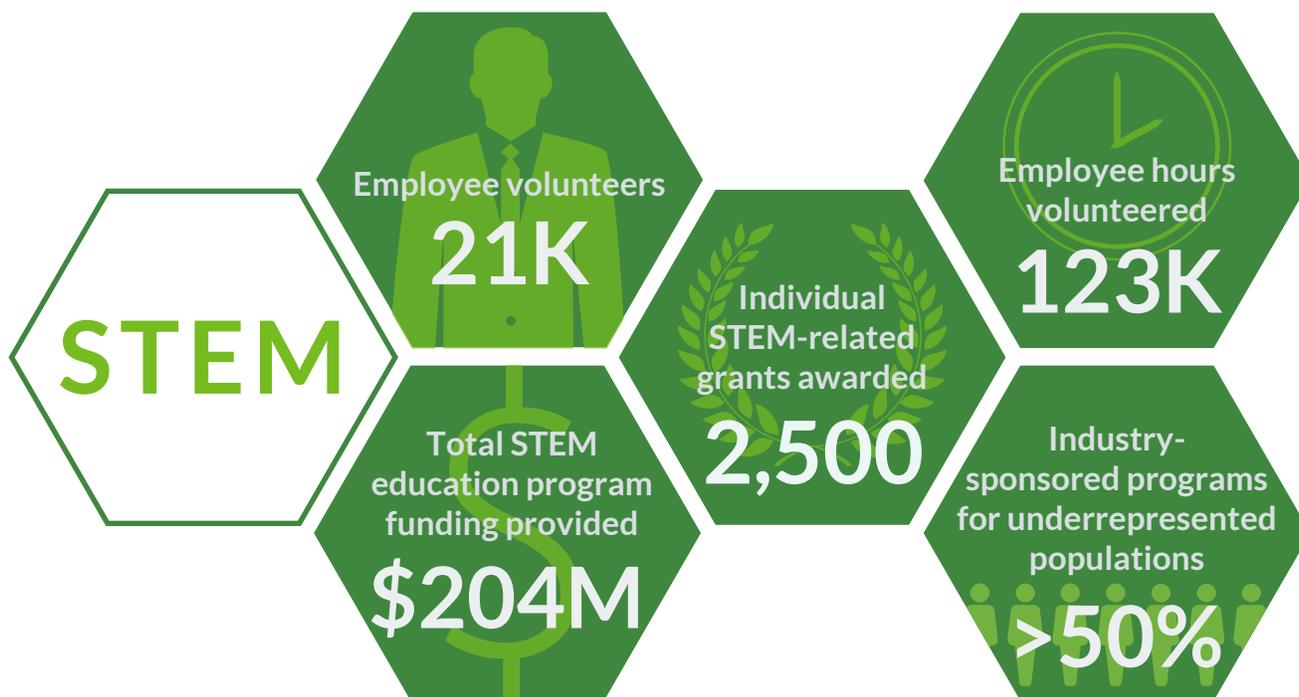


Sources: Deloitte¹⁸; IFPMA¹⁹

The Biopharmaceutical Industry Is Advancing STEM Education in the United States

The STEM workforce accounts for more than 50% of the nation's sustained economic growth. From 2015 to 2020, PhRMA member companies and their foundations supported more than 70 STEM education programs across the United States, impacting more than 7.2 million students and 21,000 teachers.

PhRMA member company and foundation contributions to STEM education in the United States include:



Source: TEconomy Partners²⁰

Other Nations Are Challenging US Leadership in Biopharmaceutical Innovation

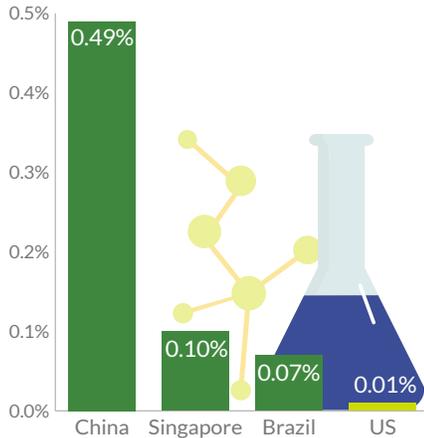
Emerging economies are exceeding US performance on key measures related to a robust biopharmaceutical environment.



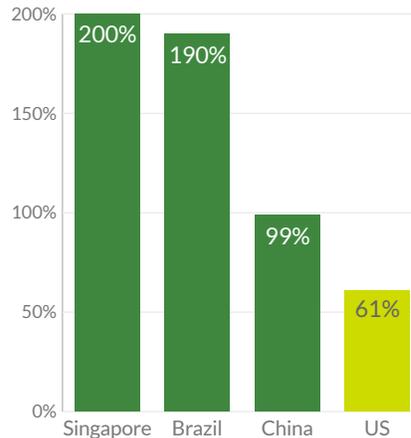
The United States is now facing increasing competition to attract and grow a biopharmaceutical presence not just from developed countries, but also from emerging nations such as Brazil, China, and Singapore that are laying the groundwork for future growth.”

TEconomy Partners²¹

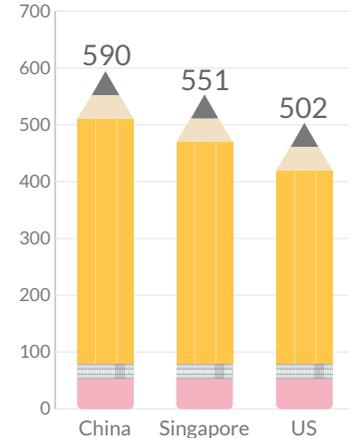
Difference in R&D as Percentage of GDP
2010-2018²²



Percent Increase in Patent Awards
2010-2018²³



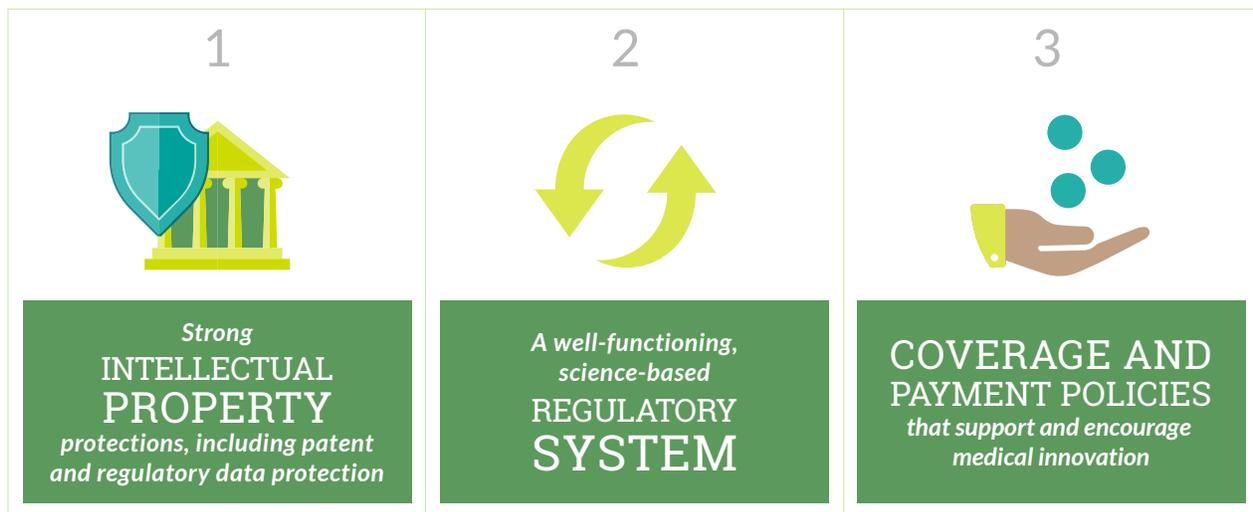
Science Achievement Scores
2018²⁴



Sources: TEconomy Partners²¹; Innovation Research Interchange²²; WIPO²³; OECD²⁴

Fostering Growth of the US Biopharmaceutical Industry Depends on Policies That Support R&D Investment

Industry analysts have consistently identified 3 policy areas as critical for the US biopharmaceutical industry to remain an engine of economic growth and innovation:



The capability to innovate is fast becoming the most important determinant of economic growth and a nation's ability to compete and prosper in the 21st century global knowledge-based economy.”

Battelle Technology Partnership Practice²⁵

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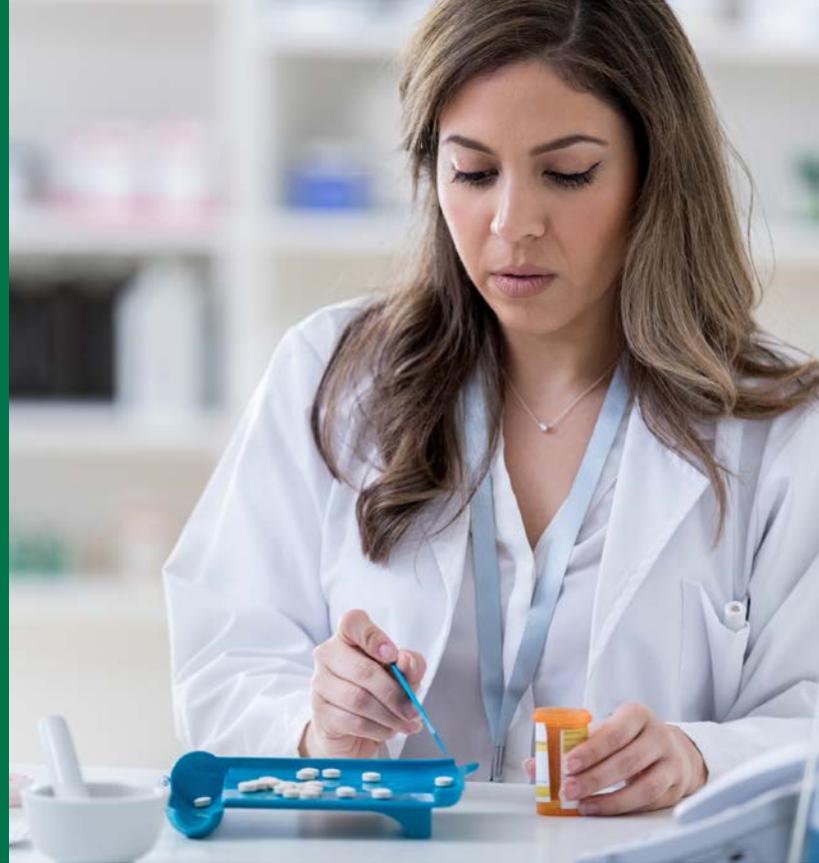




8

INTERNATIONAL COSTS AND ACCESS

Prescription Medicines in
Other Developed Countries



Of the new medicines launched globally each year, far more are available in the United States than in other developed countries. As a result, US patients have better outcomes for conditions where new medicines are most critical.

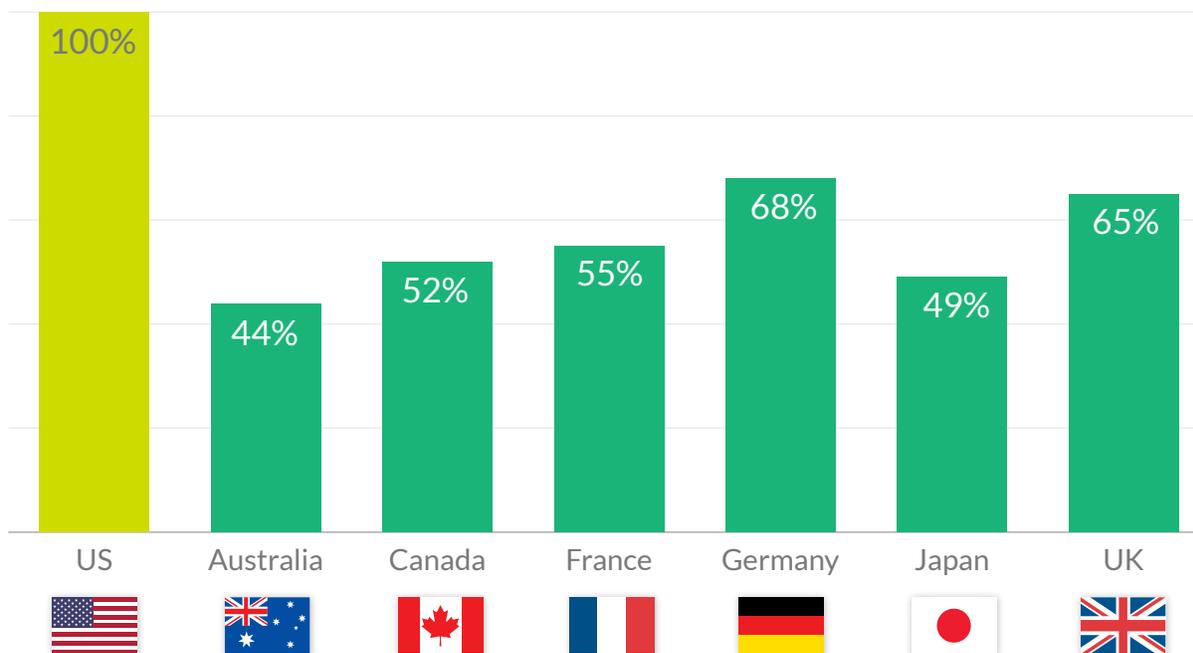
Spending on prescription medicines is a similarly small percentage of total health care spending in the United States as in other developed countries. Consequently, medicines account for a small share of the overall difference in per capita health spending between the US and these other countries.

US market-based prices for new medicines incentivize the large and uncertain investments required to bring new medicines to market. While the US system makes efficient use of cost saving generics and competition among brand medicines, other wealthy countries use a variety of government mandates or controls to set artificially low prices for new medicines. Emulating those practices in the United States would lead to reduced R&D and innovation, harming patients with unmet medical needs.

Many New Medicines Available to US Patients Are Not Available in Other Countries

Other governments' controls on access and prices lead to reduced availability of medicines.

Availability of New Medicines for Select Developed Countries*

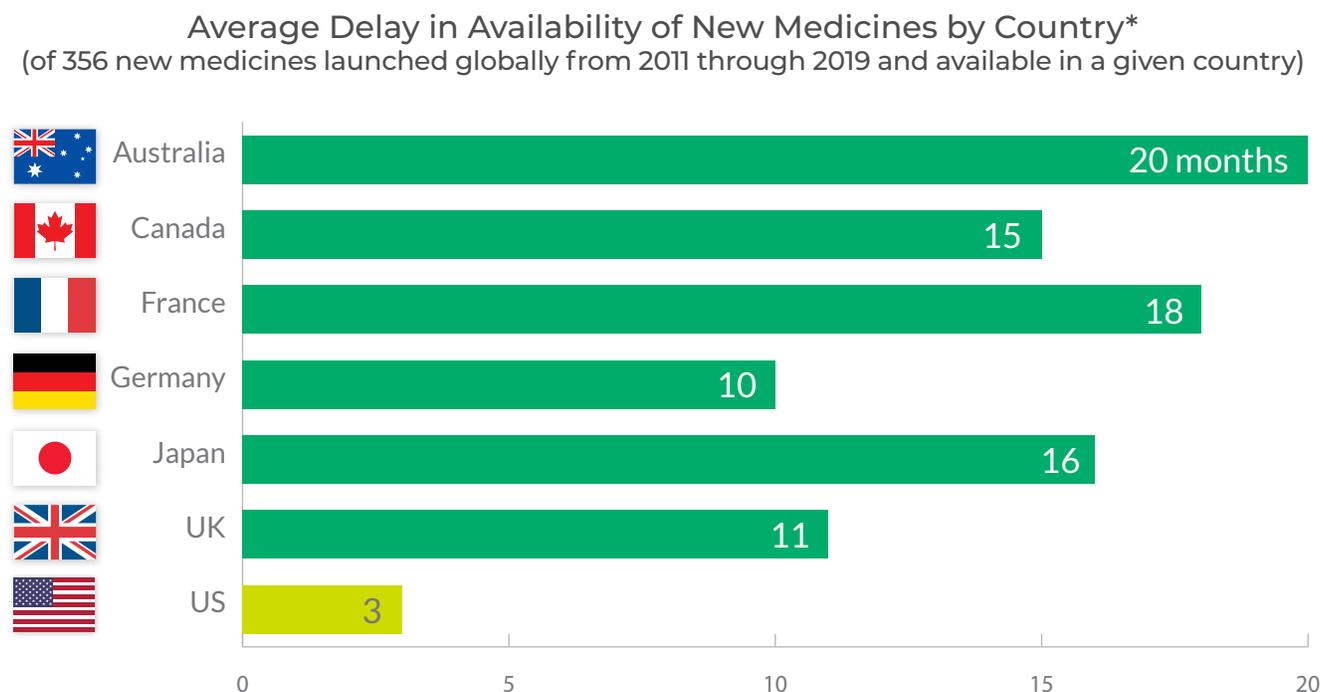


*Includes new active substances launched in the United States from January 1, 2011, to December 31, 2019.

Sources: PhRMA analysis of IQVIA Analytics Link data¹; PhRMA analysis of FDA, EMA, PMDA, Health Canada, and TGA²

US Patients Typically Gain Access to Medicines Much Earlier Than Patients in Other Countries

To the extent that patients in other developed countries have access to medicines, they have to wait longer to access those medicines compared to patients in the United States.



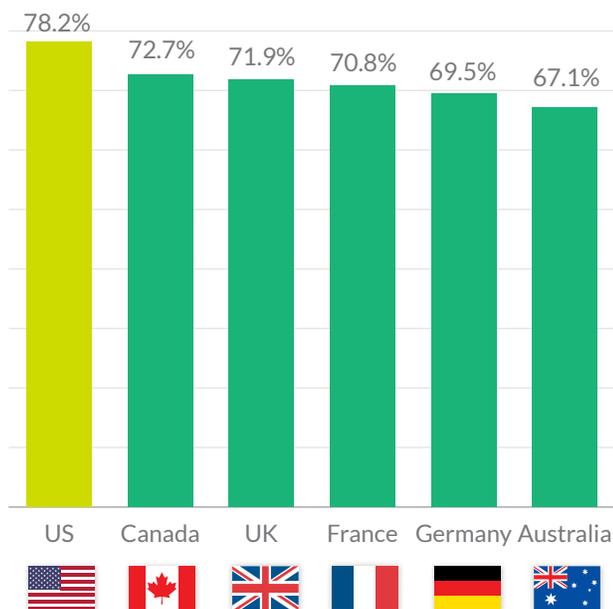
*Includes new active substances launched globally from January 1, 2011, to December 31, 2019. Average delay equals the time in months since global first launch among new active substances that have launched in a given country.

Sources: PhRMA analysis of IQVIA Analytics Link data³; PhRMA analysis of FDA, EMA, PMDA, Health Canada, and TGA⁴

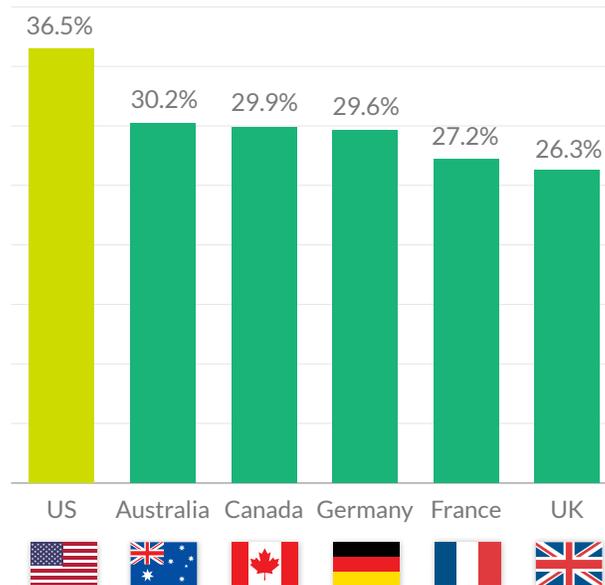
US Patients Have Better Outcomes for Conditions Where New Drugs Are Most Critical

Cancer survival rates are higher in the United States, where patients have greater and more timely access to cancer medicines than in other countries.

Pediatric Brain Cancer 5-Year Survival Rate, 2010-2014



Adult Brain Cancer 5-Year Survival Rate, 2010-2014

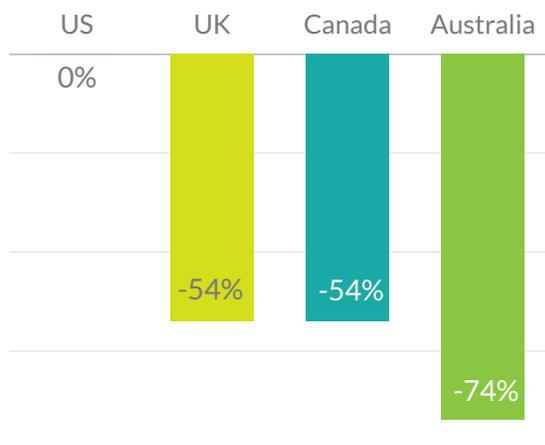


Source: Allemani C et al⁵

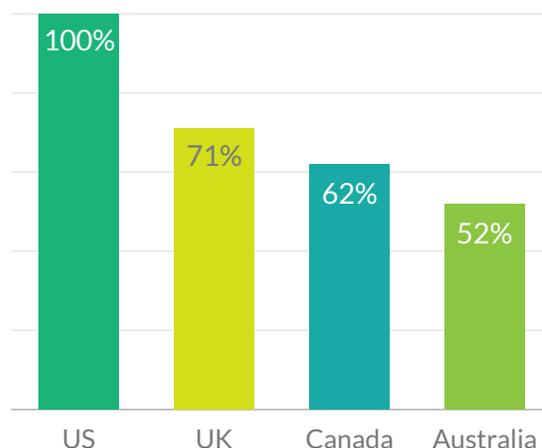
Lung Cancer Patients Experience Better Survival Under the Market Access Policies in the United States

Government price setting in other countries leads to access restrictions and poorer outcomes for conditions that benefit from innovative medicines, such as lung cancer.

Survival Gains US Patients Would Lose Under Foreign Access Restrictions⁶



Availability of Cancer Medicines Approved in the US^{7,8*}



AMERICAN PATIENTS

with lung cancer would have poorer outcomes if they had the same access to medicines seen in other countries.



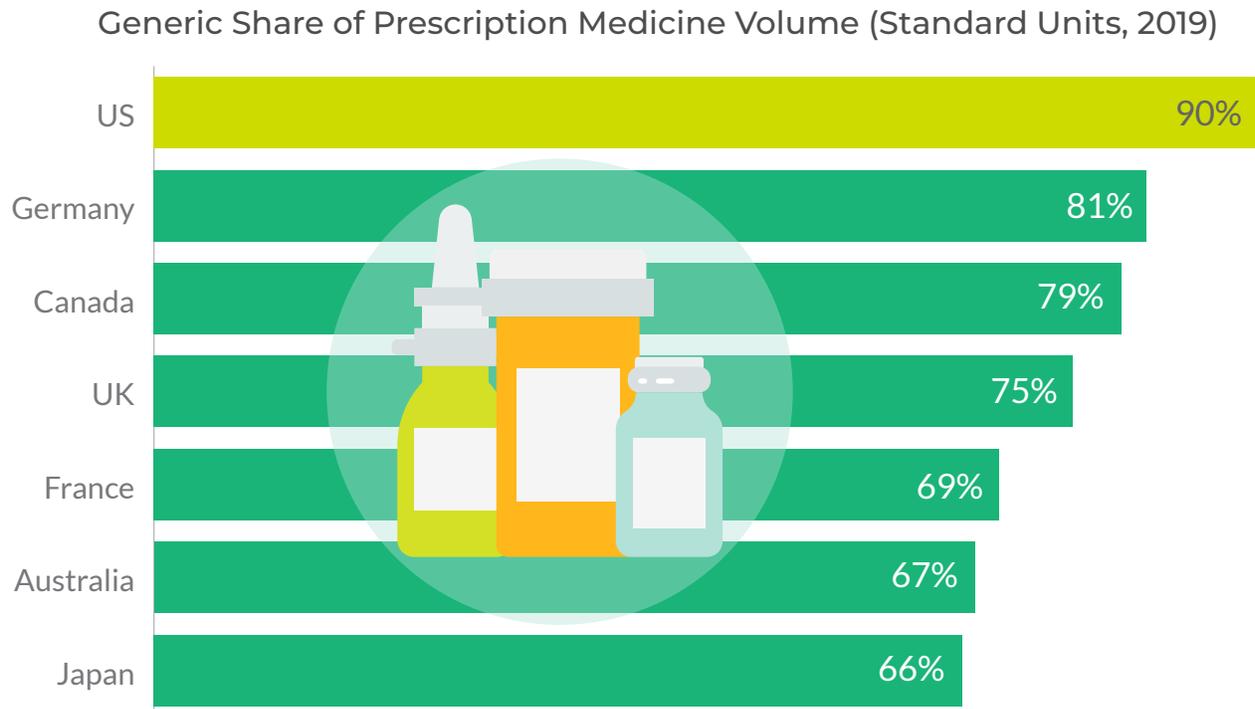
This is in part because patients with cancer in other countries do not have access to all medicines available in the US.

*Includes oncology new active substances launched in the United States from January 1, 2011, to December 31, 2019. Not reflected on the chart are the additional coverage restrictions other countries impose on many medicines.

Sources: IHS Markit⁶; PhRMA analysis of IQVIA Analytics Link data⁷; PhRMA analysis of FDA, EMA, Health Canada, and TGA⁸

Use of Generic Medicines Remains Highest in the United States

Payers in the United States drive rapid and widespread adoption of generic medicines, allowing them to allocate more resources toward covering and reimbursing innovative medicines than payers in other developed countries.

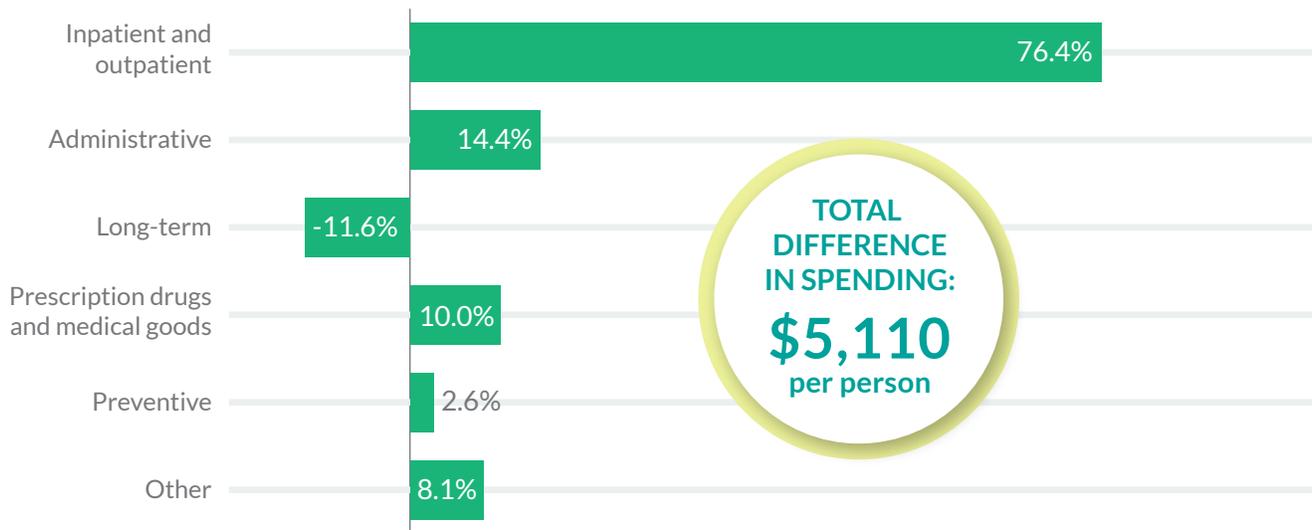


Source: PhRMA analysis of IQVIA Innovation Insights data⁹

Prescription Medicines Are Not Driving the Difference Between US and International Health Care Spending

More than three-quarters of the difference between US and other developed countries' spending on health care is driven by inpatient and outpatient care.

Contribution of Spending Categories to Difference in Per Capita Health Spending Between the US and Other Developed Countries, 2018



Other developed countries include Austria, Belgium, Canada, France, Germany, the Netherlands, Sweden, Switzerland, and the United Kingdom.

Source: Peterson Center on Healthcare and Kaiser Family Foundation¹⁰

Foreign Governments Set Prices and Mandate Other Harmful Practices That Undervalue Innovative Medicines

A variety of approaches are used by foreign governments to limit spending on medicines, which results in reduced access for patients in these countries.

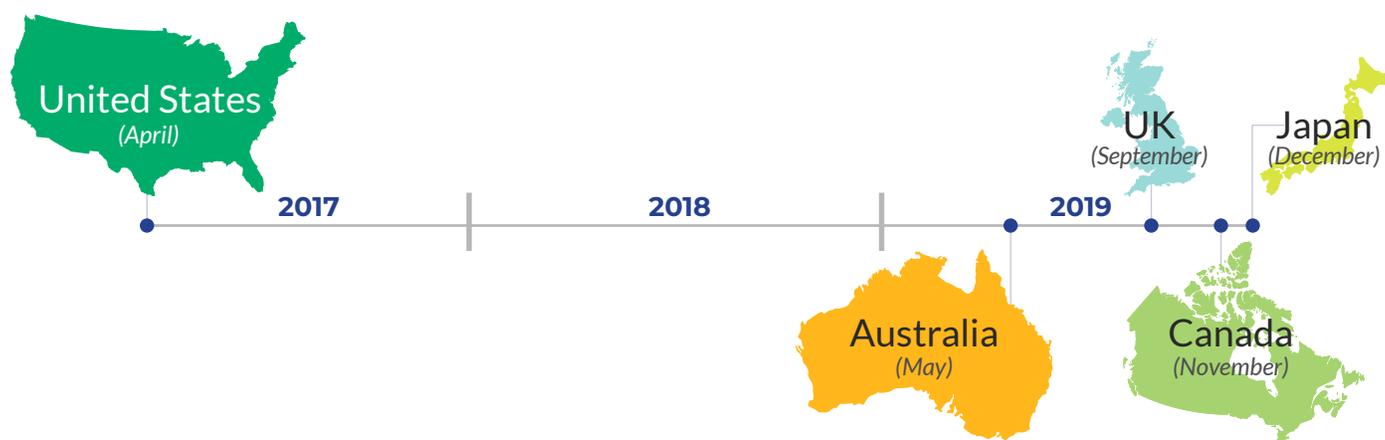
	 Australia	 Canada	 France	 Germany	 Japan	 UK
International Reference Pricing						
Therapeutic Reference Pricing						
Mandatory Price Cuts & Clawbacks						
Quality-Adjusted Life Years (QALYs) Assessments						

Sources: PhRMA analysis of IQVIA Pharmaceutical Pricing & Reimbursement Country Guides¹¹; PhRMA analysis of IHS Markit Economics and Country Risk¹²

Patients in Other Countries Experience Delayed Access to Medicines Including Those to Treat Rare Conditions

In countries with government price setting and strict criteria for coverage and reimbursement, children with Batten disease, a rare, deadly genetic condition, had delayed access to a first-in-class, innovative treatment.

Timing of Access to First-in-Class Medicine for Batten Disease*



... an extraordinary medical breakthrough for the CLN2 Batten community who have been waiting... for more than a century.”

Margie Frazier, PhD, Batten Disease Support and Research Association¹⁵

*Access month/year indicates when the medicine was generally available and covered by a public program in that country, including through special programs that provide access to certain new medicines prior to broad coverage decisions.

Sources: PhRMA analysis of IQVIA Analytics Link data¹³; PhRMA analysis of FDA, EMA, PMDA, Health Canada, TGA, and member company information¹⁴; CenterWatch¹⁵

Overwhelming Evidence Shows Innovation Suffers When Governments Set the Price of Medicines

If the US government set prices for medicines based on prices in foreign countries, US patients would have access to far fewer treatments than they do today.



117 FEWER
new medicines
launched between
1986 and 2004 if
the US had price
controls¹⁶



23%-33% LESS
global R&D investment
under a US government
price setting scheme¹⁷



If the United States had adopted the centralized drug pricing policy in other developed nations twenty years ago, then the world may not have highly valuable treatments for diseases that required significant investment.”

Council of Economic Advisers¹⁸

Notes and Sources

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