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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 challenge of treatment gaps and improving use of prescribed therapies, communications with patients and health care providers, and the role of the biopharmaceutical sector in the U.S. economy.

Data and information found 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 about the role of medicines in the U.S. health care system.
Advances in Treatment

Medicines’ Impact on Health and Quality of Life

Prescription medicines continue to yield important advances against serious disease, helping patients live longer and healthier lives. Over the last 25 years, prescription medicines have transformed the trajectory of many debilitating diseases, including HIV/AIDS, cancer, and heart disease, resulting in decreased death rates, improved health outcomes and often better quality of life for patients. Recent advances continue to improve outcomes for patients across a broad range of chronic conditions — including, for example, diabetes and hepatitis C. Medicines today are at the forefront of science with an increasing number of targeted therapies that, in some cases, are providing the very first treatments for patients suffering from a number of rare diseases. Continued advances in biopharmaceutical innovation will be key in addressing future health care challenges and helping patients lead better lives.
While nutrition, sanitation, other public health measures, and expanded access to care have been major sources of increasing human health, innovative medicines have also played a profound role in this progress.

— The President’s Council of Advisors on Science and Technology

*Life expectancies prior to 1997 were calculated using a slightly different methodology than for those post-1997.

Source: President’s Council of Advisors on Science and Technology; CDC
A Decade of Advances

2004
- First anti-angiogenic medicine for cancer
- New Rx for most common form of lung cancer

2005
- First new kidney cancer Rx in over a decade
- Three new therapies for diabetes

2006
- First vaccine for the prevention of cervical cancer
- First Rx for chronic chest pain in 20 years
- First once-a-day HIV medicine

2008
- A new type of treatment for Crohn’s disease
- The first Rx for symptoms of Huntington’s disease

2009
- First treatment for peripheral T-cell lymphoma
- First new Rx for gout in 40 years

2010
- Two new multiple sclerosis drugs
- First therapeutic cancer vaccine

2011
- First lupus drug in 50 years
- Two hepatitis C drugs offer better chance for a cure
- Two new personalized medicines

2013
- Two new personalized medicines to treat the most dangerous forms of skin cancer
- More than 5,000 medicines in development

Sources: FDA\(^3\); Analysis Group\(^4\)
HIV/AIDS
In the last two decades, advances in treatment have contributed to a more than 80% decline in death rates and transformed the disease from an acute, fatal illness to a chronic condition.

Cancer
New therapies have contributed to a 20% decline in cancer deaths since the 1990s. Today, 2 out of 3 people diagnosed with cancer survive at least 5 years.

Multiple Sclerosis
Oral and biologic treatments approved over the past 15 years have dramatically improved outcomes for MS patients, slowing disability progression and offering fewer relapses.

Rheumatoid Arthritis
Therapeutic advances have transformed the RA treatment paradigm over the last 20 years, from focusing on symptom management to now aiming for slowed disease progression and even disease remission.

Sources: National Multiple Sclerosis Society; Boston Healthcare; CDC; American Cancer Society
Rheumatoid Arthritis: Medicines Are Transforming the Lives of Patients

THEN:
Treatments for RA were effective at reducing joint inflammation but were limited to treating the symptoms of the disease, allowing for steady progression from disease onset to disability fairly rapidly.

NOW:
Biologic disease-modifying antirheumatic drugs (DMARDs) can target the underlying sources of inflammation, which improves physical functioning and prevents irreversible joint damage — making disease remission possible.

Source: Boston Healthcare
The number of U.S. AIDS deaths decreased dramatically following the introduction of highly active antiretroviral treatment (HAART). Today, research shows that 20-year-olds diagnosed with HIV can expect to live into their early 70s — a life expectancy close to that of the general population.\textsuperscript{10}

**Annual Number of AIDS Deaths in the United States**

Sources: H. Samji, et al.\textsuperscript{10}; CDC\textsuperscript{11}
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.

1981
AIDS first reported

1984
HIV identified as the cause of AIDS

1987
First treatment (AZT) introduced (a nucleoside analog reverse-transcriptase inhibitor)

1991
AZT labeling expanded for dosing (IV), earlier use, and pediatric use

1994
AZT found to reduce the risk of transmission from mother to infant

1995
First protease inhibitors approved

1996
HAART combinations introduced

2001
First nucleotide analog approved

2003
First fusion inhibitors approved

2006
Rates of transmission from mother to infant have dropped to less than 2%
First one-pill-once-a-day treatment approved

2007
First CCR-5 co-receptor agonist approved

2011
U.S. HHS recommends earlier initiation of treatment to control immunologic response

2012
U.S. death rate has dropped by more than 80%

Source: Boston Healthcare
Cancers: Decline in Death Rates

According to the American Cancer Society, improvements in treatment contributed to the increase in cancer survival.\(^\text{13}\)

*Percent Change by Decade in U.S. Death Rates from Cancer*

<table>
<thead>
<tr>
<th>Decade</th>
<th>Percent Change</th>
</tr>
</thead>
<tbody>
<tr>
<td>1970–1980</td>
<td>4.7%</td>
</tr>
<tr>
<td>1980–1990</td>
<td>3.9%</td>
</tr>
<tr>
<td>1990–2000</td>
<td>-7.6%</td>
</tr>
<tr>
<td>2000–2011</td>
<td>-15.5%</td>
</tr>
</tbody>
</table>

Source: R. Siegal, et al.\(^\text{13}\); CDC\(^\text{14}\)
Chronic Leukemias: Increased Survival Rates

When imatinib was first approved in 2001 to treat chronic myeloid leukemia (CML), the full value of the medicine had not been completely realized.

- After initial approval, continued research revealed that imatinib had an even greater impact when initiated earlier in the progression of the disease.
- Further research also revealed that imatinib was effective in combating other devastating forms of leukemia, as well as gastrointestinal stromal tumors.
- Today, survival rates have improved dramatically and CML patients are living close to normal life spans.\(^{17}\)

![5-Year Survival Rates for CML Patients\(^{19,20}\)](image)

Sources: Boston Healthcare\(^{15}\); C. Gambacorti-Passerini, et al.\(^{16}\); American Cancer Society\(^{17}\); B. Druker, et al.\(^{18}\); FDA\(^{19}\); CDC\(^{20}\)
Rare Diseases: Drug Approvals for Rare Diseases Have Increased

Rare diseases are those that affect 200,000 or fewer people in the United States. There are nearly 7,000 rare diseases affecting a combined 30 million Americans.

*Number of Drug Approvals for Rare Diseases*

*Approvals for rare diseases include initial approvals of new medicines and subsequent approvals of existing medicines for rare disease areas.*

Source: FDA

1983: Orphan Drug Act passed

Source: FDA

*Approvals for rare diseases include initial approvals of new medicines and subsequent approvals of existing medicines for rare disease areas.*
Cardiovascular Disease: Declining Rates of Death and Heart Failure

“Factors contributing to the decline in heart disease and stroke mortality include better control of risk factors, improved access to early detection, and better treatment and care, including new drugs and expanded uses for existing drugs.”

— U.S. Centers for Disease Control and Prevention


Source: CDC
Hepatitis C: Recent Treatment Advances Dramatically Improve Outcomes for Patients

With baby boomers representing 75% of adults infected with Hepatitis C, in the absence of recent treatment advances the impact of the disease would be expected to grow substantially in coming years.

- Hepatitis C kills 5 times as many Americans as AIDS and is the leading cause of liver transplants and liver cancer.

- With the recent introduction of direct-acting antiviral (DAA) medicines:
  - Cure rates have reached 90% or higher
  - Treatment duration has been cut from 48 weeks to as low as 12 weeks
  - Side effects have gone from debilitating to few.

Sources: CDC; Cleveland Clinic; B. Horoldt, et al.; E. Lawitz, et al.

*Previous standard treatment option.

Potential Cure Rates for Hepatitis C Patients

<table>
<thead>
<tr>
<th>Therapy Type</th>
<th>Cure Rate</th>
</tr>
</thead>
<tbody>
<tr>
<td>Interferon-based Therapy* (2000s)</td>
<td>50%</td>
</tr>
<tr>
<td>DAAs (2013)</td>
<td>90%</td>
</tr>
</tbody>
</table>

Sources: CDC; Cleveland Clinic; B. Horoldt, et al.; E. Lawitz, et al.
Future Impact: Need for New Treatments for Alzheimer’s Disease

The development of a new treatment that delays the onset of Alzheimer’s could reduce Medicare and Medicaid spending on patients with Alzheimer’s by more than $100 billion annually by 2030.*

*Assumes research breakthroughs that delay the average age of onset of Alzheimer’s disease by 5 years beginning in 2010.

**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 Association28
Notes and Sources


Notes and Sources


Notes and Sources


Notes and Sources


RESEARCH AND DEVELOPMENT

The Process of Drug Discovery and Development

More than 5,000 medicines are in development globally with the potential to aid U.S. patients. PhRMA member companies invested $51.1 billion in biopharmaceutical research and development (R&D) in 2013, accounting for the majority of private biopharmaceutical R&D spending. Development of new medicines is a rigorous and long process, and it has become more costly and complex over the last decade. Even among the new drug candidates reaching Phase III trials, about one-third fail. Companies “race” to bring the first medicine in a class to market, and just two in ten approved drugs are ultimately commercial successes. Recent biopharmaceutical advances — driven by scientific research and creative ingenuity — would not have been possible without a system of laws that provide the structure, stability, and opportunity for the needed investment.
In 2011, 5,408 medicines* were in clinical development worldwide with the potential to aid U.S. patients.

*Defined as single products which are counted exactly once regardless of the number of indications pursued.

Source: Analysis Group¹
More than 900 Biologic Medicines in Development in 2013

Biologic medicines — large, complex molecules derived from living cells — frequently represent novel strategies that have the potential to transform the clinical treatment of disease.

Autoimmune Disorders: 71
Blood Disorders: 43
Cancers/Related Conditions: 338
Cardiovascular Disease: 58
Diabetes/Related Conditions: 28
Digestive Disorders: 26
Eye Conditions: 25
Genetic Disorders: 30
Infectious Diseases: 176
Musculoskeletal Disorders: 34
Neurologic Disorders: 39
Respiratory Disorders: 38
Skin Diseases: 30
Transplantation: 13
Other: 58

*Some medicines are being explored in more than one therapeutic category.

Source: PhRMA²
Personalized Medicines Increasingly Shape the R&D Pipeline

Biopharmaceutical companies have increased R&D investment in personalized medicine by 75% between 2005 and 2010.3

“Oncology is on fire with [personalized medicine], with treatment selections based on individual molecular characteristics. This is also happening with chronic infectious diseases, and genetic diseases are not far behind.”

— Janet Woodcock, Director Center for Drug Evaluation and Research, U.S Food and Drug Administration4

<table>
<thead>
<tr>
<th>Therapeutic Area</th>
<th>No. of Trials</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cardiovascular</td>
<td>16</td>
</tr>
<tr>
<td>Central Nervous System/Neurology/Behavior</td>
<td>20</td>
</tr>
<tr>
<td>Dermatology</td>
<td>1</td>
</tr>
<tr>
<td>Endocrinology/Metabolics</td>
<td>8</td>
</tr>
<tr>
<td>Gastrointestinal</td>
<td>3</td>
</tr>
<tr>
<td>Infectious Disease</td>
<td>10</td>
</tr>
<tr>
<td>Oncology/Hematology</td>
<td>85</td>
</tr>
<tr>
<td>Ophthalmology</td>
<td>2</td>
</tr>
<tr>
<td>Renal</td>
<td>3</td>
</tr>
<tr>
<td>Respiratory/Pulmonary</td>
<td>7</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td><strong>155</strong></td>
</tr>
</tbody>
</table>

Sources: Tufts CSDD3; J.T. Aquino4; D. Nelleson, et al.5
Potential First-in-Class Medicines in the Pipeline

An average of 70% of drugs across the pipeline are potential first-in-class medicines.

Source: Analysis Group

Percentage of Projects in Development that Are Potentially First-in-Class Medicines in Selected Therapeutic Areas, 2011

- Neurology: 84%
- Cardiovascular: 81%
- Cancer: 80%
- Psychiatry: 79%
- Immunology: 72%
- Diabetes: 71%
- HIV/AIDS: 69%
- Infections: 57%

Source: Analysis Group
Cutting-Edge Research Drives Development of Medicines

Tomorrow’s medicines are often born from innovative scientific approaches.

Select examples include:

**Antisense RNA interference (RNAi)** targets RNA in order to silence gene expression associated with certain diseases.

**Cell therapy** is a type of regenerative medicine, introducing new cells into a tissue in order to treat a disease.

**Gene therapy** involves the alteration of genes within cells and tissue, often to counteract genetic defects.

**Conjugated Monoclonal Antibodies (mAbs)** target and kill tumors while sparing nearby healthy cells.

*Medicines in development as of December 2011.*

*Source: Analysis Group*
The Research and Development Process

Developing a new medicine takes an average of 10 to 15 years.

Source: PhRMA®
Government and Industry Roles in Research & Development

Government and biopharmaceutical industry research complement one another.

PhRMA Member Companies: $51.1 Billion

Clinical Research

Translational Research

Basic Research

National Institutes of Health: $29.2 Billion*

*NIH spending is for FY 2012. PhRMA member companies’ spending is estimated for CY 2012. PhRMA member companies account for the majority of private biopharmaceutical R&D spending. Nonmember company data are not included.

Sources: PhRMA9; NIH Office of Budget10; adapted from E. Zerhouni11
Innovative Biopharmaceutical Companies Sit at the Heart of a Dynamic R&D Ecosystem in the U.S.

While research-based biopharmaceutical companies are responsible for bringing new medicines to patients, they are part of an ecosystem marked increasingly by collaborations with academic institutions, government agencies, venture capital firms, nonprofit foundations, patients and others, as illustrated.
Corporate Venture Capital Helping to Fill Early-Stage Funding Gap

Venture capital investments in emerging biotech companies have dropped 22% from 2007 to 2013, with the most rapid declines seen in first-round deals. The corporate venture capital (CVC) arms of established biopharmaceutical companies are helping fill this growing gap. The share of early-stage biotech companies receiving CVC investment has doubled since 2007.

Source: PwC and National Venture Capital Association

Share of Early-Stage Biotech Deals Involving CVC Funds, 2007 vs. 2012

Source: PwC and National Venture Capital Association
PhRMA Member Company R&D Investment

“The pharmaceutical industry is one of the most research-intensive industries in the United States. Pharmaceutical firms invest as much as five times more in research and development, relative to their sales, than the average U.S. manufacturing firm.”

— Congressional Budget Office

PhRMA Member Company R&D Expenditures: 1995–2013

*Estimated FY 2013.

Source: Congressional Budget Office; PhRMA
Drug Development Costs Have Increased

According to a 2007 study, it costs an average of $1.2 billion to develop one new drug.\textsuperscript{16} More recent studies estimate the costs to be even higher.\textsuperscript{17}

*The Average Cost to Develop One New Approved Drug — Including the Cost of Failures*

Sources: J.A. DiMasi and H.G. Grabowski\textsuperscript{16}; J. Mestre-Ferrandiz, et al.\textsuperscript{17}; J.A. DiMasi, et al.\textsuperscript{18}
Complexity of Clinical Trials Has Increased

During the last decade, clinical trial designs and procedures have become much more complex, demanding more staff time and effort, and discouraging patient enrollment and retention.

**Trends in Clinical Trial Protocol Complexity**

<table>
<thead>
<tr>
<th></th>
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<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Total Procedures per Trial Protocol (median) (e.g., bloodwork, routine exams, x-rays, etc.)</td>
<td>105.9</td>
<td>166.6</td>
<td>57%</td>
</tr>
<tr>
<td>Total Investigative Site Work Burden (median units)</td>
<td>28.9</td>
<td>47.5</td>
<td>64%</td>
</tr>
<tr>
<td>Total Eligibility Criteria</td>
<td>31</td>
<td>46</td>
<td>58%</td>
</tr>
<tr>
<td>Clinical Trial Treatment Period (median days)*</td>
<td>140</td>
<td>175</td>
<td>25%</td>
</tr>
<tr>
<td>Number of Case Report Form Pages per Protocol (median)</td>
<td>55</td>
<td>171</td>
<td>227%</td>
</tr>
</tbody>
</table>

*These numbers reflect only the “treatment duration” of the protocol.

Source: K.A. Getz, et al. and Tufts CSDD19
Illustrative Pharmaceutical Lifecycle

New pharmaceutical medicines face competition after a relatively short period on the market.

Average time to develop a new medicine = 10–15 yrs

Average time on market before generic entry = 12.6* yrs

*For brand medicines with more than $100 million in annual sales in 2008 dollars, which account for 97% of sales of the brand medicines analyzed.

Sources: PhRMA; H. Grabowski, et al.
Earlier and More Frequent Patent Challenges by Generic Companies

As early as 4 years after brand launch, a generic company may file with FDA a Paragraph IV certification to “challenge” patents associated with the brand-name 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**

<table>
<thead>
<tr>
<th>Year</th>
<th>Number of Years</th>
</tr>
</thead>
<tbody>
<tr>
<td>1996</td>
<td>15.6</td>
</tr>
<tr>
<td>2004</td>
<td>7.7</td>
</tr>
<tr>
<td>2012</td>
<td>6.9</td>
</tr>
</tbody>
</table>

**... and are more common**

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

<table>
<thead>
<tr>
<th>Year</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>1996</td>
<td>14%</td>
</tr>
<tr>
<td>2004</td>
<td>41%</td>
</tr>
<tr>
<td>2012</td>
<td>81%</td>
</tr>
</tbody>
</table>

All numbers are three-year moving averages.

Sources: H.G. Grabowski, et al.22
Competing Medicines Race for Approval

By 1995, nearly all first-in-class medicines being approved already had potential competitors in Phase II clinical testing.

Source: J.A. DiMasi and L.B. Faden

Percentage of First-in-Class Medicines with a Competitor Already in Phase II Clinical Testing at Time of Approval

Source: J.A. DiMasi and L.B. Faden
Increasing Competition Within Therapeutic Categories

The time a medicine is the only drug available in its therapeutic class has declined dramatically — from a median of more than 10 years in the 1970s to less than two years by 1998.

*Source: Tufts CSDD*
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.

*Just 2 in 10 Approved Medicines Produce Revenues that Exceed Average R&D Costs*

Source: J.A. Vernon, et al.25
Notes and Sources


Notes and Sources

22. Ibid.
SPENDING AND COSTS

Understanding Spending on Medicines

Prescription medicines represent a small share of national health spending. Since 2000, growth in prescription drug spending has slowed markedly, while prices for prescription medicines have risen in line with overall medical inflation.

Innovator pharmaceutical companies produce medical advances through pioneering scientific work and large-scale investments. The innovators’ work and investment lead both to new medicines and, over time, to generic copies that consumers use at low cost for many years.

Health plans use many tools — such as tiered formularies and cost sharing — to steer use toward generics and lower-cost medicines. Payers also typically require patients to pay a higher share of the costs of medicines out of pocket compared with other health services.
Sharply Declining Prescription Medicine Spending Growth: 1999–2011*

Spending growth for prescription medicines has slowed dramatically over the past decade, with historically low rates of growth observed in recent years.

*Total retail sales including brand medicines and generics.

Source: CMS1
Medicines Account for a Small and Declining Share of Health Spending Growth

Growth in Health Care Expenditures Attributable to Prescription Drugs, 1998–2012

- 1998–2002: 16% Prescription Drugs, 84% All Other Health Care
- 2003–2007: 11% Prescription Drugs, 89% All Other Health Care
- 2008–2012: 5% Prescription Drugs, 95% All Other Health Care

Source: CMS²
Retail Spending on Prescription Medicines Is a Small Share of Total U.S. Health Care Spending

*Other includes dental, home health, and other professional services as well as durable medical equipment costs.

Source: PhRMA analysis based on CMS³
Growth in Prescription Medicine Prices Has Been in Line with Other Health Care Prices

Source: PhRMA analysis based on Bureau of Labor Statistics

Source: PhRMA analysis based on Bureau of Labor Statistics

Consumer Price Index (2003 = 100)
More Than Four Out of Five U.S. Prescriptions Are Filled with Generics

*Generic share includes generics and branded generics. “Other” category from IMS NPA not included in calculation.

Source: IMS Health

**Generic Share* of Prescriptions Filled 1984–2013**

*Generic share includes generics and branded generics. “Other” category from IMS NPA not included in calculation.

Source: IMS Health
Innovator pharmaceutical companies produce medical advances through pioneering scientific work and large-scale investments. The innovators’ work and investment lead both to new medicines and, over time, to generics that consumers use at low cost for many years.

*Ten therapeutic classes most commonly used by Part D enrollees in 2006 were: lipid regulators, ACE inhibitors, calcium channel blockers, beta blockers, proton pump inhibitors, thyroid hormone, angiotensin II, codeine and combination products, antidepressants, and seizure disorder medications.

Source: M. Kleinrock
Insurance Covers a Lower Share of Prescription Drug Costs than of Other Medical Services

On average, privately insured consumers pay out-of-pocket more than 20% of their total prescription drug spending, compared to 4% of spending for inpatient hospital care and 7% on hospital outpatient care.7

*Average Patient Cost-Sharing by Type of Service in the Commercial Market*

*Includes brand & generic.

Sources: PhRMA analysis based on Medical Expenditure Panel Survey (MEPS)7; P.J. Cunningham8
Powerful Purchasers Negotiate on Behalf of Patients

A small number of large purchasers dominate the U.S. prescription drug market.

*Figures may not sum to totals due to rounding.

**Medco was acquired by Express Scripts in April 2012. Figure for Express Scripts/Medco is the sum of the individual script totals for each entity for the most recently reported 12-month period in 2012.

** Prescription Volume by Pharmacy Benefit Management (PBM) Companies, 2012

<table>
<thead>
<tr>
<th>Company</th>
<th>Number of Prescriptions</th>
<th>Market Share (%)*</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Express Scripts/Medco Health Solutions**</td>
<td>1,411 million</td>
<td>29.5%</td>
</tr>
<tr>
<td>2. CVS/Caremark</td>
<td>775 million</td>
<td>16.2%</td>
</tr>
<tr>
<td>3. Argus Health Systems</td>
<td>504 million</td>
<td>10.5%</td>
</tr>
<tr>
<td>4. OptumRx, Inc.</td>
<td>319 million</td>
<td>6.7%</td>
</tr>
<tr>
<td>5. ACS, Inc.</td>
<td>250 million</td>
<td>5.2%</td>
</tr>
<tr>
<td>Top 5 PBM Total</td>
<td>3,259 million</td>
<td>68.2%</td>
</tr>
<tr>
<td>Top 10 PBM Total</td>
<td>4,107 million</td>
<td>85.9%</td>
</tr>
<tr>
<td>Top 15 PBM Total</td>
<td>4,584 million</td>
<td>95.9%</td>
</tr>
<tr>
<td>All PBM s in U.S.</td>
<td>4,780 million</td>
<td>100%</td>
</tr>
</tbody>
</table>

Source: Atlantic Information Services, Inc.9
In the U.S. System, Health Plans Have Powerful Tools to Reduce Spending on Medicines

- **Tiered Copays**: Higher cost to patients for brands than for generics and preferred brands.
- **Formularies**: List of covered drugs.
- **Prior Authorization**: Physicians required to justify medicine’s use before it’s covered.
- **Concentrated Purchasing Power**: Individual Pharmacy Benefit Managers buy medicines for more people than in entire European countries.
- **Financial Incentives**: Payments to physicians and/or pharmacies for generic prescribing or switching patients to preferred drugs.
- **Step Therapy**: Patients must try and fail on alternatives before certain medicines are covered.

Payers drive nearly all use of medicines to generics and “preferred” brands.

Source: IMS Health

---

**Note**: This content is extracted from a document discussing health plans and their strategies to reduce spending on medicines. The strategies include tiered copays, formularies, prior authorization, concentrated purchasing power, financial incentives, and step therapy. The document highlights how payers drive the use of medicines to generics and preferred brands, emphasizing the role of health plans in controlling costs and managing medication use.
Plans Increasingly Subject Certain Medicines to Higher Cost Sharing

Patients taking medicines placed on a 4th tier or higher can be subject to higher cost sharing relative to lower tiers. Patients needing these medicines commonly face serious and challenging health conditions.

Share of Plans with Four or More Tiers

Source: KFF/HRET11
Newly Introduced Generics Are Adopted Rapidly

When a generic version of a medicine becomes available for the first time, it captures an average of three quarters of the market within 3 months,\(^{12}\) and some capture as much as 90% by that time.\(^{13}\)

*Average Generic Share of Total Use Following Launch of a Brand Medicine’s First Generic, 2011–2012*

*Average monthly generic share of total standardized units of a unique molecule/form combination.*

Sources: H. Grabowski, et al.\(^ {12}\); Express Scripts\(^ {13}\)
Medicines Account for a Small Share of Health Spending Differences Between the United States and Other Countries

For example, medicines account for 6% of the difference in total health care spending between the U.S. and Canada, and 9% between U.S. and Germany.

Source: PhRMA analysis based on Organisation for Economic Co-operation and Development
Notes and Sources


2. Ibid.


7. PhRMA analysis of Agency for Healthcare Research and Quality, Medical Expenditure Panel Survey, 2009. Available at www.meps.ahrq.gov/mepsweb/ (accessed March 2014). Prescription drug spending includes brand and generic ingredients, pharmacy, and distribution costs. Estimates are not restricted to individuals who have private coverage that includes prescription coverage, which can be expected to account for less than 2%.


Notes and Sources


OUTCOMES AND SAVINGS

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

Undertreatment of chronic disease and suboptimal use of prescribed medicines are significant public health problems, costing the U.S. economy hundreds of billions of dollars each year. An ever growing body of evidence demonstrates that improved use of prescribed medicines recommended to treat chronic conditions can result in better health outcomes, lower costs for other health care services, and increased worker productivity.
Most Americans Use Few or No Medicines — a Small Share of People Fill the Majority of Prescriptions

The top 20% of people who used medicines accounted for almost two-thirds of all prescriptions filled in 2010.

Source: PhRMA analysis based on MEPS\(^1\)

(39% of the population uses no medicines)
Medicines’ Changing Role in Recommended Care

Revisions to clinical guidelines based on the latest research have resulted in appropriate increases in the use of medicines in recent years.

Changes in the size of the treatable population as target levels change, such as lower targets for blood pressure, blood glucose, lipids

Changes in the number and type of recommended medicines — such as a shift from single to combination therapy — to better control conditions

Changes in therapeutic regimen and duration to better control conditions, such as longer continuation of treatment for depression

Source: R.W. Dubois and B.B. Dean²
Failure to Prescribe the Indicated Treatment Is the Most Common Prescribing Quality Problem

RAND researchers report that failure to prescribe an indicated treatment is a far more common quality problem than is inappropriate medicine use.

Quality Problems Among Vulnerable Older Patients

- Failure to prescribe when called for by guidelines: 50%
- Inadequate monitoring: 36%
- Inadequate education/continuity/documentation: 19%
- Inappropriate medication: 3%

*Quality indicators were developed and implemented based on systematic literature reviews and multiple layers of expert judgment.

Source: RAND Health

*Quality indicators were developed and implemented based on systematic literature reviews and multiple layers of expert judgment.
Diabetes: An Example of Underdiagnosis and Undertreatment

Uncontrolled diabetes can lead to kidney failure, amputation, blindness, and stroke.

Sources: CDC⁴; Analysis based on National Health and Nutrition Examination Survey (NHANES)⁵
Better Use of Medicines Improves Patient Health

Diabetes patients who take their medicines as prescribed experience fewer diabetes-related complications.

<table>
<thead>
<tr>
<th>Condition</th>
<th>Nonadherent Patients</th>
<th>Adherent Patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>Acute Myocardial Infarction</td>
<td>4.0%</td>
<td>1.8%</td>
</tr>
<tr>
<td>Amputation/ Ulcer</td>
<td>8.0%</td>
<td>4.0%</td>
</tr>
<tr>
<td>Cerebrovascular Disease</td>
<td>10.1%</td>
<td>7.8%</td>
</tr>
<tr>
<td>Neuropathy</td>
<td>11.8%</td>
<td>10.8%</td>
</tr>
<tr>
<td>Renal Events</td>
<td>10.8%</td>
<td>5.8%</td>
</tr>
<tr>
<td>Retinopathy</td>
<td>15.7%</td>
<td>13.0%</td>
</tr>
</tbody>
</table>

Source: T.B. Gibson, et al.6
Recommended Medicines Can Save Lives and Dramatically Improve Health

“...achieving effective blood pressure control would be approximately equivalent to eliminating all deaths from accidents, or from influenza and pneumonia combined.”
— David Cutler, Ph.D., Harvard University

**Annual Hospitalizations and Deaths Avoided Through Use of Recommended Antihypertensive Medications**

<table>
<thead>
<tr>
<th>Prevention Achieved:</th>
<th>Annual Hospitalizations Avoided</th>
<th>Annual Premature Deaths Avoided</th>
</tr>
</thead>
<tbody>
<tr>
<td>Based on Current Treatment Rates</td>
<td>833,000</td>
<td>86,000</td>
</tr>
<tr>
<td>Potential Additional Prevention:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>If Untreated Patients Received</td>
<td>420,000</td>
<td>89,000</td>
</tr>
<tr>
<td>Recommended Medicines</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Source: D.M. Cutler, et al. 7
Prescription Medicines Are Part of the Solution to Reducing Medical Spending

Better use of medicines reduces use of avoidable medical care, resulting in reductions in medical spending.

**Adherence to Medicines Lowers Total Health Spending for Chronically Ill Patients**

<table>
<thead>
<tr>
<th>Condition</th>
<th>Drug Spending</th>
<th>Medical Spending</th>
</tr>
</thead>
<tbody>
<tr>
<td>Congestive Heart Failure</td>
<td>$1,058</td>
<td>$8,881</td>
</tr>
<tr>
<td>Diabetes</td>
<td>$656</td>
<td>$4,413</td>
</tr>
<tr>
<td>Hypertension</td>
<td>$429</td>
<td>$4,337</td>
</tr>
<tr>
<td>Dyslipidemia</td>
<td>$601</td>
<td>$1,860</td>
</tr>
</tbody>
</table>

Source: M.C. Roebuck, et al.⁹
Gaining Drug Coverage Reduced Other Medical Spending

The Medicare drug benefit increased access to medicines for those previously without drug coverage, resulting in reduced non-drug medical spending and overall savings of $13.4 billion in 2007, the first full year of the program.

Sources: J.M. McWilliams, et al.; C.C. Afendulis and M.E. Chernew

*Home health, durable medical equipment, hospice, and outpatient institutional services.

Average Reduction in Medical Spending in 2006 and 2007, for Beneficiaries Gaining Drug Coverage Through Part D

- Part A: -$816
- Part B: -$268
- Other Non-drug*: -$140
- Total Non-drug Medical Spending: -$1,224

Sources: J.M. McWilliams, et al.; C.C. Afendulis and M.E. Chernew
Improving Adherence Could Yield Large Savings

Improving adherence to congestive heart failure (CHF) medicines could result in federal savings of $22.4 billion over 10 years.

Estimated 10-Year Savings to Medicare from Improved Adherence to CHF Medications, 2013–2022

- Additional savings to Medicare if adherence reached recommended levels: $22.4 Billion
- Savings to Medicare from gaining Part D Coverage: $26.9 Billion

Source: T.M. Dall, et al.11
Better Use of Medicines Yields Significant Health Gains and Savings on Other Services

Due to a growing body of evidence, CBO’s budget estimates now recognize reductions in other medical expenditures associated with increased use of prescription medicines in Medicare.

Numerous studies demonstrate that medicines can improve health outcomes and reduce use of other medical services:

- Better adherence to antihypertensive medications could save approximately 200,000 lives over 5 years.\(^{13}\)
- Improved medication adherence among diabetes patients could prevent more than 1 million emergency department visits and hospitalizations annually, for potential savings of $8.3 billion each year.\(^{14}\)
- Nonadherence has also been linked to excess hospitalizations for conditions such as chronic obstructive pulmonary disease,\(^{15,16}\) osteoporosis,\(^{17}\) congestive heart failure, hypertension, diabetes, and dyslipidemia,\(^{18,19,20}\) with costs of roughly $170 billion per year.\(^{21}\)

> 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.”

— Congressional Budget Office\(^{12}\)

High Cost Sharing Reduces Adherence

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

Source: D.P. Goldman, et al.22

Percent Change in Adherence from Doubling Medicine Co-Pays

-45% -44% -34% -33% -32% -26% -26% -25%

Percent Change in Days Supplied of Medicine

Source: D.P. Goldman, et al.22
Patients Facing High Cost Sharing Commonly Abandon Their Medicines

Patients taking medicines on a specialty tier, such as medicines to treat rheumatoid arthritis and multiple sclerosis, face higher out-of-pocket expenses than patients taking medicines not on this tier. These patients also commonly abandon their medicines at the pharmacy.

*Select therapies include those treating patients with Parkinson's disease and dyslipidemia.

Source: A. King and L. Mitchell[23]
Improving Adherence Increases Productivity

Adherent patients miss fewer days of work and experience less short-term disability. For workers with Asthma/COPD alone, adherence averages over $3100 in savings per worker annually.

Fewer Days of Absence and Short-Term Disability for Adherent Patients as Opposed to Nonadherent Patients

Source: G.S. Carls, et al.24
5. IHS Global Insight analysis based on 2010 National Health and Nutrition Examination Survey (NHANES).
Notes and Sources


Informing Consumers and Providers About Medicines

Biopharmaceutical marketing and promotion are important and extensively regulated ways of informing consumers and health care professionals about medicines.

Biopharmaceutical company representatives help speed the dissemination of advances in medical care, and many physicians value this information.

Direct-to-consumer advertising (DTCA) by biopharmaceutical companies often leads patients to seek additional information and consult their doctors about previously untreated conditions; it also informs patients about medicines’ risks and benefits.

While effective communication can increase awareness of medical treatment options, other factors, including formulary design and utilization management strategies, often have a greater impact on prescribing decisions.
Many Factors Affect Physicians Prescribing Decisions

**Factors Influencing Prescribing Decisions in the United States in 2013**

- **Clinical knowledge and experience**
  - A great deal: 91%
  - Some: 8%

- **Patient's particular situation, including drug interactions, side effects, and contraindications**
  - A great deal: 89%
  - Some: 9%

- **Articles in peer-reviewed medical journals**
  - A great deal: 50%
  - Some: 42%

- **Clinical practice guidelines**
  - A great deal: 48%
  - Some: 44%

- **Patient's insurance coverage and formulary**
  - A great deal: 39%
  - Some: 44%

- **Information from colleagues and peers**
  - A great deal: 38%
  - Some: 54%

- **Pharmaceutical company-sponsored educational programs featuring physician speakers, not CME**
  - A great deal: 11%
  - Some: 47%

- **Information from pharmaceutical company representatives**
  - A great deal: 10%
  - Some: 53%

- **Information from insurance and prescription benefits manager representatives**
  - A great deal: 10%
  - Some: 35%

Source: KRC Research¹
Physicians Find Biopharmaceutical Representatives’ Information Up to Date, Useful, and Reliable

Physicians’ Assessment of Biopharmaceutical Representatives’ Information

- **Up to date and timely**
  - Strongly agree: 43%
  - Somewhat agree: 52%
  - Total: 94%

- **Useful**
  - Strongly agree: 22%
  - Somewhat agree: 71%
  - Total: 92%

- **Reliable**
  - Strongly agree: 22%
  - Somewhat agree: 55%
  - Total: 84%

Source: KRC Research^2
Direct-to-Consumer Advertising Often Prompts Patients to Seek Additional Information

Consumer Responses to Viewing Advertisements for Prescription Medicines

- 47% Sought Information
- 27% Initiated Conversation with Doctor
- 14% Newly Aware of Medical Condition
- 8% Requested Specific Medication

Source: Princeton Survey Research Associates International
Direct-to-Consumer Advertising Increases Awareness of the Benefits and Risks of New Medicines

Awareness of Benefit and Risk Information Among People Who Saw an Advertisement on TV

Source: Prevention Magazine National Survey Data
Physicians Respond to Patients’ Requests for Specific Treatments with a Broad Range of Alternatives

When Asked by a Patient About a Specific Treatment, Physicians Frequently...

- Recommend lifestyle or behavior changes: 50%
- Recommend over-the-counter drug: 18%
- Recommend no treatment: 14%
- Recommend a different prescription drug: 14%
- Give prescription for requested drug: 5%

Source: Kaiser Family Foundation
Direct-to-Consumer Advertising Encourages Appropriate Use of Medicines

Study finds Direct-to-Consumer Advertising (DTCA) promoted appropriate use of oral breast cancer therapies and did not promote inappropriate use.*

Medically Appropriate Population:
Breast cancer patients age 60+

DTC advertising of aromatase inhibitors

Other breast cancer patients (medically inappropriate population) — less than 60 y.o.

Doctor

Significant increase in prescribing of aromatase inhibitors to medically appropriate population

No increase in prescribing of aromatase inhibitors to medically inappropriate population

*Study measured the effect of DTCA on patients and doctors regarding the use of aromatase inhibitors (AIs) consistent with medical practice guidelines. Study found that DTCA spending on AIs was associated with an overall new AI prescription increase of 0.18% after 3 months (approximately 118 new AI prescriptions per million dollars spent). There was “no significant change associated with DTCA spending for AIs for those aged 40 years or less at any time from 0 to 6 months.”

Source: G.A. Abel, et al.6
Patients Benefit from Direct-to-Consumer Advertising

DTCA increases the likelihood that patients will seek and receive treatment for undiagnosed conditions.

*“Consumer welfare” refers to the population-wide net value to consumers (patients) from their use of health care services.

Source: J. Jayawardhana

- A study examining DTCA and cholesterol-reducing medications found that patients between the ages of 50 and 70, who are more likely to have high cholesterol levels, experienced the greatest welfare gains of 12–13% of the average cost of a prescription.

- The study’s author notes that “DTCA helps bring the under-diagnosed consumers to physicians' offices, which in turn helps to improve consumer welfare.”
According to Government and Academic Experts, Marketing Costs Do Not Add to Prescription Drug Prices

“[Direct-to-consumer advertising] can empower consumers to manage their own health care by providing information that will help them, with the assistance of their doctors, to make better informed decisions about treatment options.... Consumers receive these benefits from DTC advertising with little, if any, evidence that such advertising increases prescription drug prices.”

— Federal Trade Commission

“One sometimes hears it said that the industry would have more money for R&D if it would cut down its marketing costs. This comment reflects misunderstanding of the economics of the industry. If a firm did so, it would be less profitable and would attract less capital for R&D or would have fewer internally generated funds to invest [in R&D].”

— J.P. Newhouse, Harvard University

Sources: Federal Trade Commission; J.P. Newhouse
Notes and Sources


2. Ibid.


ECONOMIC IMPACT

The Biopharmaceutical Sector’s Role in the Economy

America’s biopharmaceutical research companies serve as the foundation for one of the country’s most dynamic innovation and business ecosystems. The biopharmaceutical industry is among the most R&D-intensive industries in the United States and accounts for 20% of all domestic R&D funded by U.S. businesses. The industry and its large-scale research and manufacturing supply chain supports high-quality jobs across the U.S. economy.

American biopharmaceutical research companies are leaders in charitable contributions, including significant support for education efforts in science, technology, engineering, and mathematics (STEM).
The Biopharmaceutical Sector is the Single Largest Funder of Business R&D in the United States

The biopharmaceutical sector accounts for the single largest share of all U.S. business R&D, representing nearly 20% of all domestic R&D funded by U.S. businesses.

*The remaining 43% share of business R&D spending is conducted by other industries including subsectors of the machinery sector, the electrical equipment sector, and the professional, scientific, and technical services sector among others.

Source: National Science Foundation

Share of Total U.S. Business R&D by Industry, 2010*

- Pharmaceuticals and Medicines: 20%
- Software: 12%
- Semiconductors: 10%
- Automobiles: 5%
- Communications Equipment: 5%
- Aerospace: 5%
The Biopharmaceutical Sector is the Most R&D-Intensive in the United States

Biopharmaceutical companies invested more than ten times the amount of R&D per employee than manufacturing industries overall.

**R&D Expenditures per Employee, by Manufacturing Sector, 2000–2010**

- **Pharmaceuticals and medicines**: $104,567
- **Communications equipment**: $63,975
- **Semiconductors**: $46,888
- **Aerospace products**: $19,772
- **Motor vehicles, trailers, parts**: $15,294
- **All manufacturing**: $11,222

Source: National Science Foundation

2
The Economic Reach of the U.S. Biopharmaceutical Industry

<table>
<thead>
<tr>
<th>Direct Jobs</th>
<th>Indirect Jobs</th>
<th>Induced Jobs</th>
<th>Total Jobs</th>
</tr>
</thead>
<tbody>
<tr>
<td>814,000</td>
<td>1,022,000</td>
<td>1,528,000</td>
<td>3,364,000</td>
</tr>
</tbody>
</table>

The biopharmaceutical industry supported 3.4 million jobs across the U.S. economy in 2011.

Source: Battelle Technology Partnership Practice³
U.S. Leads in Biopharmaceutical Intellectual Property

The intellectual property related to more than half of new medicines resides in the United States.

U.S. Patents Granted in Pharmaceutical Technology, Selected Years 1997–2012, Location of Inventor by Region/Country

- United States, 52.6%
- European Union, 25.9%
- Japan, 9.9%
- All Others, 7.3%
- China, 0.7%
- Asia, * 3.6%

*Asia includes India, Malaysia, Singapore, South Korea, Taiwan and others (but excludes China and Japan).

Source: National Science Foundation
U.S. Biopharmaceutical Exports Have Grown

Biopharmaceutical exports have nearly doubled over the 10-year period between 2004 and 2013, accounting for 3.2% of all U.S. exports by 2013.

Source: U.S. Dept. of Commerce, International Trade Administration

*Estimated

Source: U.S. Dept. of Commerce, International Trade Administration

6 • Economic Impact
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.”
— F.M. Scherer, AEI-Brookings Joint Center for Regulatory Studies

“...the 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 Office

“Usual profit figures greatly overstate the industry’s economic profit rate.”
— J.P. Newhouse, Harvard University

Sources: F.M. Scherer; CBO; J.P. Newhouse
Biopharmaceutical Industry Advancing STEM Education in the U.S.

The science, technology, engineering, and mathematics (STEM) workforce accounts for over 50% of the nation’s sustained economic growth. Over the past 5 years, PhRMA member companies and their foundations have supported over 90 STEM education programs across the U.S., impacting over 1.6 million students and 17,500 teachers.

*PhRMA member company and foundation contributions to STEM education in the U.S. include:*

- Number of employee volunteers: 4,500
- Number of employee hours volunteered: 27,000
- Percentage of Industry-sponsored STEM programs focused on K-12: 85%
- Number of individual STEM-related grants awarded: 600
- Total STEM education program funding provided: $100M

Source: Battelle Technology Partnership Practice®
### Biopharmaceutical Companies Lead Corporate Giving

Biopharmaceutical companies led corporate giving* in 2011. Nearly 90% of these contributions were in the form of in-kind product donations.

<table>
<thead>
<tr>
<th>Average Corporate Giving by Sector</th>
<th>Total Giving as % of Pre-Tax Profit</th>
<th>Total Giving per Employee</th>
</tr>
</thead>
<tbody>
<tr>
<td>All Companies</td>
<td>1.0%</td>
<td>$695</td>
</tr>
<tr>
<td>Biopharmaceuticals</td>
<td>13.0%</td>
<td>$18,273</td>
</tr>
<tr>
<td>Energy</td>
<td>0.5%</td>
<td>$2,766</td>
</tr>
<tr>
<td>Utilities</td>
<td>1.1%</td>
<td>$1,027</td>
</tr>
<tr>
<td>Information Technology</td>
<td>0.8%</td>
<td>$702</td>
</tr>
<tr>
<td>Consumer Staples</td>
<td>1.2%</td>
<td>$673</td>
</tr>
<tr>
<td>Industrials</td>
<td>0.7%</td>
<td>$255</td>
</tr>
</tbody>
</table>

*Domestic giving makes up the largest portion of total corporate giving across all sectors surveyed. Domestic giving comprised 79% of total giving in 2012.

Source: Committee Encouraging Corporate Philanthropy
Notes and Sources


