THE CHALLENGE OF DEVELOPING NEW TREATMENTS & CURES

Innovative medicines offer great hope to patients and the health care system, but developing these new treatments and cures is a complex and risky undertaking.

- The average amount of time it takes for a new medicine to go through the entire R&D process: 10 years
- The average cost to develop a new medicine: $2.6 billion
- The percentage of drugs entering clinical trials that result in an approved medicine: 12 percent

BETWEEN 1998 AND 2014:

The number of unsuccessful vs. successful attempts to develop medicines to treat certain diseases

<table>
<thead>
<tr>
<th>Disease</th>
<th>Unsuccessful Attempts</th>
<th>Approved Medicines</th>
</tr>
</thead>
<tbody>
<tr>
<td>ALZHEIMER'S DISEASE</td>
<td>123</td>
<td>4</td>
</tr>
<tr>
<td>MELANOMA</td>
<td>96</td>
<td>7</td>
</tr>
<tr>
<td>LUNG CANCER</td>
<td>167</td>
<td>10</td>
</tr>
</tbody>
</table>

- The number of medicines currently in development around the world: more than 7,000
- The percentage of new medicines in development that are potential first-in-class therapies, meaning they use a completely new approach to fighting a disease: 70 percent
- The percentage of new medicines in the pipeline that have the potential to be personalized medicines: 42 percent

Learn more about the cost and value of medicines at www.phrma.org/cost
WHAT IS THE MEDICAID DRUG REBATE PROGRAM?

Designed to help ensure coverage of prescription medicines for Medicaid enrollees, the Medicaid Drug Rebate Program requires pharmaceutical manufacturers to have agreements in place to provide rebates in exchange for Medicaid coverage of the manufacturers’ prescription medicines both for patients enrolled in fee-for-service and Medicaid managed care.

MINIMUM REBATES ARE SPLIT BETWEEN

- Federal Government
- State Medicaid Programs

ENSURING MEDICAID RX COVERAGE

600

Approximately 600 companies are currently participating in the program.

HOW DO DRUG REBATES WORK?

- **Fee-for-Service Rebates**
  - Patient
  - Medicine A
  - State Medicaid Program pays for Medicine A
  - Drug manufacturer pays
  - Minimum Rebate
  - Federal Government
  - State Medicaid

- **Medicaid Managed Care Organization (MCO)**
  - A private insurer that administers Medicaid for the state
  - State Medicaid Agency pays Medicaid MCO as part of a capitated rate
  - Drug manufacturer pays
  - Minimum Rebate
  - Federal Government
  - State Medicaid

DID THE ACA CHANGE REBATES?

The Affordable Care Act (ACA) included a number of changes to the Medicaid Drug Rebate program, most notably by extending Medicaid rebates to Medicaid MCO prescriptions and increasing minimum rebate amounts.

MINIMUM REBATES UNDER ACA

Percentage of the average manufacturer price

- **Innovative, Brand Name Medicines**
  - Pre-ACA: 15.1%
  - Post-ACA: 23.1%
  - Increase: 8%

- **Non-Innovative, Generic Drugs**
  - Pre-ACA: 11%
  - Post-ACA: 13%
  - Increase: 2%

WHAT NOW?

Manufacturers report the amount of rebates being paid to the states & federal government.

CONCLUSION

Prescription medicines are critical to helping patients live longer, healthier lives and are not a main driver of health care cost growth.

$86 BILLION

Total value of rebates paid by manufacturers, 2010-2014*

*Second half of 2014 projected as continuation of Q2 2014 volume and costs. Rebates for 2014 projected at same percentages used for 2013 brand and generics.

www.PhRMA.org
Spending on retail prescription medicines has consistently accounted for just 10 percent of health care spending – a figure that is projected by government actuaries to remain stable through the next decade. This is possible because of high utilization rates of generics, competition among brand-name medicines and aggressive tactics by insurers and pharmacy benefit managers to negotiate lower prices.

Recent activity with hepatitis C medicines highlights the uniqueness of this marketplace. When new innovative hepatitis C medicines with cure rates of more than 90 percent were recently approved, insurers and pharmacy benefit managers made exaggerated claims about the cost impact these medicines would have on the U.S. health care system – claims that were refuted once competition among brand-name hepatitis C medicines resulted in significant cost savings.

We previously saw the competitive marketplace work with HIV medicines, it has been evident with recent activity in the hepatitis C market, and we expect to see similar outcomes with new classes of cholesterol medicines that are on the horizon. As we continue to discuss costs, it is important to understand the key parts of our nation’s distinctive competitive marketplace:

**Companies race to be first to market with a new medicine.**

Medicines race to be first to the market. It is a race because most medicines already have competitors also in development. This race closes quickly after medicines are approved and generally there is competition from another brand in less than two years. Once these patents end, brand medicines then face additional competition from lower-cost generics. According to an article in *Forbes*, in 2012 alone, the sales value of medicines coming off patent was $33 billion and another $47.5 billion is estimated this year.

**Innovator companies produce medical advances that lead to low-cost generic copies.**

Innovator companies invest in pioneering research to bring new treatments to patients, and over time those medicines become available as lower-cost generic copies. Currently nearly 90 percent of all medicines prescribed to U.S. patients are generics. Because significant research and development resources are not required for the manufacturing of generic medicines, the cost of a generic medicine is typically up to 80 percent less than that of the brand medicine.

**Insurers and pharmacy benefit managers aggressively negotiate the prices of medicines.**

Medicines are purchased in a uniquely competitive market, where concentrated purchasers use more aggressive cost containment tools than for other parts of the U.S. health care system. In fact, Express Scripts has publicly stated it will save the U.S. $4 billion annually as a result of its aggressive negotiations for hepatitis C medicines. The irony is that they excluded such savings in their recent public estimate of drug spending. This is surprising since public reports indicate that private payers are receiving discounts of up to 40 percent for hepatitis C medicines.

Our nation’s competitive marketplace is why we have seen such tremendous – and sustainable – progress against hepatitis C, cancer and other challenging diseases.
Prescription medicines account for just 4-5 percent of total Medicaid spending and this share is projected to remain stable through the next decade, even as important advances, such as those made against hepatitis C continue. That’s because competition among brand-name medicines and aggressive tactics by insurers to negotiate prices all help to keep costs under control.

This is why we have seen state Medicaid programs negotiate steep discounts, on top of the statutorily mandated 23.1 percent rebate, that have further reduced the cost of hepatitis C treatment by 40-65 percent.

Discussions on spending on medicines in Medicaid programs need to include the following facts:

**Prescription medicines represent a small share of Medicaid spending and are not a main driver of Medicaid spending growth.**

Prescription medicines account for just 4-5 percent of total Medicaid spending. The most recent National Health Expenditure estimates project that between 2014 and 2023 prescription medicines will account for just 5.1 percent of Medicaid spending growth. This is critical to keep in mind as Medicaid spending on hospitals over the next decade is projected to total $140.2 billion, more than three times total spending on prescription medicines. Over this same period, nursing home and home health care are projected to grow by a combined $65 billion, underscoring the vital need for new medicines that can help people stay healthier longer as our nation ages.

**Medicaid costs for the newest hepatitis C medicines have been greatly overstated.**

Claims that state spending for new hepatitis C medicines could exceed $55 billion are wildly exaggerated. Medicaid covered an estimated 50,394 prescriptions for sofosbuvir in 2014, which represents 3.2 percent of Medicaid prescription drug spending (net of Medicaid rebates) and less than a quarter a percent of annual Medicaid costs. And some spending on the new generation of medicines is replacing prior spending on older drugs and health services previously used to treat hepatitis C and its effects. For example, in 2012 Medicaid spent approximately $443 million on the first generation of direct acting medicines for hepatitis C.

**Reported discounts for hepatitis C treatments demonstrate the significant savings that are provided by market competition.**

Discussions on spending on medicines too often focus solely on the list price and ignore publicly reported discounts. Jeff Myers, chief executive of Medicaid Health Plans of America, recently characterized the increased competition as positive noting that “market forces are beginning to take hold.” Leveraging increased competition in the hepatitis C market, state Medicaid programs are negotiating deep discounts with manufacturers, on top of statutorily mandated rebates, that reduce the cost of treatment by 40-65 percent.

**New and forthcoming hepatitis C treatments represent a remarkable advance against a very serious disease.**

The newest hepatitis C treatments have a cure rate of more than 90 percent against a disease that kills more patients than HIV/AIDS, is the leading cause of liver transplants and is the primary driver of the increase in liver cancer. Individuals with hepatitis C have a complex disease requiring advanced medical care. The cost of treating commercially insured patients with hepatitis C averages 5.5 times the cost of commercially insured patients overall. Hospitalization costs alone for hepatitis C patients with
advanced liver disease increased 44 percent between 2004-5 and 2010-11 – averaging nearly $35 billion in the 2011. Without these treatments, annual medical costs to treat patients with hepatitis C have been projected to nearly triple, from $30 billion to $85 billion over the coming decades, because of increased hospitalizations and more patients suffering with advanced liver disease.

**Medicaid agencies and patients need a system that supports and encourages innovation and development of new treatments for Alzheimer's and other costly conditions.**

We need a robust innovation ecosystem that permits the ability to recoup investment not just for the few medicines that successfully make it to market, but also for the many others that don’t. The research and development process is challenging and characterized by many failures, for example:

+ Research in Alzheimer’s disease has resulted in 123 unsuccessful drug candidates and just 4 new drug approvals between 1998 and 2014.
+ In melanoma, there have been 7 new approvals since 1998, but 96 unsuccessful drug candidates.
+ In lung cancer, there have been 10 new approvals since 1998, but 167 unsuccessful drug candidates.
+ In brain cancer, there have been 3 new approvals since 1998, but 75 unsuccessful drug candidates.
THE FIVE ESSENTIAL TRUTHS ABOUT PRESCRIPTION DRUG SPENDING

Every day patients around the world are living healthier, more productive lives thanks to innovative medicines developed by biopharmaceutical companies. Retail prescription medicines have consistently accounted for just 10 percent of United States health care spending and federal actuaries project this share will remain stable through the next decade.

At the same time, these medicines have helped to curb downstream health care system costs by reducing spending on expensive hospitalizations and long-term care. Any discussion of prescription drug spending needs to consider the following five facts:

1. Government data shows that retail prescription medicines will continue to account for just 10 percent of U.S. health care spending through the next decade.
   Contrary to recent rhetoric, spending on prescription medicines accounts for the same percentage of health care spending today as in 1960 – just 10 percent. In fact, the most recent national health spending projections show that it is expected to grow at rates in line with overall health care spending through 2023. This share is expected to remain stable for at least the next decade, even as important advances continue in areas such as cancer, hepatitis C, and rare diseases. To put this in context, private insurers spent roughly as much on drugs as on administrative costs in 2013 and the U.S. will spend $13.6 trillion on hospital care over the next decade, more than three times total spending on prescription medicines.

2. 2014 was an unusual year, in which roughly 10 million of uninsured patients gained coverage, and a record number of new medicines were approved.
   Increases in health care costs were expected in 2014, given the expansion of insurance coverage under the Affordable Care Act. In addition, the U.S. Food and Drug Administration’s Center for Drug Evaluation and Research (CDER) approved a record number of 41 new medicines, of which nearly 41 percent were first in class treatments, and more than 20 percent were personalized medicines. Among the new medicines are innovative cancer treatments with the potential to prolong and transform patients’ lives, therapies for hepatitis C with cure rates of more than 90 percent, and a record-setting 17 medicines to treat rare diseases. The latest official government projection for spending growth for retail drugs in 2014 is 6.8 percent (slightly higher than, but in-line with overall healthcare spending growth of 5.6 percent). IMS Institute for Healthcare Informatics projects that after spiking in 2014 at 13.1 percent, growth will moderate in the next few years to 5-8 percent – back in line with health spending. Neither IMS nor National Health Expenditures has published actual figures for 2014.

3. Focusing on a small subset of medicines and excluding rebates is misleading.
   Private payers are receiving discounts of up to 40 percent for hepatitis C medicines. In addition, a recent actuarial analysis of pharmacy benefit manager drug trend reports show that trends for “specialty” medicines are frequently misleading, and often use inconsistent definitions and methods which can inflate and bias reported trends.

Every day patients around the world are living healthier, more productive lives thanks to innovative medicines developed by biopharmaceutical companies. Retail prescription medicines have consistently accounted for just 10 percent of United States health care spending and federal actuaries project this share will remain stable through the next decade.
4. **Medicines can play a crucial role in controlling future health care costs.**

Many medicines shift the treatment paradigm toward prevention by allowing patients to avoid expensive hospital and long-term care. And every additional dollar spent on medicines for adherent patients with congestive heart failure, high blood pressure, diabetes and high cholesterol generated $3 to $10 dollars in savings on emergency room visits and inpatient hospitalizations. Researcher findings from University of Chicago establish that a 10 percent decrease in the cancer death rate is worth roughly $4.4 trillion in economic value to current and future generations. By preventing the need for expensive hospital, emergency, or long term care, medicines can reduce the growth in health care spending, and in some cases, result in savings. For example, based on a large body of research showing that better use of medicines can reduce spending on other medical services, the Congressional Budget Office (CBO) now credits Medicare policies that increase use of medicines with savings on other Medicare costs.

5. **Medicines help patients live longer, healthier lives.**

In many cases, research and medicines from the biopharmaceutical sector are the only chance for survival for patients and their families. Medicines have helped raise average U.S. life expectancy from 47 years in 1900 to 78 years. Since its peak in 1991, the cancer death rate in the U.S. has fallen 22 percent and 2 out of 3 patients diagnosed with cancer are now living at least 5 years following diagnosis. And new hepatitis C therapies have cure rates above 90 percent and dramatically decrease the burden of the disease on the U.S. health care system and the economy.