

# A NEW ERA OF BIOPHARMACEUTICAL INNOVATION

How medicines prevent disease,  
transform lives and create a  
healthier America

PhRMA  
FORUM

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# Our industry's commitment to a healthier America

## Letter from PhRMA CEO Stephen J. Ubl and Board Chair Daniel O'Day

The path to a healthier America begins with addressing disease. Disease threatens our health and well-being, burdens our health care system and limits the potential of our economy. Most devastatingly, it robs individuals of their potential – the ability to live full lives, pursue dreams and contribute to their communities.

Over the past 25 years, groundbreaking medical innovations have transformed health care and, in the process, given hope to patients and their families. We've developed therapies that can cure hepatitis C, helped turn HIV/AIDS into a manageable condition, cured many cases of childhood leukemia, prevented millions of illnesses with vaccines, helped revolutionize cancer treatment with cell therapies and developed new treatments to fight obesity and prevent many related chronic conditions. These advances show us the immense promise of medicines to change lives for the better.

Yet we have much more to do. Alzheimer's disease, cancer, heart disease and stroke, infectious diseases, immune system diseases and rare and genetic diseases, among others, represent profound challenges. The toll of these illnesses will continue to impact patient lives and overwhelm our health system unless we can change the trajectory of the diseases themselves.

**Our industry is committed to making big, bold investments on medicines that will change the course of disease for all of us – because all of us are patients at some point. We are driven to take on the diseases that are the most serious, the most challenging and those that create the greatest burden for the health care system. This is the work that thousands of scientists are doing every day in laboratories across the country. Together, we are working towards a healthier America.**

This work makes America the leader in biopharmaceutical innovation – and it's only possible because of our unique ecosystem and policies that are conducive to investment and progress. America can and should continue to be the global leader as we enter a new era of transformational innovation, built on the incredible progress we have made and spurred by the new technologies available today.

This report gives a brief snapshot of how far we've come over the last 25 years and a glimpse of how we believe medicines can help to solve the biggest challenges we face in health care. This is only the beginning. We understand how much the country is counting on new medicines to meet their needs – and we will deliver.



■ *Stephen J. Ubl*

**Stephen J. Ubl**

President and CEO, PhRMA



■ *Daniel O'Day*

**Daniel O'Day**

Chairman and CEO, Gilead Sciences

# Looking Back: 25 Years of Transformational Medicine

In the past 25 years, scientific advancements have changed the lives of patients, their families and communities in profound ways. Medicines developed by America's biopharmaceutical companies are transforming how we fight disease – harnessing the power of our own immune systems to fight cancer, bringing gene therapies to patients with rare genetic diseases, developing new platform technologies to respond to health threats much more quickly and reducing the burden of chronic diseases for millions.

But we haven't done it in a vacuum. This progress is a testament to and a result of the United States' global leadership in biopharmaceuticals and the ecosystem that has been built up to make it possible. This leadership is not accidental, nor is it guaranteed. It has been enabled by a predictable regulatory environment, strong intellectual property protections and a functioning market-based pricing system for new medicines.

## ■ Medicines are key to many of the most important health care advances of the past 25 years.

Biopharmaceutical innovation over the last 25 years has led to significant advancements in health care and improvements in patient outcomes, especially for patients who previously had no treatment options. Industry-led advances have also allowed us to intervene earlier in the disease cycle, halting disease progression and transforming patient care.

### A FEW EXAMPLES OF PROGRESS IN THE FIRST QUARTER OF THE 21ST CENTURY



Overall, **cancer mortality rates have declined** an astounding 34% between 1991 and 2022, driven in part by improved therapeutics as well as public health initiatives, leading to the prevention of roughly 4.5 million deaths.<sup>1</sup> Between 2000 and 2016 alone, new cancer medicines were estimated to be associated with 1.3 million prevented cancer deaths.<sup>2</sup>



New medicines introduced in the last 10 years have made **substantial progress in meaningfully reducing the risk of hospitalization and death from heart failure.**<sup>3</sup>



**New medicines to address the country's mental health crisis have come to market**, including an important new treatment from the first novel class of medicines for schizophrenia in many decades, offering hope for patients who did not respond to or could not tolerate prior antipsychotics.



New GLP-1 treatments for diabetes and obesity – two conditions that collectively kill hundreds of thousands of people and cost the nation trillions each year – could also prevent associated illnesses like heart, liver and kidney disease,<sup>4,5</sup> sleep apnea<sup>6</sup> and some cancers.<sup>7</sup> **Medicines are not only treating chronic diseases but helping to prevent them in the first place.**<sup>8,9</sup>



“Thanks to our nation’s incredible biopharmaceutical researchers who work tirelessly to develop treatment options for patients like me, I am able to have a second chance at life. I am blessed to say that now over 25 years later, I am still cancer-free.”

**Terry**  
Patient

■ CASE STUDY

## Immunotherapy is increasing survival rates and improving quality of life for cancer patients.

As recently as the early 2000s, the promise of immunotherapy – a type of medicine that uses the body’s own immune system to fight diseases – in treating cancer was not widely accepted or well-understood, and only a few immunotherapy drugs were FDA approved.

In the years since, immunology has revolutionized the field of oncology and shifted the paradigm of cancer care, with the introduction of multiple approaches including therapeutic cancer vaccines, immune checkpoint inhibitors, CAR-T cell therapies, cytokines and oncolytic viruses.<sup>10</sup> More than 30 immunotherapy drugs have been approved for 25 cancers since 2010.<sup>11</sup>

Today, immunotherapies have wide applicability across many indications and are improving and extending the lives of cancer patients through better outcomes and reductions in mortality. A robust pipeline of more than 1,000 immunotherapies in clinical trials is offering hope to transform the treatment of many forms of cancer.<sup>12</sup>



■ CASE STUDY

## Changing the game: CAR-T helps track down and eliminate cancer cells.

The advent of genome sequencing, spurred by the Human Genome Project completed in 2003, enabled the development of medicines based on a patient's individual characteristics.

One example is CAR-T. CAR T-cell therapy is a type of immunotherapy that uses a patient's own T-cells to treat cancer. The patient's T-cells are removed and then modified in a lab to produce proteins called chimeric antigen receptors (CARs). Hundreds of millions of these cells are then infused back into the patient's body to hunt down and kill cancer cells.

Today, nearly 800 CAR-T assets in the pipeline<sup>13</sup> are aiming to improve patient outcomes with heightened efficacy and safety and reduced manufacturing times.<sup>14</sup>



"During my residency, I cared for my first patient who was responding to a novel immunotherapy in a clinical trial, offering hope for a previously untreatable cancer. Nearly 15 years later, the strides in cancer treatment remind me of how far we've come and the long journey ahead in turning science into life-saving reality."

**Michael Ybarra, MD**  
Chief Medical Officer, PhRMA

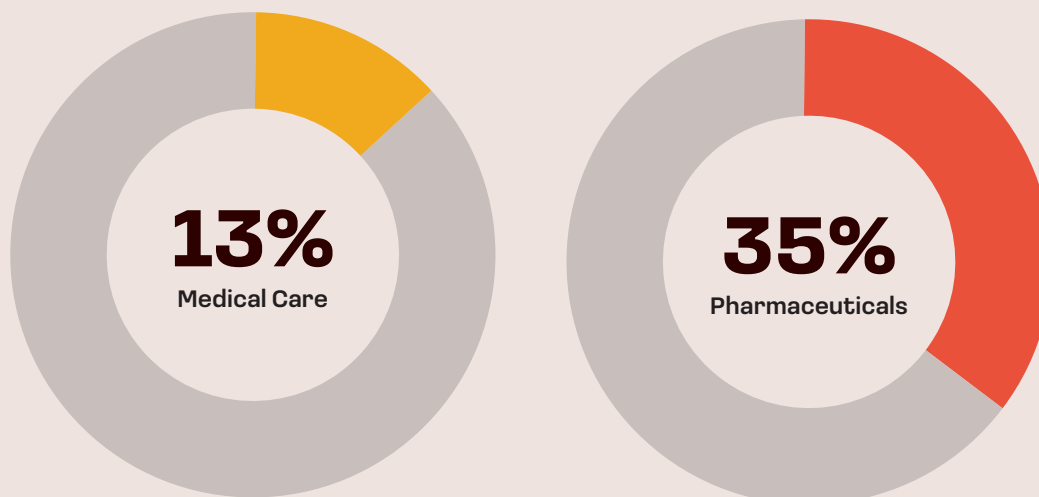
## ■ Biopharmaceutical innovation is a valuable tool to help Americans live healthier lives.

In the last two and a half decades, medicines have demonstrated their unmatched value – to the patients who need them, to the health system by preventing more costly interventions and to society as a whole by reducing the burden of disease. Medicines can help prevent the hundreds of billions of dollars that the United States spends each year on untreated chronic diseases by helping patients avoid expensive hospital stays, caregiver costs, physician office visits and other, costlier health care services.

### ■ MEDICINES PROVIDE SIGNIFICANT VALUE IN HEALTH CARE.

One way to assess the value of medicines is to look at their ability to extend life compared to traditional medical care. Research shows that pharmaceuticals generate roughly three times the impact on life expectancy when compared to other kinds of medical care from 1990-2015.<sup>15</sup> Spending on medical care was significantly higher than on medicines during this time period,<sup>16</sup> underscoring the tremendous value medicines have on both patients and the health system when compared to costlier interventions, such as hospital visits. The improvements in health outcomes made over the past 25 years were not only a result of investments in new medicines but were further bolstered by advancements in vaccines and public health initiatives – such as smoking cessation programs – that encouraged healthier living with a greater focus on prevention. As we look towards the future, it's clear that medicines, vaccines and public health programs that promote health and wellness have an important role to play in improving American health – and providing value to the system.

#### MEDICINES GENERATE 3X IMPACT ON LIFE EXPECTANCY



Percentage impact on improved life expectancy from 1990-2015

#### ■ CASE STUDY

## New medicines effectively cured hepatitis C and are estimated to save the health care system \$49 billion over 10 years.

In the early 2000s, hepatitis C (HCV) placed a major burden on patients and the health system. Treatment options had lower cure rates than today<sup>17</sup> and left patients with many side effects and lifelong infection.<sup>18</sup>

The introduction of direct-acting antiviral (DAA) drugs effectively cured chronic HCV with high efficacy rates.<sup>19,20</sup> Although upfront costs are higher, DAA treatments provide cost savings to the health system by reducing the risk of costly complications, such as cirrhosis, liver disease, liver cancer or even liver transplantation. Cures for hepatitis C have been so effective that by 2026, the cumulative savings will reach \$43 billion for the federal government and states.<sup>21</sup>

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#### ■ CASE STUDY

## Preventive and prophylactic HIV therapies increased lifespans by ~20 years and reduced new infections.

When cases of HIV, the virus that causes AIDS, soared in the United States several decades ago, little was known about the virus, medication was nearly nonexistent and AIDS was the top cause of death among men ages 25-44.<sup>22</sup>

Expanded prevention and treatment options, including antiretroviral therapies (ARTs) and preventive prophylactics (PrEP), have since brought down death rates,<sup>23</sup> increased adherence, improved life expectancies and quality of life<sup>24</sup> and paved the way for a future without HIV/AIDS.

Biopharmaceutical researchers are working on next-generation therapies, such as long-acting ART and PrEP, which may reduce administration frequency to as little as once every 3 to 6 months,<sup>25</sup> and have their sights set on curing HIV completely.

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#### ■ CASE STUDY

## Vaccines have a long track record of protecting patient health and providing cost savings – recent advances carry on that tradition.

Vaccines are one of the most cost-effective tools we have for preventing and mitigating diseases and supporting healthy aging.

They reduce the significant burden of disease and mortality associated with infectious diseases to address the historically high unmet patient need. Over the last 30 years, childhood vaccines have prevented more than one million early deaths,<sup>26</sup> and vaccination of children born between 1994 and 2013 will save an estimated \$295 billion in direct costs and \$1.4 trillion in indirect costs.<sup>27</sup>



# Smart policy has made America the 21st century biopharmaceutical leader and enabled faster access to new medicines for Americans.

In the last 25 years, U.S. leadership in global biopharmaceutical innovation has led to Americans having the most medicines launched first, the highest share of new medicines available and the fastest access to new medicines for patients. The United States doesn't have to rely on other countries for the latest medicines because around half of the global pipeline of medicines is being developed here.<sup>28</sup>

- Americans have access to 85% of new medicines, compared to less than 40% for Europeans, on average.<sup>29</sup>
- 68% of novel drug approvals in 2024 were first approved in the United States.<sup>30</sup>
- Coverage of new medicines in other OECD countries is also delayed. Patients in other OECD countries wait an average of 3.4 years longer than U.S. patients for their government health plan to cover new medicines.<sup>31</sup>



"We need to be innovating and staying abreast of all the potential that science has to offer. That's why I think that ensuring there are policies that maintain innovation is so critical."

**Sandra Milan**

Vice President, Project Team Leadership,  
Molecular Oncology, Cenentech

Smart policies enacted over the last several decades have been a key factor in enabling our world-leading innovation ecosystem that relies on strong intellectual property protections and a predictable regulatory environment, rewards risk-taking, attracts the top scientists in the world and fosters industry collaboration with academics and research centers. These include:

+ **Intellectual property protections**  
(e.g., Hatch-Waxman Act, Biologics Price Competition and Innovation Act)

+ **Collaborative ecosystem between government, academia and the private sector**  
(e.g., Bayh-Dole Act, Operation Warp Speed)

+ **Predictable regulatory environment**  
(e.g., FDA user fee programs, including the Prescription Drug User Fee Act)

+ **Incentives for drug development**  
(e.g., Orphan Drug Act, Best Pharmaceuticals for Children Act)

+ **Government programs that support access**  
(e.g., Medicare Part D)

+ **Expedited programs**  
(e.g., Accelerated Approval program, Breakthrough Therapies Designation)

These policies have led to a gold-standard intellectual property and regulatory system that is producing more new medicines and lower-cost alternatives than at any other time in our country's history. This is the result of an ecosystem in which innovation is rewarded, competition facilitated and others, like universities and government, play an important role and benefit from being part of a world-leading sector.



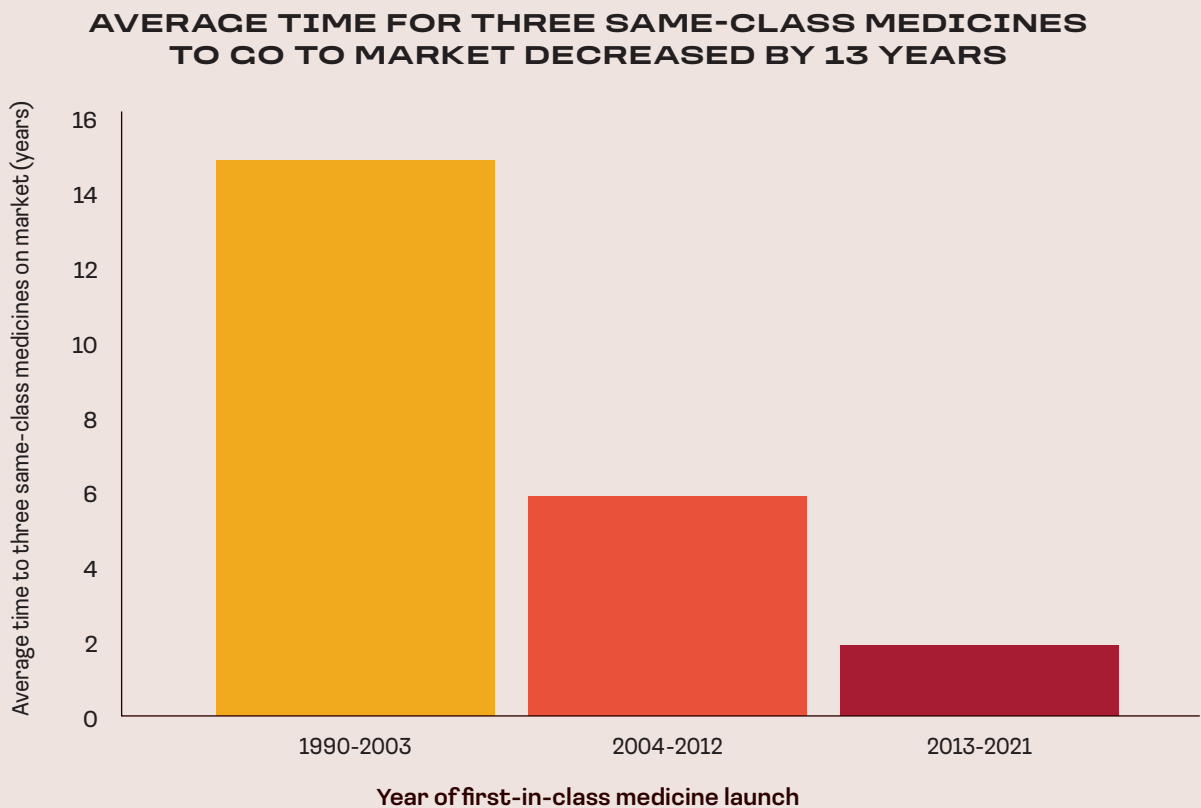
Only 10 drugs were approved for rare diseases before the Orphan Drug Act (ODA) was passed in 1983.<sup>32</sup>

Spurred by the ODA, there has been a 7% increase per year in the rare disease pipeline<sup>33</sup> with approved therapies now available for ~500 rare diseases<sup>34</sup> and candidates for ~600 diseases.<sup>35</sup>

## ■ Intellectual property protections fuel competition and patient choice.

Our intellectual property system balances innovation and affordability, as shown by a robust pipeline of new medicines and broad access to lower-cost generics and biosimilars.

Patents and other forms of intellectual property protections are designed to incentivize biopharmaceutical firms to invest in costly, high-risk R&D without guarantee of success. Our intellectual property framework fuels competition, which drives down costs by encouraging innovators to develop competing brand products different from others already on the market. This not only leads to improvements in any given class but also spurs brand-to-brand competition that further drives savings to the system and patients. Leveraging this robust competition, payers negotiate discounts and rebates, which can lower the price of brand medicines by 50% or more, on average.<sup>36</sup>



+

The average time for three same-class medicines to go to market in the United States fell from ~15 years (1990–2003) to ~2 years (2013–2021).<sup>37</sup>

+

Today, the top drug classes are highly competitive. In 2024, 16 of the top 30 drug classes had more than 4 approved medicines, with some classes (e.g., GLP-1s) expected to double in the number of medicines by 2030.<sup>38</sup>

■ CASE STUDY

## The dynamics of brand-to-brand competition have played out in a number of therapeutic classes.

Following the introduction of a class of treatments for migraine (CCRPs) in 2018, competition lowered the net price of treatment by more than 50% as more drugs launched in the class.<sup>39</sup>

Since the first direct-acting antiviral launched for hepatitis C, net prices have dropped nearly 90%. Similarly, for a class of high cholesterol medicines (PCSK9s), the net price of treatment has dropped by 86% since the first drug launched in the class.<sup>40</sup>

Most recently, in the case of obesity medicines (GLP-1s), the net price of treatment has dropped by 47% since the first launch in the class.<sup>41</sup>



One of the benefits of a strong patent system is that in order to receive patent protection, innovators must publicly disclose their inventions in their patent applications, aiding the market entry of generics and biosimilars after patents and other intellectual property protections expire. The widespread use of generics and biosimilars in the United States offsets spending on newer brand drugs and keeps spending on medicines a small and stable share of overall health care spending.<sup>42</sup> Every generic medicine or biosimilar started out as a patent-protected brand medicine. No company has a monopoly on treating a disease, and promising new medicines often have significant competition from other brand medicines.

**90%**

Today, 90% of prescriptions in the United States are filled with generics, compared to 75% in 2009.<sup>43</sup>

**\$6**

The average patient copay for a generic medicine is around \$6 today.<sup>44</sup>

**\$445B**

The United States saved \$445 billion in 2023 alone from using generics and biosimilars.<sup>45</sup>

■ CASE STUDY

## The development of innovative statins and availability of generics have reduced both near- and long-term health care system costs while improving patient outcomes.

The introduction of statins, along with other cardiovascular medicines, made major strides in addressing heart disease, reducing cardiovascular disease events by 25% and overall mortality by 16%.<sup>46</sup> Innovation has continued with the ongoing development of new statins and other cardiovascular treatments to address unmet patient need.

When the first generic option became available in 2001, followed by many others, statins became more affordable not only for patients but for the entire system.

Statins remain a highly effective treatment for many and bring cost savings through low up-front costs and prevention of serious cardiovascular events. Following the introduction of generics, total cost savings (including direct and indirect costs) from statins have reached \$41 billion per year.<sup>47</sup>

## Critics routinely underestimate the value of medicines.

While it is clear that competitive market forces and strong intellectual property protections consistently lower prices and deliver significant savings to the health care system, misleading narratives about the costs of new medicines to the health care system are common. Many claim that these innovations will bankrupt the health care system, routinely overestimating costs, ignoring substantial rebates and discounts and undervaluing the long-term savings these medicines provide. The evidence points the other way: that medicines generate tremendous value for patients and the health care system.

One example of these dynamics is hepatitis C. When promising, curative treatments first launched in 2014, public and private insurers imposed strict barriers to coverage despite their high cure rates and minimal side effects, meaning few of those diagnosed received timely treatment. In the decade since, hepatitis C treatments have proven their effectiveness and driven extensive cost savings to the system.



# Looking Ahead: Understanding the Health Challenges We Face

While diseases that claimed countless lives in decades past can now be prevented or well-managed, other diseases remain challenging or have emerged as leading threats. These challenges are further compounded by rising system costs and the growing global race for biopharmaceutical leadership.

## ■ AMERICANS ARE FACING MAJOR HEALTH CHALLENGES, AND NEW MEDICINES ARE AN IMPORTANT PART OF SOLVING THEM.

6/10

Six in 10 Americans have 1 or more chronic conditions, and 42% have 2 or more.<sup>48</sup>

42%

Obesity is a chronic disease affecting 42% of adults,<sup>51</sup> increasing the risk of many other health problems. Hundreds of thousands of Americans die from obesity-related conditions every year.<sup>52</sup>

14M

The number of people living with Alzheimer's disease is expected to double by 2050, reaching nearly 14 million people.<sup>49</sup>

1/3

Roughly 1 in 3 young adults aged 17 to 24 are too heavy to join the U.S. military.<sup>53</sup>

40%

40% of Americans will be diagnosed with cancer at some point in their lives.<sup>50</sup>

30M

25-30 million Americans suffer from one of 7,000 rare disorders, yet only about 500 of these disorders have treatments.<sup>54</sup>





## ■ DISEASE IS THE LEADING CAUSE OF RISING HEALTH CARE COSTS.

Rising costs associated with chronic diseases and aging place mounting pressure on our health care system. At the same time, the long-term savings associated with many new medicines is often undervalued, despite evidence showing significant cost savings with the use of generics and biosimilars.

**90%** Chronic diseases and mental health conditions cost the health care system 90% of the total \$4.5 trillion spent annually<sup>55</sup> and impact patients' quality of life and productivity.

**2x** The number of individuals with three or more chronic conditions is projected to nearly double by 2030, greatly increasing the economic burden imposed by these illnesses.<sup>56</sup>

**\$1T** Direct health care costs for Alzheimer's disease are estimated to reach \$1 trillion annually by 2050, not accounting for lost wages, stress and lost productivity.<sup>57</sup>

**\$2T** Heart disease and stroke are expected to cost the United States roughly \$2 trillion by 2050<sup>58</sup> unless we find new ways to intervene and reduce the burden of disease.

**\$966B** Just 400 rare diseases impacting 15.5 million Americans cost the United States \$966 billion annually.<sup>59</sup>

## American biopharmaceutical preeminence is not guaranteed.

Who leads in this next era of medicine will depend on the policies that the United States adopts, as countries, such as China, seek to become world leaders in this important sector. American biopharmaceutical leadership is at risk if intellectual property isn't protected, a strong regulatory system isn't maintained and the United States government fails to implement the appropriate incentives amid increasing global competition for talent and investment.

**+** Global R&D investment in 2021 was \$276 billion across 4,000+ companies.<sup>60</sup> U.S.-headquartered companies contribute to 55% of total R&D investment, Europe contributes 29% and Asia/Pacific contributes 15%.<sup>61</sup>

**+** U.S. scientific and engineering investment has dropped relative to the rest of the world, while spending has risen rapidly in a number of Asian countries.<sup>62</sup> China alone accounts for nearly a third of the increase in global R&D growth since 2000.<sup>63</sup>

# Medicines are an important tool in fighting disease.



## ■ WE ARE TAKING ON THE BIGGEST CHALLENGES FOR PATIENTS AND OUR HEALTH SYSTEM.

As an industry, we are laser-focused on developing innovative medicines that transform lives and create a healthier America.

**We have done it before, and we will do it again.** We will build on the learnings of the last quarter century as we develop new medicines that prevent and treat disease and fight for solutions to help patients access and afford the treatments they need.

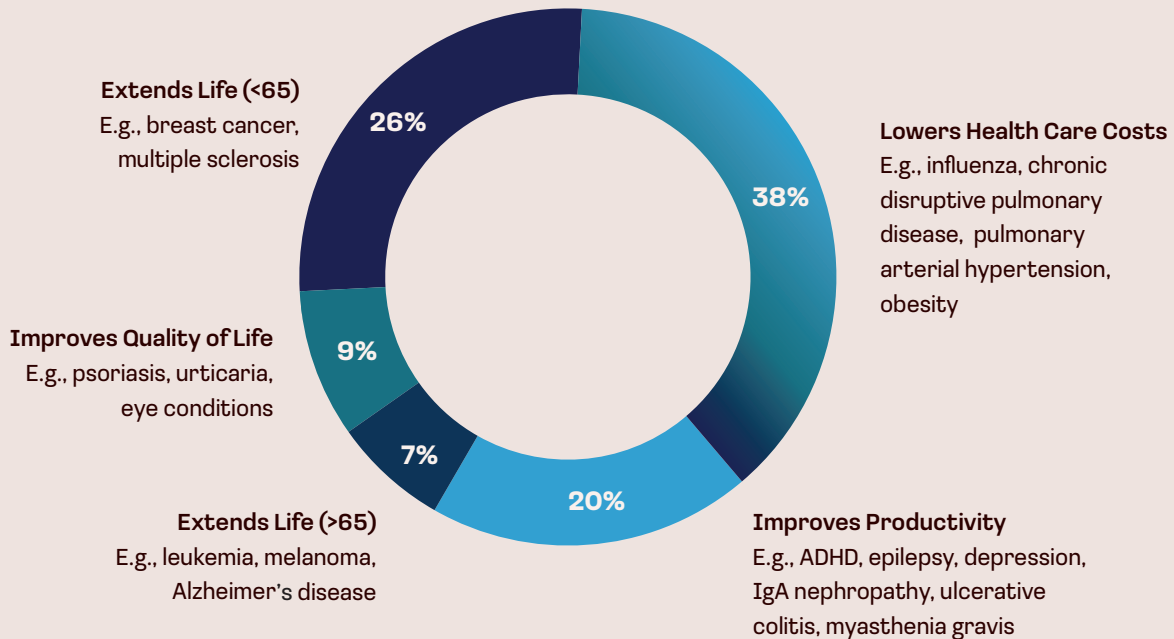
As we enter the second quarter of the 21st century, we are making four commitments to the American public:

## 01. We will change the trajectory of the most serious diseases, with an increased focus on prevention, to help protect patients and support our health care system.

Taking steps to prevent disease and intervene earlier in disease progression are the best investment we, as an industry and society, can make. We've already seen remarkable progress to prevent and change the course of disease.

Medical science has never been more promising. The next 25 years promise to be even more revolutionary in our ability to change what's possible, including more personalized medicines tailored to the individual, new cell and gene therapies that provide hope to patients with diseases that previously had no treatment options, breakthroughs in treatments for neurological diseases that delay progression, regenerative medicines that don't just treat but repair damage caused by disease and so much more. Each discovery will mean healthier bodies, more lives saved and fewer families impacted.

**PROPORTION OF THE TOP 100 MEDICINES  
PROJECTED TO LAUNCH IN THE UNITED STATES  
BETWEEN 2024 AND 2028<sup>64</sup>**



## New medicines are part of the solution.

Too often, people see a new medicine as simply a new expense for the health care system. But changing the trajectory of disease, through a greater focus on prevention and by halting disease progression, is key to making our health system sustainable.

- + If we delay the onset of Alzheimer's disease by 5 years, then the number of Americans with Alzheimer's disease will decrease by approximately 42% by 2050, reducing total costs to all payers by more than \$350 billion in a single year.<sup>65</sup>
- + If we reduce the cost of obesity by 10%, then medical cost savings could reach \$26 billion annually, and related comorbidities could also be reduced.<sup>66</sup>
- + If we reduce the health and economic impact of heart disease, stroke and other cardiovascular conditions by 10%, then we could save roughly 1.5 million years of life while improving quality of life<sup>67</sup> and yielding about \$42 billion in total savings (~\$25 billion in direct savings and ~\$17 billion in indirect savings).<sup>68</sup>

■ CASE STUDY

## Gene therapy is transforming the lives and outcomes of patients with rare diseases.

Gene therapies are treatments that aim to fix or replace faulty genes to prevent or treat diseases like genetic disorders, certain cancers and rare diseases. Due to their long-term and potentially curative effects, gene therapies allow patients to live longer, healthier lives and can help to avoid the significant burden and costs associated with existing standards of care.

With additional research and investment, these therapies will continue to advance, offering treatment for a growing number of genetic diseases. More than 2,000 gene therapies are in the pipeline with the potential to provide greater treatment options for the ~6,500 rare diseases that currently lack an FDA-approved treatment.<sup>69</sup>

“One of the areas that I’m particularly excited about is the significant amount of growth across the ecosystem on cell and gene therapies. We are on the very front end of a revolution of new medicines that can intercept disease in very young children.”

**Thomas Miller**

Vice President and Global Head of the Acute, Chronic and Pediatric Disease Nucleus, Bayer



## 02. We will use the latest technology to improve our efficiency because patients need new treatments as soon as possible.

Time is of the essence because disease doesn't wait. It takes a long time and a large investment to develop new medicines, not just in the laboratory, but through years of testing in progressively larger clinical trials to ensure we understand fully how a medicine will work in patients and the potential risks. New technology can help us develop better hypotheses and test them more quickly.

- **WE'RE USING NEW TECHNOLOGIES LIKE ARTIFICIAL INTELLIGENCE (AI) TO IDENTIFY PROMISING IDEAS, IMPROVE RESEARCH AND DEVELOPMENT AND CLINICAL TRIALS AND SAFELY DELIVER MEDICINES FASTER.**

Artificial intelligence holds great promise to improve the efficiency of new medicine development and help fight disease sooner, allowing researchers to streamline the identification and optimization of drug candidates, identify patient populations most likely to respond to treatment and accelerate clinical trials through optimized design and automated analysis and reporting. **AI has the potential to reduce molecule discovery time and improve efficiency.**<sup>70</sup>



## 03. We will continue to do our part to support America in leading the world in biopharmaceutical innovation so that Americans can realize the benefits of that leadership.

American leadership in medicine has driven economic growth, creating jobs, boosting productivity and improving quality of life through faster access to innovative treatments.

A leading biopharmaceutical industry supports millions of high-tech, high-skill American jobs for the 21st century, provides hundreds of billions of dollars in sustained economic impact in the United States, provides global influence and diplomacy and means patients are more likely to have rapid access to the latest medicines.<sup>71</sup>



■ **U.S. BIOPHARMACEUTICAL LEADERSHIP ISN'T A GUARANTEE. OUR POLICY CHOICES MATTER.**

While countries around the world seek to overtake U.S. dominance in the research and development of new medicines, the robust policy environment that has supported this leadership is eroding. For example, the drug price-setting policies in the Inflation Reduction Act discourage companies from pursuing risky, innovative research.

To support U.S. leadership in biopharmaceutical innovation and the treatments it brings to patients, it is critical to protect the American intellectual property system and support a robust, predictable regulatory system. Our country delivers more treatment options than anywhere else because our system encourages risk-taking and collaboration that turn ideas into medicines.

Our industry is committed to doing our part. We will advance solutions that protect and promote both innovation and patient access, but American leadership requires a strong ecosystem and supportive and predictable policy environment.



**Policy Agenda:** A roadmap of the policies that will spur innovation, protect our global leadership and ensure patient access.

**04.** We will work to improve the health care system so that it works better for patients.

Too many Americans are paying more than they should or being denied access to medicines their doctor prescribes. That's because insurance companies and pharmacy benefit managers (PBMs) decide what medicines patients can get and the price they pay at the pharmacy, and they are using this power to enrich themselves. Half of every dollar spent on brand medicines goes to someone who didn't make it.<sup>72</sup> Rebates, discounts, hidden markups and fees and other payments mean people are paying too much for their medicines.



We believe that all Americans deserve reliable, affordable access to medicines, and we will continue to advocate to improve the system to increase transparency and ensure patients are put first.

We are advocating for policies that would improve the market and lower out-of-pocket costs for patients by ensuring savings go to patients, not middlemen, and reforming 340B to improve medicine access for low-income patients.

**We're committed** to making real progress and sharing regular updates and reports on our advancements, including the number of new therapies in development and disease areas being targeted, the type of patient impacts these new therapies will have and potential cost savings to the health system.



“There’s nothing worse than the feeling of not being able to breathe. Sometimes I need more than one inhaler for a month, sometimes I need two, and my insurance will only cover one inhaler for a month. I don’t want to worry that my inhaler won’t be covered by insurance.”

**Renne**  
Patient

# A Vision for the Future

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We've made real progress in the first quarter of the 21st century. Americans are better off today when faced with serious and chronic illness than they were at any other point in human history.

We have new and innovative ways to prevent and slow hundreds of diseases that affect people across their lifespans – from genetic diseases afflicting children to diseases that occur with age.

We owe this success to a policy environment that supports innovation and progress. If we promote smart policies that support intellectual property, a predictable regulatory environment and patient access to cutting-edge medicines, we can make unprecedented progress. We can achieve great things.

The next quarter century could be the most remarkable period of biopharmaceutical innovation we have ever witnessed. We are on the cusp of revolutions in medicine that build on the progress we've already made with the potential for more preventive and transformational treatments than ever before. These breakthroughs could fundamentally reduce the burden of disease for millions of people and for health systems around the world. We will do everything we can to reach that potential and create a healthier America for us all.

# Sources

1. CA. Cancer statistics, 2025. January 2025.
2. Oncology. Changes in mortality associated with cancer drug approvals in the United States from 2000 to 2016. November 2020
3. American Heart Journal Plus. Sudden cardiac death prevention in the era of novel heart failure medications. March 2023.
4. Novo Nordisk. FDA approves Ozempic® (semaglutide) as the only GLP-1 RA to reduce the risk of worsening kidney disease and cardiovascular death in adults with type 2 diabetes and chronic kidney disease. January 2025.
5. JAMA Intern Med. GLP-1 Receptor Agonists and Risk for Cirrhosis and Related Complications in Patients With Metabolic Dysfunction-Associated Steatotic Liver Disease. November 2024.
6. U.S. Food and Drug Administration. FDA Approves First Medication for Obstructive Sleep Apnea. December 2024.
7. JAMA Network Open. Glucagon-Like Peptide 1 Receptor Agonists and 13 Obesity-Associated Cancers in Patients With Type 2 Diabetes. July 2024.
8. Mayo Clinic. Obesity. July 2023.
9. World Obesity Federation. World Obesity Atlas 2023. 2023.
10. PhRMA. 40 years of cancer immunotherapy research: Paving the way for breakthrough treatments. November 2022.
11. Cancer Research Institute. Regulatory Approval Timeline of Active Immunotherapies.
12. ClinicalTrials.gov.  
<https://clinicaltrials.gov/search?cond=cancer&intr=Immunotherapy>.
13. Citeline. Pharma R&D Annual Review 2024. 2024.
14. Nature. Long-term outcomes following CAR T cell therapy: what we know so far. April 2023.
15. McKinsey analysis of [Health Affairs 2020 analysis](#) of life expectancy.
16. Altarum. Projections of the Non-Retail Prescription Drug Share of National Health Expenditures. July 2022.
17. Annals of Gastroenterology. Interferon-based combination treatment for chronic hepatitis C in the era of direct acting antivirals. Jan-Mar 2015.
18. Hepatology. Side effects of alpha interferon in chronic hepatitis C. September 1997.
19. The New England Journal of Medicine. Sofosbuvir for Previously Untreated Chronic Hepatitis C Infection. May 2013.
20. The New England Journal of Medicine. Glecaprevir–Pibrentasvir for 8 or 12 Weeks in HCV Genotype 1 or 3 Infection. January 2018.
21. American Journal of Managed Care. Impact of Direct-Acting Antiviral Use for Chronic Hepatitis C on Health Care Costs in Medicaid: Economic Model Update. December 2022.
22. American Psychological Association. AIDS in the 90s: Did You Know?. 2017.
23. IJID Regions. Exploring the impact of therapeutic advances in HIV-related mortality in the United States. March 2024.
24. The Lancet HIV. Life expectancy after 2015 of adults with HIV on long-term antiretroviral therapy in Europe and North America: a collaborative analysis of cohort studies. May 2023.
25. Expert Opin Drug Deliv. Long-acting approaches for delivery of antiretroviral drugs for prevention and treatment of HIV: a review of recent research. July 2020.
26. Centers for Disease Control and Prevention. Health and Economic Benefits of Routine Childhood Immunizations in the Era of the Vaccines for Children Program – United States, 1994 – 2023. August 2024.
27. American Journal of Managed Care. Assessing the Cost of Vaccine-Preventable Diseases. December 2019.
28. Citeline. Pharma R&D Annual Review 2024. 2024.
29. PhRMA. Global Access to New Medicines Report. April 2023.
30. Center for Drug Evaluation and Research. Advancing Health Through Innovation: New Drug Therapy Approvals 2024. January 2025.
31. PhRMA. Global Access to New Medicines Report. April 2023.
32. StatPearls. Orphan Drug Approval Laws. June 2023.
33. Nature. Analysis of US Food and Drug administration new drug and biologic approvals, regulatory pathways and review times, 1980–2022. February 2024.
34. National Institute of Health. The Promise of Precision Medicine: Rare Diseases. November 2023.
35. Orphanet Journal of Rare Diseases. A comprehensive study of the rare diseases and conditions targeted by orphan drug designations and approvals over the forty years of the Orphan Drug Act. June 2023.
36. MedPAC. July 2024 Data Book. Health Care Spending and the Medicare Program. July 2024.
37. McKinsey analysis of Evaluate Pharma data, October 2024.
38. McKinsey analysis of Evaluate Pharma data, October 2024.
39. PhRMA Analysis of SSR Health Data.
40. PhRMA Analysis of SSR Health Data.
41. PhRMA Analysis of SSR Health Data.
42. Centers for Medicare & Medicaid Services. NHE Fact Sheet. December 2024.
43. U.S. Food and Drug Administration. Generic Drugs. April 2024.
44. Association for Accessible Medicines. 2023 U.S. Generic and Biosimilar Medicines Savings Report. September 2023.
45. Association for Accessible Medicines. 2024 U.S. Generic & Biosimilar Medicines Savings Report. September 2024.
46. Vasc Health Risk Manag. Benefits of statin therapy and compliance in high risk cardiovascular patients. October 2010.
47. McKinsey analysis of data from [American Heart Association](#), [IQVIA](#), [Journal of Clinical Lipidology](#), [British Journal of Clinical Pharmacology](#).
48. Buttorff C, Ruder T, Bauman M. Multiple Chronic Conditions in the United States. Rand Corporation, 2017.
49. Alzheimer's Association. 2024 Alzheimer's disease facts and figures. April 2024.
50. National Cancer Institute. Cancer Statistics. May 2024.
51. NORC at the University of Chicago. Obesity Prevalence & Comorbidity Map.
52. The Lancet. Excess mortality associates with elevated body weight in the US and demographic subgroup: A modelling study. April 2022.
53. Centers for Disease Control and Prevention. Unfit to Serve. February 2024.
54. National Institute of Health. The Promise of Precision Medicine: Rare Diseases. November 2023.
55. Centers for Disease Control and Prevention. Fast Facts: Health and Economic Costs of Chronic Conditions. July 2024.
56. Partnership to Fight Chronic Disease. What is the Impact of Chronic Disease on America?
57. American Journal of Managed Care. The economic and societal burden of Alzheimer disease: managed care considerations. September 2022.
58. Centers for Disease Control and Prevention. Fast Facts: Health and Economic Costs of Chronic Conditions. July 2024.
59. The Everylife Foundation. The National Economic Burden of Rare Disease Study. February 2021.
60. Nature. Comprehensive measurement of biopharmaceutical R&D investment. August 2024.
61. Nature. Comprehensive measurement of biopharmaceutical R&D investment. August 2024.
62. U.S. National Science Foundation. The State of U.S. Science and Engineering 2020. January 2020.
63. National Institute of Health. China overtaking US as global research leader. February 2020.
64. McKinsey analysis of Evaluate Pharma data, October 2024.
65. Alzheimer's Association. Changing the Trajectory of Alzheimer's Disease: How a Treatment by 2025 Saves Lives and Dollars. 2015.
66. J Manag Care Spec Pharm. Direct medical costs of obesity in the United States and the most populous states. March 2021.
67. JAMA Cardiology. Burden of Cardiovascular Diseases Among US States, 1990–2016. May 2018.
68. Circulation. 2024 Heart Disease and Stroke Statistics: A Report of US and Global Data From the American Heart Association. February 2024.
69. Orphanet Journal of Rare Diseases. A comprehensive study of the rare diseases and conditions targeted by orphan drug designations and approvals over the forty years of the Orphan Drug Act. June 2023.
70. McKinsey analysis of an anonymized example of real impact embedding AI into molecule discovery process.
71. PhRMA. The Economic Impact of the U.S. Biopharmaceutical Industry: 2022 National and State Estimates. May 2024.
72. Berkeley Research Group. The Pharmaceutical Supply Chain, 2013–2023. January 2025.



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