FUTURE OF MEDICINE:

Cell and Gene Therapies

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American Leadership in Cell and Gene Therapies

Letter from PhRMA President and CEO Stephen J. Ubl

For decades, America has been the global leader in medical innovation, guided by a commitment to improving health by unlocking the ability to confront devastating diseases and transform patients' lives.

Cell and gene therapies address the root causes of disease, offering the potential to change or halt its course rather than simply managing symptoms. For patients, that means something once unimaginable: the chance for durable, even curative, outcomes with a single administration.

These breakthroughs, along with hundreds of promising treatments in development, signal a new frontier for treating cancers, rare genetic conditions, neurological disorders and other debilitating diseases.

This progress rests on America's world-leading innovation ecosystem. Scientific leaders who are committed to developing transformative medicines, coupled with strong intellectual property (IP) protections, market-based competition and an advanced regulatory system, make America the global leader in life sciences and biotechnology.

But America's leadership isn't guaranteed. Other countries are expanding their capabilities and have a robust pipeline. China is on pace to catch up with our research and development in innovative cell and gene therapies.

Continuing to foster U.S. innovation of cell and gene therapies is critical to ensuring American patients have access to these life-changing medicines. **We need to make sure that our health care system, and its coverage and payment structures, evolve to ensure that all patients can access these life-changing treatments.**

Cell and gene therapies have already transformed lives — and there is so much more to discover. Driven by our mission to build a healthier future, we're committed to unlocking the full potential of these therapies to keep American patients healthy.

Stephen J. Ubl

Stephen J. Ubl
President and CEO, PhRMA

From Breakthrough to Reality

Cell and gene therapies are transforming the way we treat and prevent disease by targeting disease at its source — at the cellular and genetic levels. As a result, these transformational therapies often produce long-term or even curative benefits with a single administration.

Leveraging the body's own processes to fight disease

Cell and gene therapies can address the underlying causes of disease, but they work in different ways.

CELL THERAPY:
Cells are modified outside the body and returned to the patient to treat conditions where their own cells are damaged or diseased.

GENE THERAPY:
A patient's disease-causing genes are modified, replaced or deleted to treat, cure or prevent disease.

GENE-MODIFIED CELL THERAPY (E.G., CAR T-CELL):
Cells are genetically modified outside the body and then reintroduced into the patient to treat the disease.

Treatment options for American patients have expanded greatly since the first gene therapy was approved by the Food and Drug Administration (FDA) in 2017.

As of January 2026, there are[1]:

25 approved cell and gene therapies

7 of which are approved CAR T-cell therapies

9 of which are approved cancer therapies

CASE STUDY

Engineering patients' own immune cells to track down cancer

In 2017, the first CAR T-cell therapy in the United States was approved to treat childhood acute lymphoblastic leukemia, a rare blood cancer. Since then, **CAR T-cell therapy has become a cornerstone of cancer care** for children and adults, with long-term studies showing its effectiveness in treating, and in some cases even appearing to cure, advanced cancers.[2]

"When my daughter was diagnosed with cancer, I went from planning for her future to fighting for it. Years of research have given her treatment options that did not exist a generation ago, and now cell and gene therapies offer even greater possibilities. Innovation does not just create new medicines — it gives cancer patients a second chance at life."

— Nicki H.

CASE STUDY

Repairing genes in children with spinal muscular atrophy

A gene therapy approved for children under two years old with spinal muscular atrophy (SMA), a debilitating and rare genetic disorder that was once considered one of the leading genetic causes of infant mortality, has **transformed the outlook for SMA patients**. Infants who were once not expected to live past their first year are now not only surviving but also reaching key development milestones — sitting up independently, standing and, in some cases, even walking.[3]

"With SMA, muscles and balance weaken over time, vision and vocals are impacted, and every day brings unplanned challenges. It's a disease that causes you to lose abilities that most people never think twice about. New treatment insights for this rare condition give patients like me hope for forward progress."

— Sue M.

Next-Generation Treatments on the Horizon

438
cell and gene therapies in development in the United States

70%
increase since 2018

These therapies target a range of diseases across therapeutic areas, including hard-to-treat cancers, rare blood disorders, neurologic disorders and autoimmune diseases, among many others.[4]

Pipeline provides life-changing promise for patients

A gene therapy is being developed for adult late-stage **Pompe disease**, a rare genetic disorder that leads to progressive muscle weakness. The innovative therapy delivers a gene to the muscle tissues of adults with Pompe disease, improving symptoms, including muscle weakness, fatigue and difficulty breathing and swallowing.

A gene therapy is in development for children with **nephropathic cystinosis**, a rare genetic disorder that causes kidney damage in infancy. This new therapy would help treat the condition and its debilitating symptoms, such as growth delays or soft-bowed bones.

A cell therapy is in development for **Parkinson's disease**, a progressive, neurodegenerative condition. This therapy has the potential to restore motor and non-motor functioning in patients who can currently only manage symptoms by replacing the neurons that produce dopamine.

> "We've pioneered cell therapy for 20 years, and now we're using that expertise to tackle autoimmune diseases. By using CAR T technology to reset the immune system, we are opening the door to treatment-free remission once considered out of reach. This is a pivotal moment, and I am so excited BMS is helping write the story."
>
> **— Lynelle Hoch, President, Cell Therapy Organization Bristol Myers Squibb**

Competitive pressure threatens U.S. biopharma leadership

Despite the United States' edge in medicines in the pipeline, other countries, like China, are catching up — accelerating clinical trial activity and increasing investment in advanced therapies.[5]

GLOBAL PERCENTAGE OF CLINICAL TRIAL SITES FOR CELL AND GENE THERAPIES IN 2024: UNITED STATES VS. CHINA

19%

39%

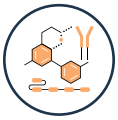
China's global share of clinical trials for cell and gene therapies has increased by 15% since 2019, surpassing the United States in the global race.[6]

Delivering Long-Term Value and Savings

While cell and gene therapies may have higher upfront costs, their **long-term and potentially curative benefits** significantly reduce overall health care spending.

By replacing years of ongoing treatment, hospitalizations and disease complications, these therapies lower lifetime costs, ease the burden of illnesses and **deliver value long after they are initially paid for**.

CELL AND GENE THERAPIES



Replace costly, lifelong care via one-time or short-duration treatments

Often target small patient populations, minimizing large-scale burden to the health system

Avoid downstream system and patient costs

Reduce significant financial and emotional burden and indirect costs

CASE STUDY

Reducing health care costs and treatment burden for patients with Hemophilia B

A gene therapy approved by the FDA in 2022 offers a potential cure for severe Hemophilia B. Patients can avoid a lifetime of burdensome and extremely costly blood factor replacement therapy, which is usually administered two to three times a week to prevent life-threatening bleeding events.

This gene therapy enables patients to discontinue these expensive, frequent treatments, **potentially leading to as much as $600,000 in savings** in the year following a single administration alone.[7]

"My son has hemophilia, and his childhood has revolved around aggressively avoiding injuries and managing bleeds. New cell and gene medicines could mean we can spend less time planning for emergencies and more time letting him be a kid."

— Stephanie H.

CASE STUDY

Building a better future for patients with sickle cell disease

Sickle cell disease (SCD) is a blood cell disorder that can lead to debilitating pain and fatigue, impacting patients' quality of life and ability to work. In 2023, the FDA approved two gene therapies to treat SCD — a major breakthrough given the limited treatment options previously available to patients.

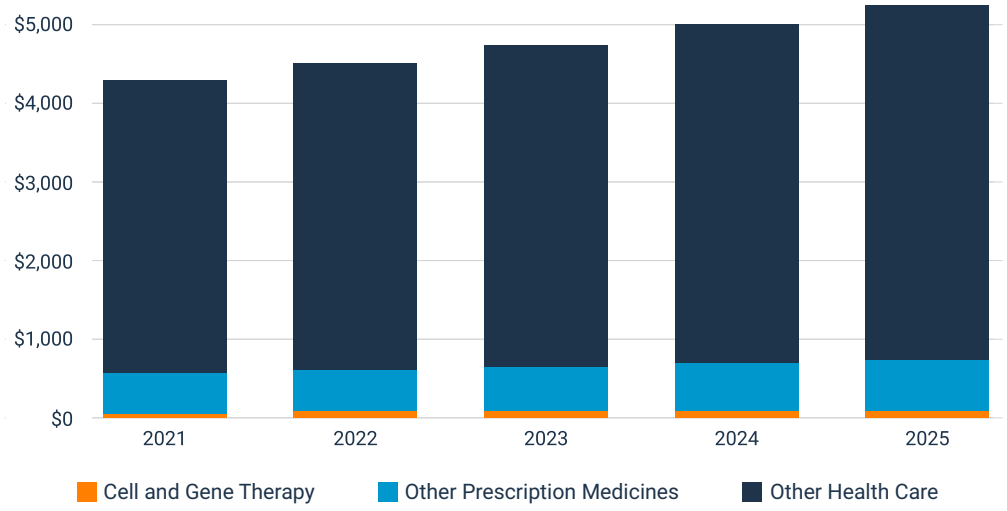
By reducing painful complications from SCD, gene therapy can **help patients earn $21,000 more each year** in average income.[7]

Bridging the Gap Between Innovation and Patient Access

Even as the pipeline continues to grow, spending on cell and gene therapies is expected to remain small and steady in the years ahead, estimated to reach **just 0.1% of health care spending and 1.3% of drug spending by 2030**.[8,9,10]

PROJECTED SPENDING ON PRESCRIPTION MEDICINES, TOTAL HEALTH CARE AND NEWLY LAUNCHED CELL AND GENE THERAPIES ($B), 2021–2025

Even when accounting for future spending on cell and gene therapies, prescription medicines will remain steady at about **14%** of total U.S. health care spending.



While these costs are manageable to the health care system, the upfront costs of single-administration therapies can pose unique challenges to payers.

Innovative payment models such as value-based contracts and alternative financing arrangements have the potential to lower costs for payers, reduce patient cost-sharing and reinforce the evidence of the value these therapies provide over time, especially for small plans and Medicaid programs. Additional reforms could help address barriers caused by outdated regulations and the uncertainty associated with innovative contracts.

Medicare's payment systems also present challenges in accessing cell and gene therapies. Ensuring appropriate reimbursement in both the inpatient and outpatient settings in Medicare can help address these challenges.

Eligible patients are losing their chance at CAR T-cell therapy

Despite the promise of CAR T-cell therapies, patients in the United States face immense challenges accessing these treatments. **Just one in 10 eligible Medicare fee-for-service patients is being treated with CAR T-cell therapy**.[11] Low CAR T-cell therapy uptake is primarily due to several systemic barriers that limit access to treatment, including:

Limited treatment centers

Inadequate Medicare reimbursement

Payer coverage restrictions

Incentivizing American Discovery and Investment

We must ensure that the United States does not cede further leadership to China in the development of cell and gene therapies.

Supporting investment in R&D, protecting IP and strengthening the gold-standard, predictable regulatory framework will encourage discovery at home and help ensure American patients have first access to these therapies.

The Trump Administration has committed to reducing barriers to developing cell and gene therapies and ensuring patient access to these life-changing treatments.

To realize the full potential and protect American leadership, we must work together to:

- Ensure a strong, appropriately staffed and science-based U.S. regulatory review system, resourced through a combination of congressionally appropriated funds and user fees from the regulated industry.
- Support pro-innovation policies that advance and optimize development, manufacturing and supply.
- Protect our world-leading innovation and IP ecosystem.
- Address outdated regulations that can limit the adoption of innovative contracting and payment approaches.
- Modernize Medicare reimbursement to ensure access to CAR T-cell therapies are not impeded.

Powering the future of cell and gene therapies in America

The individualized nature of cell and gene therapies presents **unique challenges for manufacturing and delivery to patients**. These therapies may be produced in small, personalized batches with very short shelf lives. These features bring unique constraints to quality testing and supply chain management. A nimble and risk-based framework that appropriately recognizes the potential benefits of these therapies could do much to bring cell and gene therapies to patients.

> Biopharmaceutical companies are investing to bring the development of these next-generation treatments to the United States.

CASE STUDY

AstraZeneca's state-of-the-art cell therapy manufacturing facility in Maryland will support clinical trials for next-generation T-cell therapies aimed at treating some of the most challenging cancers and diseases.

CASE STUDY

Astellas' gene therapy manufacturing facility in North Carolina is accelerating the development and delivery of life-changing treatments for patients with serious, rare genetic conditions.

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