

Medicines are Transforming the Trajectory of Many Diseases

Today, new medicines are targeting the underlying causes of disease in ways that just a few years ago may have been regarded as science fiction. Thanks to biopharmaceutical science, diseases previously thought of as deadly are now manageable and in some cases curable. In this new era of medicine, scientific and technological advancements are leading to more targeted treatments transforming the way we treat patients with a broad range of chronic and rare conditions.

Over the past few years, America's biopharmaceutical companies have applied their expertise to a once-in-a-generation challenge by delivering safe and effective COVID-19 vaccines and treatments to patients. This experience has underscored the vital role biopharmaceutical innovation plays in meeting the critical needs of patients and addressing our most pressing global health challenges.

I Progress Against Disease

Medicines are transforming the trajectory of many debilitating diseases, resulting in decreased death rates, improved health outcomes and better quality of life for patients:

- **Cancer:** Game-changing new treatment approaches such as immunotherapies and targeted therapies are transforming the cancer treatment paradigm and driving dramatic reductions in mortality and increasing survival. Since peaking in the early 1990s, cancer death rates in the United States have declined 32%.¹ Experts agree and a growing body of evidence underscores the role of new medicines in recent accelerated declines in mortality.^{2,3} Between 2000 and 2016 alone, new cancer drug approvals were associated with 1.3 million prevented cancer deaths.⁴ Many recent treatment advances against cancer are due to ongoing and complex research and development (R&D) conducted after initial FDA approval. Post-approval R&D is critical to progress in fighting cancer. In fact, nearly 60% of cancer medicines approved a decade ago received additional approvals in later years, often changing patients' lives.⁵
- **COVID-19:** In the United States, numerous vaccines and treatments have either been approved or received emergency use authorization to protect against the virus, mitigate its impact and speed recovery should infection occur.⁶ In fact, since the introduction of novel COVID-19 vaccines, the U.S. vaccination program is estimated to have saved 2 million lives and averted up to 17 million hospitalizations.⁷
- **HIV/AIDS:** Once considered acutely fatal, HIV is now a chronic and manageable disease. This dramatic change followed the introduction of highly effective antiretroviral therapy in the mid-1990s, which transformed treatment and led to a 91% decline in death rates in the United States.⁸ Between 2010 and 2017 alone, the death rate from HIV has declined by nearly half. This decline is attributed to improvements in early diagnosis and helping people get on and stay on lifesaving treatment.⁹
- **Cardiovascular disease:** Due in large part to advances in treatment, tremendous strides have been made against cardiovascular disease over the past 40 years. For example, between 1980 and 2000, approximately two-thirds of the decline in coronary heart disease mortality, the most common type of heart disease, is attributable to medical therapies.¹⁰ Since 2000 alone, the death rate from heart disease has declined by nearly 40%.¹¹
- **Hepatitis C (HCV):** Historically, HCV was the leading cause of liver cancer and the most common reason for a liver transplant. Just over 10 years ago, the only available treatment option cured just half of patients and caused debilitating side effects. But today, direct-acting antiviral regimens have been developed to offer cure rates closely approaching 100%. In fact, greater than 95% of HCV infected persons can be cured of infection, including those with challenging comorbidities and treatment resistant forms of the virus.^{12,13} These treatments have dramatically reduced health care costs previously associated with treating HCV, producing a total of \$12 billion in savings net of treatment costs in Medicaid by 2022 alone.¹⁴

I Recent Medicine Approvals

Today, scientists continue to explore new frontiers in biopharmaceutical research. In 2021, the U.S. Food and Drug Administration (FDA) approved 60 new medicines, 50 of which were approved by the FDA Center for Drug Evaluation (CDER). Among CDER's approvals, 54% were first-in-class medicines, representing entirely new ways of treating disease.^{15,16} The FDA's human drug review program is the global gold standard for regulatory review and approval. In large part because of the Prescription Drug User Fee Act, the United States now leads the world in the introduction of new medicines with 38 of the newly approved medicines receiving approval in the U.S. before any other country.¹⁷

Examples of novel therapies that became available to U.S. patients in 2021 include:^{18,19}

- A treatment for the most common type of lupus as well as another medicine for a serious complication of lupus affecting the kidneys: lupus nephritis.
- A medicine to treat a deadly virus in post-transplant patients.
- Medicines to reduce the risk of cardiovascular death and heart failure hospitalization.
- The first complete HIV treatment that can be administered once every 1-2 months, rather than daily.
- 5 vaccines, including the first FDA-approved vaccine for COVID-19
- 2 CAR-T cell therapies for certain types of multiple myeloma and lymphoma. CAR-T cell therapies are a game-changing approach which involve permanently altering a patient's T-Cells to recognize, target, and kill cancer cells.
- 26 medicines to treat rare diseases, such as a medicine to treat patients 1 year and older with late-onset Pompe disease, which causes muscle weakness and premature death from respiratory or heart failure; and a medicine to reduce risk of death due to molybdenum cofactor deficiency type A, a disease presenting in the first few days of life, causing seizures, brain injury and death.

I The Promise of the Pipeline

Researchers continue to pursue cutting-edge research and novel scientific strategies and harness new technologies to continue to drive therapeutic advances for patients. There are currently more than 8,000 medicines in development globally with the potential to impact U.S. patients.²⁰ And across the medicines in the pipeline, nearly 70% have the potential to be first-in-class treatments.²¹

Medicines in development include:

- **Rare Diseases:** Rare diseases individually affect fewer than 200,000 Americans, but collectively they impact 30 million Americans, with the vast majority impacting children. While fewer than 10% of rare diseases have an approved treatment option, there is hope on the horizon with nearly 800 medicines currently in development for patients with rare diseases.²²
- **Neurodegenerative Diseases:** These diseases occur when neurons in the brain, spinal cord and/or peripheral nervous system begin to deteriorate or become functionally impaired. The most common are Alzheimer's disease, multiple sclerosis, and Parkinson's. There are currently more than 260 medicines in development for 29 devastating neurodegenerative diseases.²³
- **Health Equity:** Many conditions disproportionately affect racial and ethnic communities—often due to genetic or environmental reasons, or due to inequities in social and economic conditions. Unfortunately, many of these diseases can also place the same communities at higher risk for severe illness or death from COVID-19. Today there are 829 medicines in development that aim to address these illnesses and help reduce the health disparities that exist among these groups.²⁴

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