

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. Diseases previously regarded as deadly are now manageable and even curable. In this new era of medicine, scientific and technological breakthroughs are leading to more targeted treatments that transform the way we treat patients with a broad range of chronic and rare conditions.

Right now, in the midst of a global health emergency, researchers are working around the clock and leveraging these critical advances and decades of experience cultivated in fighting other infectious diseases to overcome the pandemic and its consequences. In times like these, the role that biopharmaceutical innovation plays in addressing global health challenges and meeting critical unmet needs – both now and in the future – could not be more apparent.

PROGRESS AGAINST DISEASE

Medicines play a central role in transforming the trajectory of many debilitating diseases, resulting in decreased death rates, improved health outcomes and better quality of life for patients.



Cardiovascular disease: Tremendous strides have been made against cardiovascular disease over the past 40 years due in large part to advances in treatment. Since 1980 alone, the death rate from heart disease has declined by more than 50%.¹ And between 1980 and 2000, approximately two-thirds of the decline in coronary heart disease mortality, the most common type of heart disease, was attributable to medical therapies.²



HIV/AIDS: Once considered acutely fatal, HIV/AIDS is now a chronic and manageable disease. This dramatic change followed the introduction of highly active antiretroviral therapy (HAART) in the mid-1990s, which transformed treatment and led to a 90% decline in death rates in the United States.³



Hepatitis C: Just nine years ago, the only available treatment for hepatitis C cured just half of patients and caused debilitating side effects. Today, a broad range of treatments with minimal side effects and cure rates approaching 100% are available for patients with all forms of the disease.^{4,5} The introduction of curative medicines also reduces health care costs previously associated with treating hepatitis C. In Medicaid, these medicines are estimated to produce a total of \$12 billion in savings net of treatment costs by 2022.⁶



Cancer: New medicines also drive gains in the life expectancy of cancer patients. Since peaking in the early 1990s, the United States has witnessed a 29% decline in cancer death rates. Last year alone saw a 2.2% decline, the single largest drop ever recorded.⁷ Researchers attribute 73% of these gains to new treatments, including new medicines.⁸ For many patients, emerging immunotherapies and targeted therapies are transforming the treatment paradigm for many forms of cancer, often reducing the use of chemotherapy, surgery and radiation.⁹

RECENT APPROVALS

Today, scientists continue to explore new frontiers in biopharmaceutical research. In 2019, the U.S. Food and Drug Administration (FDA) approved more than 55 new medicines, including 48 new medicines approved by the FDA Center for Drug Evaluation (CDER). Among CDER's approvals, 42% were first-in-class medicines, representing entirely new ways of treating disease.^{10,11}

Examples of novel therapies that became available to patients in 2019 include:



First Ebola vaccine: The first vaccine to protect against the Ebola virus disease was approved in the U.S. in 2019.¹² A second vaccine is also under review by the European Medicines Agency (EMA), with a decision expected this year. Both vaccines were advanced in response to the West African Ebola crisis, and both were successfully deployed to help contain the current outbreak in the Democratic Republic of the Congo (DRC). Ebola is a lethal and highly contagious hemorrhagic fever with mortality rate ranging from 40-90%.¹³

 **Two new drugs to treat sickle cell disease (SCD):** SCD is a debilitating disorder disproportionately impacting African Americans in which red blood cells are abnormally shaped restricting flow in blood vessels and limiting oxygen delivery to the body's tissues. The condition is also characterized by chronic inflammation that results in vaso-occlusive crisis, a frequent cause of ER visits and hospitalizations, where SCD patients experience episodes of severe pain and organ damage. The first medicine is an inhibitor of selectin, a substance that contributes to these crises.¹⁴ The second approval is an inhibitor of deoxygenated sickle hemoglobin polymerization, a central abnormality in SCD.¹⁵

 **The first gene therapy for spinal muscular atrophy (SMA):** SMA is a rare disease caused by a mutation in the SMN1 gene which is involved in the maintenance and function of motor neurons, leading to progressive muscle weakness and wasting and in severe cases, respiratory failure. The gene therapy for this leading genetic cause of infant mortality was approved to treat children less than 2 years of age with the most common and severe form of SMA.¹⁶

THE FUTURE HAS NEVER BEEN BRIGHTER

Researchers are pursuing cutting-edge research and novel scientific strategies, and they're harnessing 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.¹⁷ And across the medicines in the pipeline, 74% have the potential to be first-in-class treatments.¹⁸

Medicines in development include:¹⁹

 **Cell and Gene Therapy:** A new wave of medicines are changing the way many diseases are treated, offering the potential for one-time administration with long-term effects. There are nearly 400 novel cell and gene therapies in development for a variety of diseases and conditions from cancer to genetic disorders to neurologic conditions and many rare diseases.²⁰

 **Mental Illness:** Nearly 1 in 5 adults, or 46.6 million Americans are living with mental illness. There are nearly 140 medicines in development to treat a broad range of mental illnesses—including against depression, schizophrenia, substance use disorder, anxiety disorders, attention-deficit/hyperactivity disorders and bipolar disorders.

 **Diseases Affecting Children:** New treatment options for infants, children and adolescents can be complex and often require different clinical approaches than adult treatment pathways. America's biopharmaceutical researchers are committed to studying, developing and testing medicines to meet the needs of pediatric patients with more than 2,100 industry-sponsored clinical trials underway, testing 58 investigational medicines and involving more than 1.2 million pediatric patients across a variety of therapeutic areas where there is significant unmet need.

 **Vaccines:** There are currently 258 vaccines in development, including for the prevention of infectious disease such as COVID-19 and HIV, as well as therapeutic vaccines for the treatment of cancer and Alzheimer's Disease.

The tremendous promise that is evident in today's biopharmaceutical pipeline represents a new frontier of research with the potential to transform the lives of patients. In this new era of medicine, science that was once considered unimaginable is now on the verge of producing a complete paradigm shift in the treatment of the most complex and challenging diseases of our time.

1 CDC, Age-adjusted death rates for 72 selected causes by race and sex using year 2000 standard population: United States, 1979-98; Xu JQ, Murphy SL, Kochanek KD, et al.; CDC, National Center for Health Statistics, Deaths: final data for 2017. Natl Vital Stat Rep. 2018;68(9):1-77.

2 H Guo, D Cutler, T Graziano, M Weinstein, A Pandya. Why have we Been Dying Less from Coronary Heart Disease in the United States? Proceedings of the 22nd Annual International Meeting International Society of Pharmacoeconomics and Outcomes Research; May 2017; Boston, MA. Abstract.

3 CDC, National Center for Health Statistics. Health, United States, 2016: with chartbook on long-term trends in health. <https://www.cdc.gov/nchs/data/abus/abus16.pdf>. Published 2017; Xu JQ, Murphy SL, Kochanek KD, et al.; CDC, National Center for Health Statistics, National Vital Statistics System. Deaths: final data for 2017. Natl Vital Stat Rep. 2018;68(9):1-77

4 PhRMA. A Decade Of Innovation In Chronic Diseases: 2006-2016 February 2016.

5 US FDA. Drugs@FDA: FDA approved drug products, labels for approved DAA products.

6 Roebuck MC, Liberman JN. Burden of illness of chronic hepatitis c and impact of direct-acting antiviral use on healthcare costs in Medicaid. Am J Manag Care. June 2019.

7 Siegel RL, Miller KD, Jemal A. Cancer statistics, 2020. CA Cancer J Clin.2020;70:7-30.

8 S Seabury, "Quantifying Gains in the War on Cancer Due to Improved Treatment and Earlier Detection," Forum for Health Economics and Policy 2016; 19(1): 141-156.

9 Personalized Medicine Coalition. The Personalized Medicine Report: 2017 Opportunity, Challenges and the Future. November 2017.

10 FDA. CDER, 2019 Novel Drugs Summary.

11 FDA. CBER, 2019 Biological License Approvals

12 FDA News Release. "First FDA-approved vaccine for the prevention of Ebola virus disease, marking a critical milestone in public health preparedness and response" December 19, 2019.

13 Mao H.H., Chao S. (2019) Advances in Vaccines. Current Applications of Pharmaceutical Biotechnology. Advances in Biochemical Engineering/Biotechnology, vol 171. Springer, Cham. link.springer.com/chapter/10.1007%2F978-94-007-2101-0

14 FDA News Release. "FDA approves first targeted therapy to treat patients with painful complication of sickle cell disease" November 12, 2019.

15 FDA News Release. "FDA approves novel treatment to target abnormality in sickle cell disease," November 25, 2019.

16 FDA News Release. "FDA approves innovative gene therapy to treat pediatric patients with spinal muscular atrophy, a rare disease and leading genetic cause of infant mortality" May 24, 2019.

17 Adis R&D Insight Database. Accessed June 2019.

18 Analysis Group, "The Biopharmaceutical Pipeline: Innovative Therapies in Clinical Development," July 2017.

19 Analysis Group, "The Biopharmaceutical Pipeline: Innovative Therapies in Clinical Development," July 2017.

20 PhRMA. Medicines in Development for Cell and Gene Therapy. December 2019.