



MEDICINES ARE TRANSFORMING THE TRAJECTORY OF MANY DISEASES

Today, new medicines target the underlying causes of disease in ways never seen before. And 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. Looking forward, continued advances in biopharmaceutical innovation will be critical in addressing unmet need, improving public health and solving future health care challenges.

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%.ⁱ And 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.ⁱⁱ
- **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 an 88% decline in death rates in the United States.ⁱⁱⁱ
- **Hepatitis C:** Just eight 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.^{iv, v} The introduction of curative medicines also reduces health care costs previously associated with treating Hepatitis C. In Medicaid, beginning in 2017, this is estimated to have produced savings exceeding the cost of curative treatments and yielding a total of \$12 billion in cumulative savings by 2022.^{vi}
- **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 27% decline in cancer death rates.^{vii} Researchers attribute 73% of these gains to new treatments, including new medicines.^{viii} For many patients, emerging cell and gene therapies and immunotherapies transform the treatment paradigm for many forms of cancer and have the potential to reduce the use of traditional forms of cancer treatment—including chemotherapy, surgery and radiation.^{ix}

RECENT APPROVALS

Today, scientists continue to explore new frontiers in biopharmaceutical research. In 2018, the U.S. Food and Drug Administration (FDA) approved 65 new medicines, including 59 new medicines approved by the FDA Center for Drug Evaluation (CDER). Among CDER's approvals, 32% were first-in-class medicines, representing entirely new ways of treating disease.^{x, xi}

Examples of novel therapies that became available to patients in 2018 include:

- **A new drug to treat HIV in patients who failed other therapies:** While most patients living with HIV can be successfully treated with currently available HAART combinations, a small percentage of patients who have taken many HIV medicines in the past may have developed multidrug resistant HIV and are at high risk of developing complications and death. A new medicine from an entirely new class of treatment was approved this year, offering a critical treatment option for these patients.^{xii}
- **First new treatment in 10 years to alleviate pain caused by endometriosis:** Endometriosis occurs when tissue that lines the uterus grows in other parts of the body. It is one of the most common gynecologic disorders in the United States, affecting 1 in 10 women of reproductive age, and it can be associated with debilitating pain symptoms. Very few treatments are specifically indicated for treatment of the condition, and surgical interventions are not always curative for patients. The new treatment provides a critical option for women suffering from endometriosis pain.^{xiii, xiv}
- **A new class of medicines to prevent migraines:** 1 in 6 Americans suffer from migraines. Three new medicines were approved this year from a class of medicines called calcitonin gene-related peptide (CGRP) inhibitors which work by binding to the CGRP receptor to reduce the number of days patients suffer with migraines. The pursuit of medicines targeting CGRP challenged researchers for decades until they discovered the approach of the current class of treatments.^{xv, xvi}

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 clinical development globally with the potential to impact U.S. patients.^{xvii} And across the medicines in the pipeline, 74% have the potential to be first-in-class treatments.^{xviii}

Medicines in development include:^{xix}

- **Cancer:** In addition to cell and gene therapy approaches, which are just beginning to transform the lives of patients, a number of other novel approaches – including antibody-drug conjugates, immune checkpoint modulators, metabolic immunotherapies and vaccines – are showing tremendous promise in the pipeline against a broad range of cancers. Today, there are 1,120 medicines and vaccines currently in development for cancer.^{xx}
- **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 300 novel cell and gene therapies in development for a variety of diseases, including blood disorders, eye disorders, cancer and infectious diseases. For example, Duchenne Muscular Dystrophy (DMD) is a genetic condition where the absence of a protein called dystrophin causes muscle cells to deteriorate. A gene therapy in development works by delivering a shortened version of the protein to help keep the muscle cells intact.^{xxi}
- **Sickle Cell Disease:** This rare inherited blood disorder affects 100,000 Americans and disproportionately impacts African Americans. The painful condition causes irregularly shaped blood cells to get stuck in small blood vessels, resulting in slow or blocked blood flow and oxygen to parts of the body. This often leads to life-threatening complications such as stroke, difficulty breathing, hypertension and organ damage. There are nearly 20 medicines in development to treat this rare disease—including medicines employing cutting edge technologies such as RNA interference, gene-edited stem cell therapy and gene therapy.^{xxii}

ⁱ Centers for Disease Control and Prevention (CDC), National Center for Health Statistics (NCHS), National Vital Statistics System. Age-adjusted death rates for 72 selected causes by race and sex using year 2000 standard population: United States, 1979-98.. April 2016. J Xu, et al; SL Murphy, et al; US Department of Health and Human Services, Centers for Disease Control and Prevention, National Center for Health Statistics, National Vital Statistics System. Deaths: final data for 2016. Natl Vital Statistics Rep. 2018;67(5). July 2018.

ⁱⁱ 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.

ⁱⁱⁱ Centers for Disease Control and Prevention, National Center for Health Statistics. Health, United States, 2016: With Special Feature On Racial and Ethnic Health Disparities. <https://www.cdc.gov/nchs/data/abus/abus16.pdf>. 2017.

^{iv} PhRMA. A Decade Of Innovation In Chronic Diseases: 2006-2016 February 2016.

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

^{vi} 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.

^{vii} Siegel RL, Miller KD, Jemal A. Cancer statistics, 2019. CA Cancer J Clin. 2019;69(1):7-34.

^{viii} 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.

^{ix} Personalized Medicine Coalition. The Personalized Medicine Report: 2017 Opportunity,

Challenges and the Future. November 2017.

^x US FDA. 2018 Novel Drugs Summary. January 2019.

^{xi} US FDA. 2018 Biological License Approvals

^{xii} US FDA. FDA approves new HIV treatment for patients who have limited treatment options. March 2018.

^{xiii} US FDA. 2018 Novel Drugs Summary. January 2019.

^{xiv} AbbVie, Press Release, "AbbVie Receives U.S. FDA Approval of ORILISSA™ (elagolix) for the Management of Moderate to Severe Pain Associated with Endometriosis" July 2018.

^{xv} US FDA. 2018 Novel Drugs Summary. January 2019.

^{xvi} Dolgin, E. Antibody drugs set to revive flagging migraine target. Nature Reviews Drug Discovery volume 12, pages 249-250 (2013)

^{xvii} Adis R&D Insight Database. Accessed June 2019.

^{xviii} Analysis Group, "The Biopharmaceutical Pipeline: Innovative Therapies in Clinical Development," July 2017.

^{xix} Analysis Group, "The Biopharmaceutical Pipeline: Innovative Therapies in Clinical Development," July 2017.

^{xx} PhRMA. Medicines in Development for Cancer. May 2018.

^{xxi} PhRMA. Medicines in Development for Cell and Gene Therapy. December 2018.

^{xxii} PhRMA. Medicines in Development for Sickle Cell. April 2019.