



MEDICINES ARE TRANSFORMING THE TRAJECTORY OF DISEASE

Medicines yield important progress against some of the most challenging diseases of our time. 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, breakthrough science and personalized therapies are transforming 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 nearly 60 percent.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 in the mid-1990s, which transformed treatment and led to an 88 percent decline in death rates in the United States.ⁱⁱⁱ
- **Hepatitis C:** More recently, we've seen a remarkable transformation against another viral disease: hepatitis C. Just seven years ago, the only available treatment 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 percent are available for patients with all forms of the disease.^{iv, v} Looking forward, researchers project that with improved screening and today's cures, hepatitis C will be a rare disease by 2036.^{vi}
- **Cancer:** New medicines are also a driving force behind gains in the life expectancy of cancer patients. Since peaking in the early 1990s, the United States has witnessed a 26 percent decline in cancer death rates.^{vii} Researchers attribute 73 percent of these gains to new treatments, including new medicines.^{viii} For many patients, targeted therapies and emerging immunotherapies are transforming 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, this progress continues as scientists explore new frontiers of biopharmaceutical research. In 2017, the U.S. Food and Drug Administration (FDA) approved 56 new medicines, including 46 new medicines approved by the FDA Center for Drug Evaluation (CDER). Among CDER's approvals, 33 percent were first-in-class medicines, representing entirely new ways of treating disease.^{x, xi}

Examples of novel therapies that became available to patients in 2017 include:

- **First cell and gene therapies:** A new wave of medicines are changing the way many diseases are treated, offering the potential for one-time administration with long-term durability of effect. The first chimeric antigen receptor T-cell (CAR-T) therapies were approved in 2017 for two advanced, rare forms of blood cancer.^{xi, xiii} For these

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therapies, a patient's own cells are extracted, genetically modified and reintroduced to the patient's body with the ability to recognize and attack specific cancer cells. Additionally, in 2017, the first directly-administered gene therapy was approved for the treatment of a rare form of inherited blindness. The therapy works to restore vision by delivering to the retinal cells a normal copy of the gene that is mutated in patients with this disorder.^{xiv}

- **First medicine for primary progressive multiple sclerosis (PPMS):** Multiple sclerosis is the most common cause of neurological disability in young adults and involves a disruption in the flow of information between the brain and the body, generally leading to disability. The vast majority of patients experience a form of the disease characterized by relapses and remissions. These patients have a range of treatments available to modify the course of their disease. About 15 percent of patients have a form of the disease without distinct relapses or remissions, but rather a steady progression to disability. Ocrelizumab was approved in 2017 to treat not only relapsing forms of MS but PPMS, providing a first-time treatment for these patients with this devastating disorder.^{xv}
- **First treatment for sickle cell disease in 20 years:** A medicine was approved to treat sickle cell disease, an inherited blood disorder that restricts blood vessels, limits the delivery of oxygen to the body's tissues and causes severe and debilitating pain and organ damage. Clinical trials demonstrated those treated with the medicine experienced fewer hospitalizations. The medicine was approved to reduce severe complications associated with the disorder.^{xvi}

THE FUTURE HAS NEVER BEEN BRIGHTER

Researchers are pursuing cutting-edge research and novel scientific strategies to continue to drive therapeutic advances for patients. There are currently about 7,000 medicines in clinical development globally with the potential to impact U.S. patients.^{vii} And across the medicines in the pipeline, 74 percent have the potential to be first-in-class treatments.^{viii} Medicines in development include:^{xix}

- **Cancer:** In addition to the adoptive cell therapy 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}
- **Heart disease and stroke:** Cardiovascular disease is the leading cause of death in the United States, affecting 92.1 million American adults. There are currently 200 medicines in development for heart disease and stroke. One promising

investigational medicine is a non-viral gene therapy that targets a tissue and regeneration pathway which promotes cardiac function, cell survival and the repair of injured heart tissue in patients with ischemic heart failure.^{xxi}

- **Neurological disorders:** These disorders affect a broad range of conditions affecting the brain and nervous system—for example, epilepsy, migraine headaches, multiple sclerosis, Parkinson's disease and Alzheimer's disease. There are more than 500 medicines in development for neurological disorders. One exciting cell therapy approach for amyotrophic lateral sclerosis (ALS) involves extracting stem cells from patient bone marrow and customizing the cells to help support the survival of neurons once the cells are returned to the patient.^{xxii}

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.

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