

# 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 thought of as deadly are now manageable and in some cases even 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. This year, America's biopharmaceutical companies applied this expertise to a once-in-a-generation challenge by delivering safe and effective COVID-19 vaccines and treatments to patients in record time. In the midst of a global pandemic, the role that biopharmaceutical innovation plays in meeting the critical unmet needs of patients and addressing our most pressing global health challenges 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%.<sup>1</sup> 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.<sup>2</sup>
- *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.<sup>3</sup> Between 2010 and 2017 alone, the death rate has declined by nearly half. The Centers for Disease Control and Prevention (CDC) attributes much of this decline to improvements in early diagnosis and helping people get on and stay on lifesaving treatment.<sup>4</sup>
- *Hepatitis C*: More recently, we've seen a remarkable transformation in treatment of another viral disease: hepatitis C. Just ten years ago, the only available treatment cured half of patients and caused debilitating side effects. Today, a broad range of treatments with increasing efficacy and minimal side effects and cure rates approaching nearly 100% are available for patients with all forms of the disease, including many challenging to treat subpopulations.<sup>5,6</sup> The introduction of curative medicines also reduces health care costs previously associated with treated hepatitis C. In Medicaid, these medicines have been estimated to produce a total of \$12 billion in savings net of treatment costs by 2022.<sup>7</sup>
- *Cancer*: New medicines are also driving gains in the life expectancy of cancer patients. Since peaking in the early 1990s, cancer death rates in the United States have declined 31%.<sup>8</sup> Researchers attribute 73% of these gains to new treatments, including new medicines.<sup>9</sup> Targeted therapies and emerging immunotherapies are transforming the treatment paradigm for patients with many forms of cancer and have the potential to reduce the use of traditional forms of cancer treatment—including chemotherapy, surgery and radiation.<sup>10</sup> As a result of remarkable advances, between 2000 and 2016 alone, new cancer drug approvals have been associated with 1.3 million avoided cancer deaths across 15 of the most common tumor types.<sup>11</sup>

## Recent Approvals

Today, scientists continue to explore new frontiers in biopharmaceutical research, despite unprecedented challenges with the COVID-19 pandemic. In 2020, the U.S. Food and Drug Administration (FDA) approved more than 58 new medicines, including 53 new medicines approved by the FDA Center for Drug Evaluation (CDER). Among CDER's approvals, 39% were first-in-class medicines, representing entirely new ways of treating disease.<sup>12,13</sup> Not included among these figures are the critical COVID-19 vaccines and treatments that have been authorized for emergency use by the FDA.

Examples of novel therapies that became available to patients in 2020 include:

- **Rare Diseases:** Among last year's approvals were 31 new therapies for rare diseases. These new medicines include treatments for devastating neurodegenerative diseases such as spinal muscular atrophy and Duchenne muscular dystrophy, dangerous inflammatory conditions such as hereditary angioedema and neuromyelitis optica spectrum disorder which leads to blindness and paralysis, and several rare and deadly forms of cancers, including several for pediatric patients. Many of these provide first-time treatments for patients who previously had few or no medicines available to treat their conditions. For example, last year the first therapy was approved to treat children under the age of 2 with Chagas disease, a parasitic illness that can cause congestive heart failure.
- **Infectious Diseases:** Among last year's approvals was the first antiviral treatment for COVID-19 patients. 2020 also saw continued progress against HIV with a first-in-class therapy for patients who cannot manage the disease with available antiretroviral medications due to resistance, intolerance or safety considerations. Two treatments for Ebola infection and severe malaria which previously lacked treatment options also became available to patients in 2020. In addition to vaccines authorized by the FDA for COVID-19, two vaccines were also approved to protect against the flu and certain types of Meningococcal meningitis in patients 2 years and older.

## The Future of Medical Innovation has Never Been More Promising

Researchers are pursuing cutting-edge research and novel scientific strategies and 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.<sup>14</sup> And across the medicines in the pipeline, 74% have the potential to be first-in-class treatments.<sup>15</sup> Furthermore, in large part because of Prescription Drug User Fee Act, the United States now leads the world in the introduction of new medicines, and the FDA's human drug review program is the global gold standard for regulatory review and approval. Medicines in development include:

- **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 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.<sup>16</sup>
- **Cancer:** In addition to the cell and gene therapy approaches that are just beginning to transform the lives of patients, several novel approaches – including, antibody-drug conjugates, immune checkpoint modulators, personalized medicines, RNA therapeutics, metabolic immunotherapies and vaccines – are showing tremendous promise across the pipeline against a broad range of cancers. Today, there are more than 1,300 medicines and vaccines currently in development for cancer.<sup>17</sup>
- **Diseases Affecting Children:** New treatment options for infants, children and adolescents can be complex and often require different clinical approaches than adult treatment pathways. There are currently nearly 600 pediatric medicines currently in development to meet the unique needs of children.<sup>18</sup>
- **COVID-19:** America's biopharmaceutical companies remain committed to ending the pandemic, building a more resilient health care system and further reducing hospitalizations and mortality associated with COVID-19. As of July 7, 2021, there are 1,833 clinical trials testing COVID-19 treatments and vaccines.<sup>19</sup>

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12 FDA. CDER, **2020 Novel Drug Summary**.

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14 Adis R&D Insight Database. Accessed June 2021.

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17 PhRMA. **Medicines in Development for Cancer**. 2020.

18 PhRMA, **Medicines in Development for Children**. February 2020.

19 Analysis of publicly available databases such as [clinicaltrials.gov](http://clinicaltrials.gov), [AdisInsights](http://AdisInsights.com), and the World Health Organization's International Clinical Trials Registry Platform (WHO ICTRP) as of July 7, 2021