4 Things To Know About Biopharmaceutical Efforts To Fight Cancer

A recent study published in *Nature* is a profound reminder of how far we've come in the fight against cancer.[i] Authors of the study, which examined the long-term effects of CAR-T cell therapy, characterized one of the first patients treated as part of clinical trials in 2010 as "cured," a term rarely used in oncology. CAR-T is a form of gene modified cell therapy involving permanently altering a patient's T-cells to recognize, target and kill cancer cells, with the goal of eliciting an enduring immune response over the long-term.

There are now a total of six CAR-T therapies approved by the U.S. Food and Drug Administration (FDA) to treat a wide range of cancers--including pediatric cancer. The first of these did not receive FDA approval until 2017.

CAR-T cell therapy is just one approach within a broader field of groundbreaking immunotherapies and targeted therapies being developed by biopharmaceutical researchers that are pushing the boundaries of science and leading to new strategies for delivering more personalized, precise and more effective treatments in cancer. This wave of new treatments is built on years of research and development and is driving meaningful improvements in quality of life and survival across a range of cancers, including some cancers that previously lacked treatment options.

As we embark on the critical task of accelerating the rate of cancer therapy progress, and with the Biden administration's effort to reignite the Cancer Moonshot, here are four things to know about the biopharmaceutical industry's role in fighting cancer:

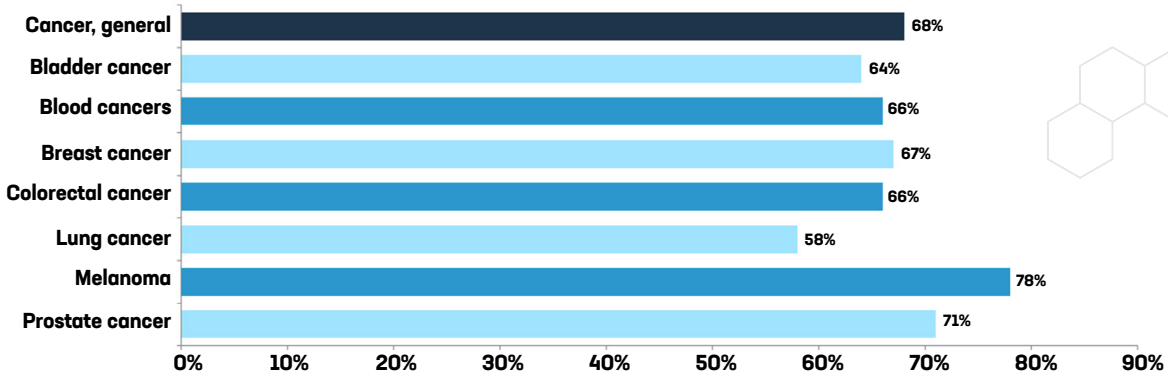
1. **A number of game-changing new approaches to treatment are now available for a wide range of cancers, contributing greatly to significant reductions in mortality and increases in survival.** Biopharmaceutical researchers' understanding of the underlying biological mechanisms and the role of the body's immune system in fighting cancer has led to groundbreaking new medicines transforming the treatment of many cancers. These range from CAR-T cell therapy to immune checkpoint inhibitors, to personalized medicines often targeted to the genetic characteristics of a patient's specific cancer, and many more medicines that have become available to patients in recent years. Due in part to this tremendous progress, the American Cancer Society recently reported cancer death rates have declined by 32% since peaking in 1991, with more accelerated drops witnessed in recent years.[ii]

   Experts agree, and a growing body of evidence underscores, the role new medicines are playing in accelerating recent declines in mortality. In fact, dramatic declines in lung cancer and melanoma deaths seen in recent years are widely attributed to advances in targeted therapies and immunotherapies.[iii, iv] Between 2000 and 2016 alone, new medicines were estimated to be associated with the prevention of 1.3 million cancer deaths–including 130,000 prevented breast cancer deaths, nearly 400,000 prevented lung cancer deaths and nearly 500,000 prevented melanoma deaths.[v]

New Approaches to Treating Cancers Represent the Majority of Medicines in the Oncology Pipeline

Researchers are using novel approaches to attack cancer at the molecular level. An average of 68% of drugs in the oncology pipeline have the potential to be first-in-class medicines.

Percentage of Projects in Development that are Potentially Novel Approaches in Selected Cancer Areas, 2020



Source: Long, G. "The Biopharmaceutical Pipeline: Innovative Therapies in Clinical Development" Analysis Group. 2021.

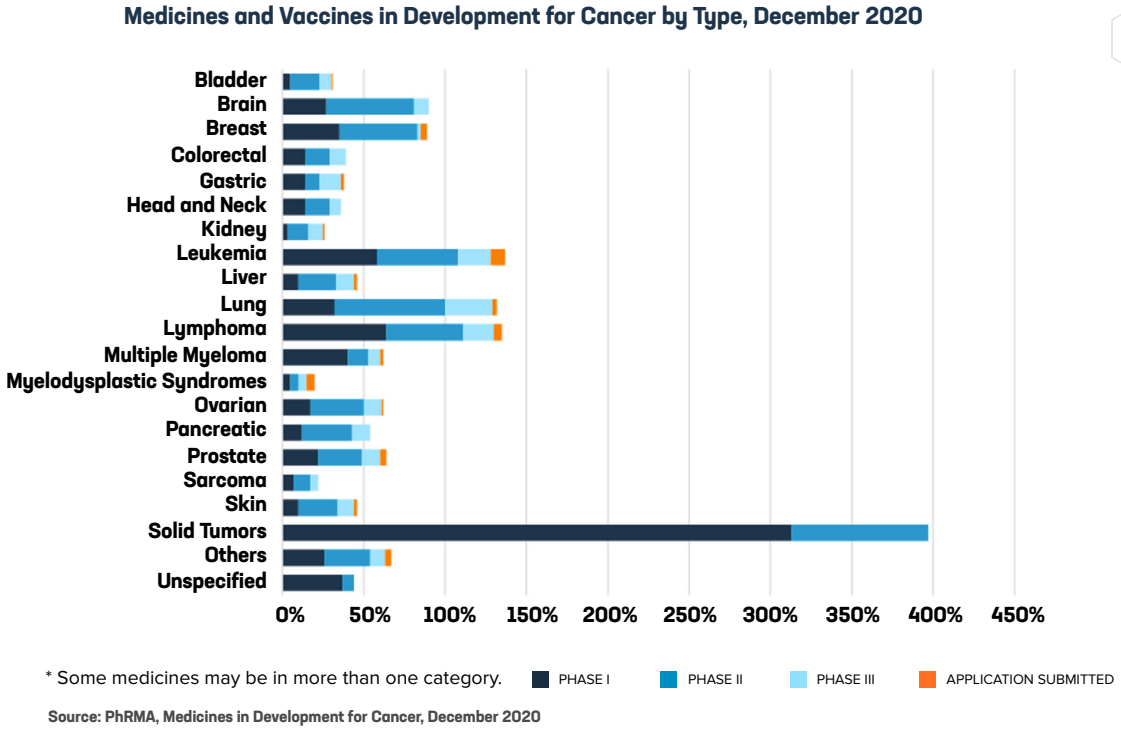
2. Continued research on medicines after FDA approval is key to advancing new cancer treatments. Initial FDA approval is a significant milestone for many patients, but it is often just the beginning as researchers continue to explore the effectiveness of a new treatment in other forms of cancer or in combination with other cancer therapies through continued clinical trials and real-world evidence generation. Research following FDA approval may demonstrate a medicine is effective in different forms of cancer, demonstrate greater efficacy when administered earlier in the progression of disease, is effective in patients with certain genetic characteristics or can be used to treat children, among other possibilities.

For example, pembrolizumab was originally approved to treat melanoma in 2011. Additional research led to the approval for use in a total of 18 different types of cancers, including for use earlier in the treatment line for many cancers and in children.

3. The biopharmaceutical cancer pipeline has never been more promising. After decades of research to advance cancer progress, an average of 68% of medicines in the oncology pipeline today are likely to be first-in-class, meaning they use a new and unique mechanism for treating a disease.[vi] More than 1,300 medicines and vaccines for various cancers are currently in development, either in clinical trials or awaiting review by the FDA. The pipeline is also ripe with innovative therapeutic approaches, like mRNA, with the potential to transform a wide range of cancers–many which have already seen approvals in recent years. For example:

- *Immunotherapies*, which include monoclonal antibodies and CAR-T, is an approach that works by unleashing the immune system to target and kill cancer cells.
- *Gene editing* involves manipulation of DNA at particular locations in order to treat a specific cancer.
- *Oncolytic viral therapies* work by zeroing in on cancer cells, to replicate and cause them to rupture.
- *Antibody Drug Conjugates* target specific cancer cells with cytotoxic agents without harming normal cells.

Promise in the Pipeline: More than 1,300 Medicines in Development for Various Cancers



4. America's health care ecosystem encourages and supports the risk-intensive work needed to transform cancer. The new era of medicine transforming our ability to treat, and in some cases even cure some of the most challenging diseases facing our country today like cancer, is built on the robust U.S. biomedical R&D ecosystem. This ecosystem is sustained by a policy framework designed to support and advance America's leadership in the innovation of new medicines, including strong intellectual property protections, a well-functioning, science-based regulatory system and coverage and payment policies that support and encourage medical innovation. The progress that we have seen in cancer in recent years and our ability to catalyze and accelerate future progress will be impeded without public policies that reward and incentivize innovation.

Despite the tremendous scientific progress that has been made, many forms of cancer are currently lacking in treatment options and tremendous unmet need remains. This year, 1.9 million Americans are expected to receive a cancer diagnosis and more than 600,000 Americans are expected to die.[vii] As the industry looks to the future, we are committed to doing everything we can to fight cancer in all its forms and in bringing new medicines to patients and families facing a cancer diagnosis.

To learn more about cancer, visit PhRMA.org/Cancer.

i https://www.nature.com/articles/s41586-021-04390-6
ii https://acsjournals.onlinelibrary.wiley.com/doi/full/10.3322/caac.21708
iii N Howlader, et al., "The Effect of Advances in Lung-Cancer Treatment on Population Mortality": N Engl J Med 2020; 383:640-649
iv J Berk-Kraus et al., "New Systemic Therapies and Trends in Cutaneous Melanoma Deaths Among US Whites, 1986-2016," American Journal of Public Health, May 2020.
v : JP MacEwan et al, "Changes in mortality associated with cancer drug approvals in the United States from 2000 to 2016, J of Med Econ, Nov 2020
vi Long, G. "The Biopharmaceutical Pipeline: Innovative Therapies in Clinical Development" Analysis Group. June 2017.
vii https://acsjournals.onlinelibrary.wiley.com/doi/full/10.3322/caac.21708