

Cancer Innovation: Why Accelerating Research and Discovery Is Essential

Over the past four decades the return on investment in cancer research has been substantial. Scientific discoveries have netted new and better ways to prevent, detect, diagnose and treat the more than 200 types of cancer. As a result, death rates have dropped by 20 percent and the population of cancer survivors has grown from 3 million in 1971 to almost 14 million today.

Yet continued progress in cancer innovation is not a certainty, especially at a time when government-funded basic research is declining steadily, private sector capital investment in biotechnology is slowing, and drug development is hampered by significant institutional and regulatory obstacles. The following is a snapshot of current areas of medical discovery in cancer, what cancer innovation has made possible, and why accelerating discovery is necessary to save lives, reduce healthcare costs and achieve economic prosperity.

What Innovation Has Achieved

Although cancers collectively remain the second leading cause of death in the U.S., biomedical innovation has transformed a cancer diagnosis from an almost certain death sentence in the 1970s to a time when death rates are declining and survival rates are steadily increasing. These improved outcomes have also resulted in gains to the U.S. economy through reduced spending on hospitalizations and physician care and increased productivity.

According to recently published reports:

- The incidence rates of all new cancers have fallen steadily since 1999.
- Between 1990 and 2007, the cancer death rate fell 22 percent for men and 14 percent for women, which translated to 898,000 fewer deaths from cancers during this period. This includes a steady decline in death rates for the four most common cancers: lung, colorectal, breast and prostate.
- There has also been a significant decline in deaths from chronic myeloid leukemia (8.4 percent), cancers of the stomach (3.1 percent) and colorectum (3.0 percent), and non-Hodgkin lymphoma (3.0 percent) over the past decade (2000-2009).
- At the same time, the National Cancer Institute has charted significant increases in survival as more therapies are developed. From 1975 to 1979, the 5-year survival rate was 48.7 percent. Now it averages 68.5 percent.
- One study found that medicines specifically accounted for 50-60 percent of the increases in cancer survival rates since 1975.
- Looking ahead to 2022, the number of cancer survivors is projected to reach almost 18 million, largely due to the translation of scientific discoveries into new and better cancer medicines.
- New cancer therapies are also associated with 50 million life years saved over the last 15 years, as well as reduced spending on hospital and physician care, amounting to an economic gain of \$1.2 million per person.
- This translates into \$3.2 trillion per year in national wealth added to the economy between 1970 and 2000, a value equal to about half the annual Gross Domestic Product (GDP) over this 30 year period.

The Need to Accelerate Cancer Innovation

Despite these major strides, the fight against cancer is far from over. Cancer still kills 1,600 Americans daily and costs the country over \$200 billion yearly in health care costs and lost productivity. What is more, cancer cases are expected to jump nearly 45 percent by 2030.

The following projections underscore what is at stake:

- An estimated 665,540 new cancer cases are expected to be diagnosed in 2014.
- However, these rates are expected to rise significantly over the next two decades due to the aging of the population. As a consequence, cancer is predicted to soon become the number one disease-related killer of Americans.
- Even now, the rates for some cancers in the U.S. are rising. This includes melanoma, non-Hodgkin lymphoma, leukemia, myeloma, childhood cancers, and cancers of the esophagus, liver, kidney, pancreas and thyroid.
- In addition, mortality rates for certain cancers remain stubbornly high, especially for liver, pancreatic and uterine cancers.
- Moreover, cancer is a global problem. Cancer incidence worldwide is predicted to increase from 12.8 million new cases in 2008 to 22.2 million in 2030. Without the continued development of new treatments, this will mean 13 million more lives lost to cancer in 2030.
- At the same time, cancer already has the greatest economic impact from premature death and disability of all causes of death worldwide. According to a 2010 report from the American Cancer Society and LIVESTRONG, the economic toll from cancer is nearly 20 percent higher than heart disease, the second leading cause of economic loss (\$895 billion and \$753 billion respectively). Without continued innovation, these costs are expected to increase dramatically.
- Demonstrating the potential of medical discovery to reduce the burden of cancer, research from the University of Chicago estimates that reducing cancer death rates by 10 percent would be worth roughly \$4.4 trillion in economic value to current and future generations.

Current Areas of Cancer Discovery

Four decades of research and biomedical discovery have resulted in new cancer medicines that precisely target cancer cells and block their growth or destroy them entirely. Due to their greater precision, these new anticancer drugs are also more effective and less toxic than previous treatments, affording great benefit to patients both in terms of success rate and quality of life. As such, through biomedical innovation, cancer care is moving from a one-size-fits-all approach to the practice of molecularly based medicine, also known as personalized medicine or precision medicine, where the genetic and molecular profile of the patient's cancer determines the best treatment strategy.

Building on these medical advances, almost 1,000 new cancer medicines and vaccines were in development in 2012, the latest year for which statistics are available. According to the National Cancer Institute and the American Association of Cancer Research, the most promising areas of cancer innovation include:

- ***Molecular-targeted drugs that interfere with cell growth signaling*** – this category includes drugs called selective estrogen receptor modulators (SERMs), such as tamoxifen, and the newer aromatase inhibitors (AIs). SERMs and AIs act like estrogen on some tissues but block estrogen's ability to promote the growth of estrogen-positive breast cancers. For this reason,

they are given to patients after primary treatment to increase the likelihood of a cure and to prevent the chance of a breast cancer recurrence.

- **Signal transduction inhibitors** – which block specific enzymes that tell cancer cells to grow. These targeted therapies seek out and destroy only cancer cells while leaving healthy cells unharmed and allow patients to avoid many of the side effects, such as nausea and hair loss, associated with traditional chemotherapy. The best known signal transduction inhibitor is imatinib (Gleevec®), taken in pill form to treat chronic myelogenous leukemia (CML) and gastrointestinal stromal tumors.
- **Angiogenesis inhibitors** – which block the formation of blood vessels like arteries that feed tumors. Angiogenesis inhibitors treat a number of cancers and because they are “smart” drugs that don’t kill healthy cells, they have fewer side effects and less chance of drug resistance.
- **Monoclonal antibodies (mAbs)** – which are substances produced in the lab that locate and bind to cancer cells wherever they are in the body. To date, FDA has approved about a dozen monoclonal antibodies to treat a number of cancers, including leukemia and lymphoma, certain types of head and neck cancer, colorectal cancer, and HER-2 positive breast cancer.
- **Immunotherapy or biological therapies** – which use the body's immune system, either directly or indirectly, to fight cancer or to lessen side effects caused by other cancer treatments.
- **Photodynamic therapy (PDT)** – this is a process of applying a medicine and then shining a special laser light on it. It may be used to treat certain cancers when surgery or radiation can't be used. To date, FDA has approved one of these treatments for use in PDT to treat esophageal and non-small cell lung cancers.
- **Nanotechnology** – this is the science of manipulating materials on a scale so small they can't be seen with a regular microscope. In the case of cancer therapies, researchers use nanoparticles 100–10,000 times smaller than human cells in existing cancer drugs to reduce the side effects and increase their effectiveness. While largely in the development phase, the first wave of nanotechnology-based cancer drugs have passed regulatory scrutiny and are now on the market.

Overcoming the Barriers to Progress

Although the majority of Americans believe that accelerating research and drug discovery is a top priority, the sobering reality is that cancer research is slowing and the rate of private sector capital and drug development is declining. According to recent reports, government funded research declined close to 20 percent since 2010, investment in biotechnology is slowing, and innovation is being hampered by clinical trials deficiencies, from lack of public awareness to no singular repository of clinical trials listings.

Also impeding progress is the time to develop new cancer treatments. Compared to an average time of two years from discovery to approval for HIV drugs, it takes nine or more years for a new cancer therapy. Development is also an uncertain process in which an estimated 19 out of every 20 experimental drugs never make it to market. Not surprisingly then, development costs for cancer

treatments have reached new highs. According to a 2010 Tufts University study, the cost of developing one innovative cancer drug can be upwards of \$1 billion.

Because the obstacles to accelerated cancer innovation affect everyone, the National Patient Advocate Foundation (NPAF) conducted a thorough review of the impediments to cancer innovation and issued a new report, *Securing the Future of Innovation in Cancer Treatment*, which identifies some readily achievable solutions. The result is a blueprint for action that involves three pillars of innovation:

1. Expand the science of innovation by reducing regulatory and logistical obstacles.

This will require moving to a more standardized regulatory-approval process, streamlining the many logistical hurdles to conducting clinical trials, allowing patients expedited access to innovative new therapies before they are approved for general use, and developing a centralized, nationwide hub from which data relating to cancer trials can be accessed and shared.

2. Improve the value of innovation by bolstering funding opportunities.

To accelerate cancer innovation, NPAF encourages a new wave of experimentation in research funding. This could include new models that increase the incentives of research and eliminate uncertainty for innovators and investors, **allowing them to pursue high-risk investments in next-generation cancer therapies.** It is also imperative that all stakeholders align around the need for increased congressional appropriations for government-funded cancer research, especially for basic biomedical research, a key driver of late-stage research.

3. Enhance the delivery of innovation through improved communication and coordination between providers and patients

According to recent estimates, only 2-5 percent of adult patients enroll in cancer clinical trials. This low uptake is the result of impediments, such as lack of awareness, the complexity of consent forms and other patient materials, and the costs for travel and other non-treatment expenses not reimbursed by payers. Addressing these problems will require improved communication and coordination between providers and patients, and regulatory policies that ensure that payers cover clinical trial costs as required by an Executive Order signed by President Clinton in 2000 and a National Coverage Decision (NCD) for Routine Costs in Clinical Trials issued by the Centers for Medicare and Medicaid Services on September 19, 2000 and reaffirmed in 2007.

All Americans –from patients and healthcare providers to research scientists, biomedical innovators, payers, venture capitalists and the business community–have a stake in accelerating the pace of progress against cancer. Thus, advancing realistic and achievable policy solutions is a crucial step towards saving lives, reducing healthcare costs and achieving greater economic prosperity. The time for action is now.

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