PhRMA

RESEARCH • PROGRESS • HOPE

CANCER CHART PACK

CANCER MEDICINES: VALUE IN CONTEXT

SPRING 2014

TABLE OF CONTENTS

INTRODUCTION

Researchers and clinicians are making remarkable progress in the fight against cancer; death rates have declined overall and for many patients, cancer has become a chronic condition to be managed, instead of a death sentence.

However, the many different forms of cancer still cause enormous suffering for patients and their families and a substantial economic burden in the United States. To sustain continued progress in an environment of increasing pressure to contain health care costs, it is important to understand the valuable role new medicines play in improving patient outcomes.

ADVANCES IN CANCER TREATMENT

Cancer Medicines Are Benefiting Patients

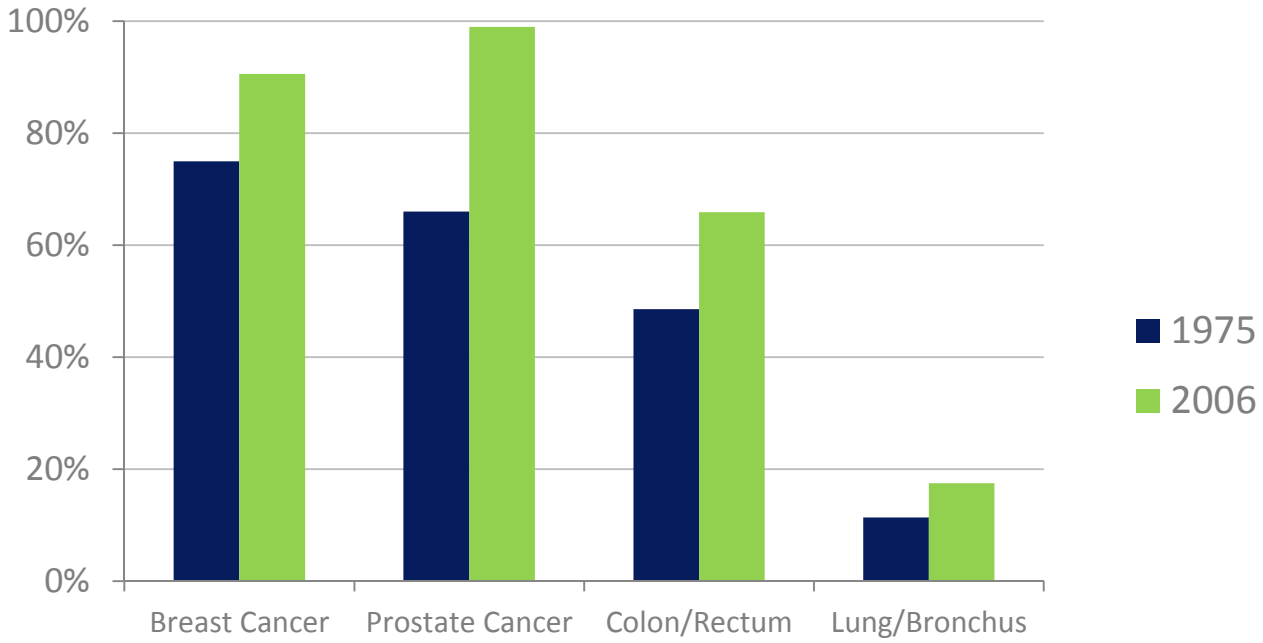
In recent decades we have seen remarkable progress in the fight against cancer. Research has advanced from viewing cancer as a monolithic disease to understanding it better on a molecular and genomic level.

With this greater understanding has come better treatments that have helped to lengthen lives, improve patients' quality of life, and increase productivity. Patients today have better options than ever before.

Five-Year Survival is Increasing for Many Types of Cancer

The chances that a cancer patient will live 5 years or more has increased by 39% across cancers.

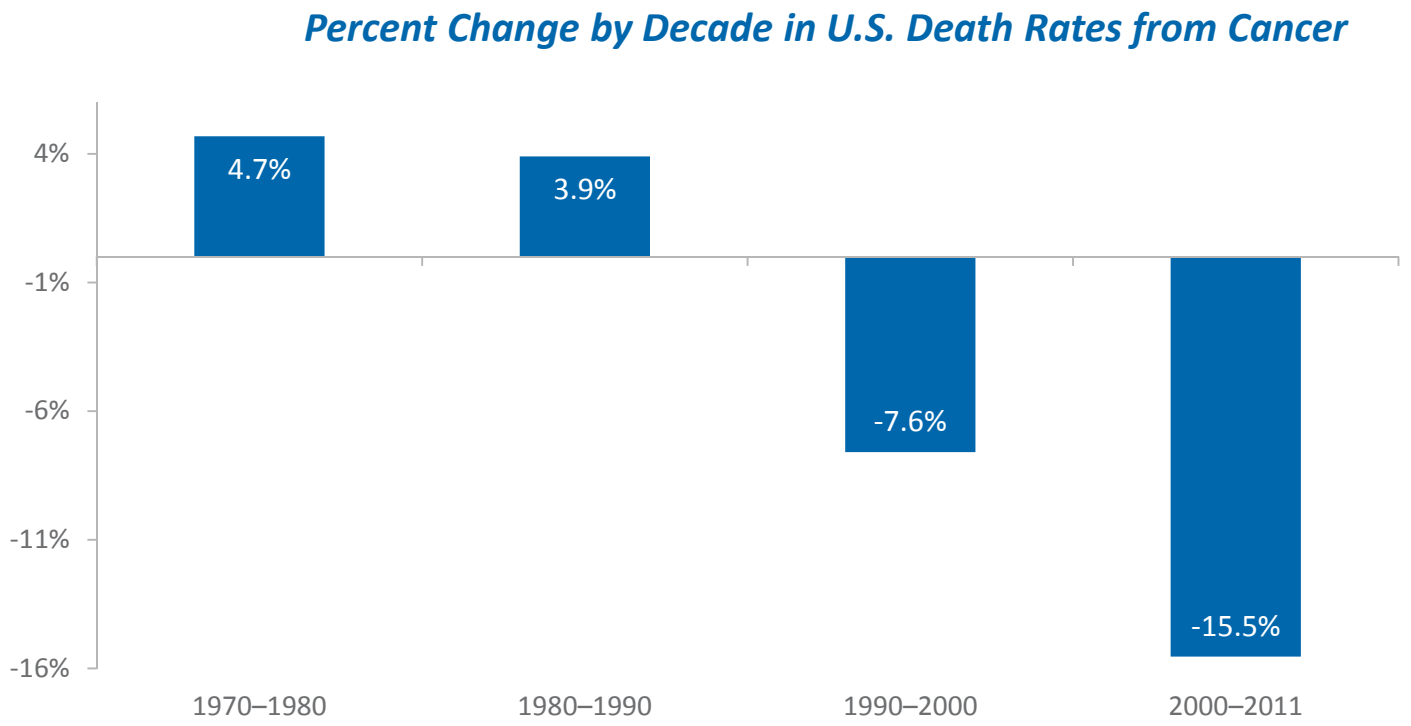
5-Year Survival Rates for Selected Cancers, 1975-2006



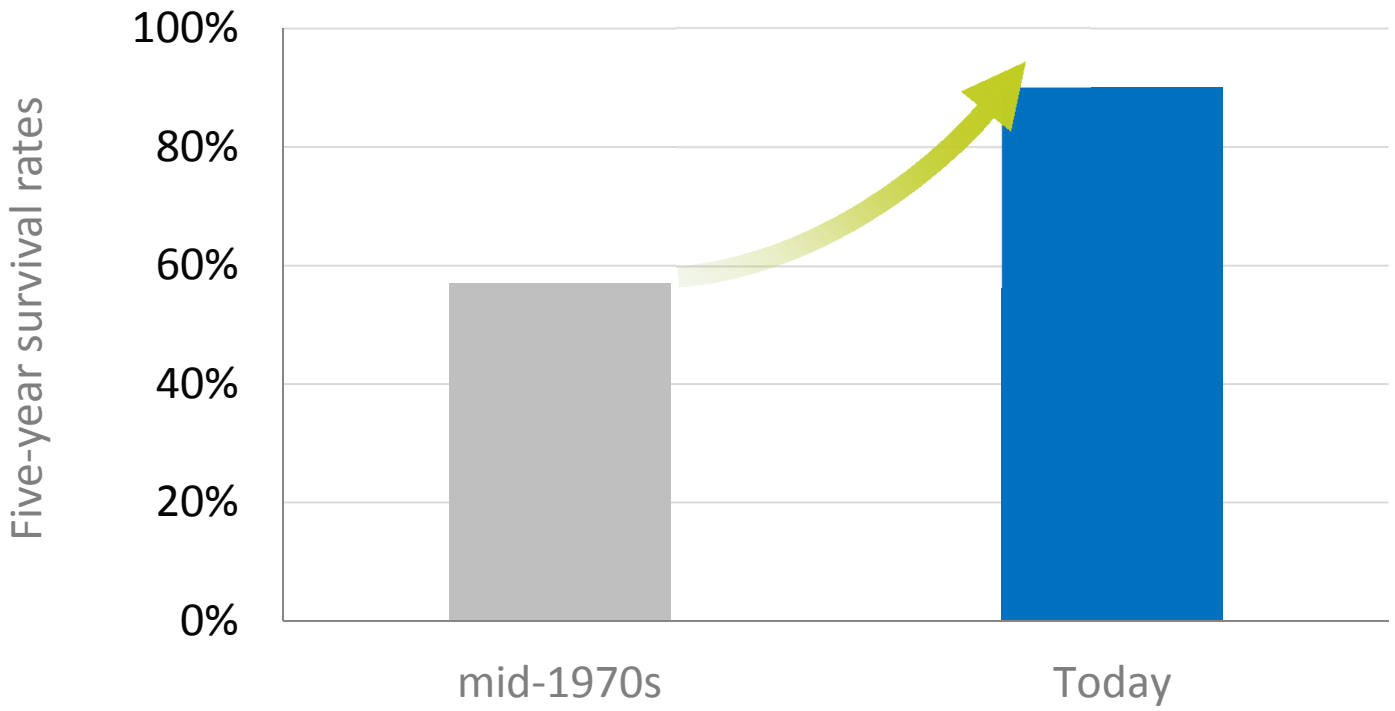
83% *of survival gains in cancer are attributable to new treatments — including medicines.*

Steady Decline in Cancer Death Rates

According to the American Cancer Society, improvements in treatment are contributing to increases in cancer survival.



Survival Rates for Childhood Cancers Have Increased 58 percent over the Last Several Decades



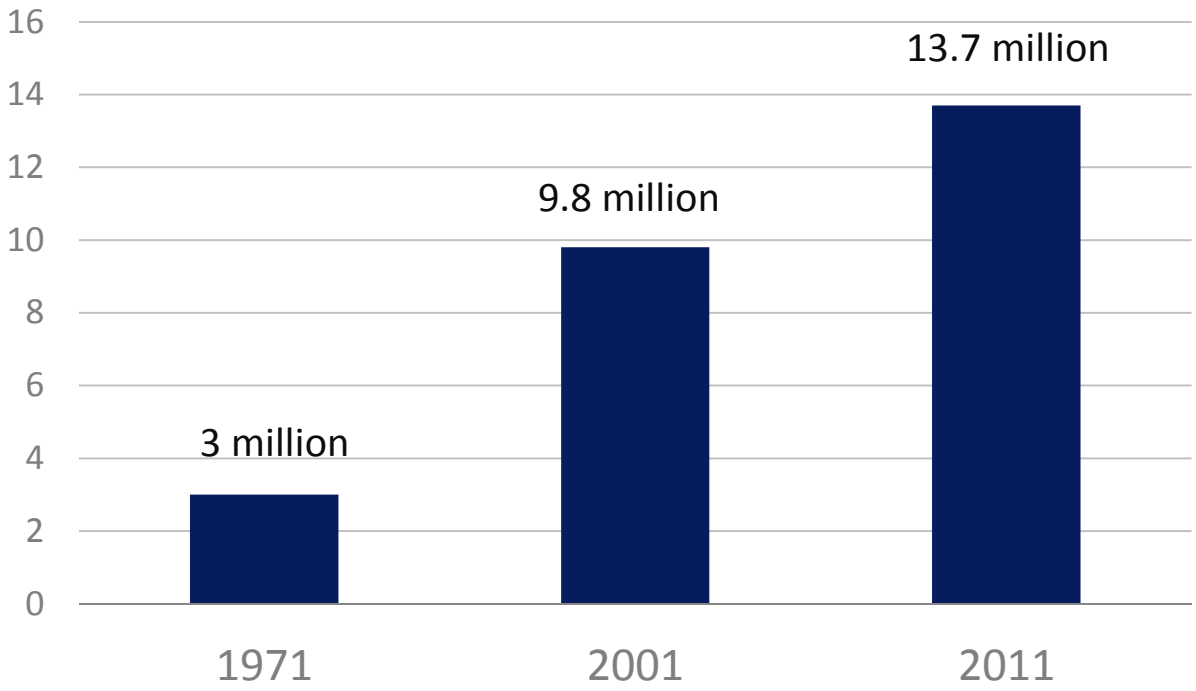
The Number of Cancer Survivors is Steadily Rising

The continued increase in survival rates is in large part attributable to earlier detection and better treatments.

"Remarkable advances are contributing to the rise in the number of people who are surviving longer and living life to the fullest after their cancer diagnosis."

— American Association for Cancer Research

U.S. Cancer Survivors Over Time, Millions



Understanding the Value of Innovation: Evolution over Time

Cancer medicines often go on to deliver more value than initial studies show.

FDA approval and introduction of a new therapy is a significant milestone for patients but it is ***only the beginning***.

Our knowledge of the full benefits of a therapy emerges ***over time***, through continued research and real world clinical practice.

Possible pathways for value to reveal itself over time:

- ✓ Earlier Use
- ✓ Use in combination with other agents
- ✓ Use in specific sub-populations of patients using diagnostics
- ✓ Use in other disease indications

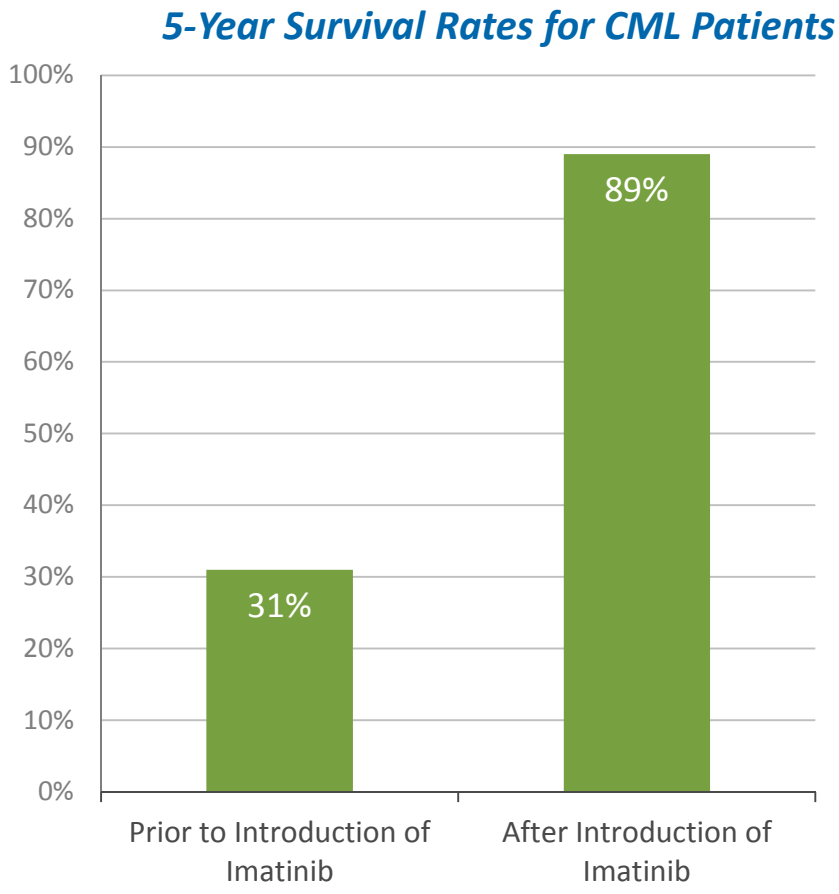
"Incremental advances can add up to transformative changes."

— Siddhartha Mukherjee, MD, author of *The Emperor of All Maladies*

Chronic Leukemias: Increased Survival Rates

When imatinib was first approved in 2001 to treat chronic myeloid leukemia (CML), the full value of the medicine had not been completely realized.

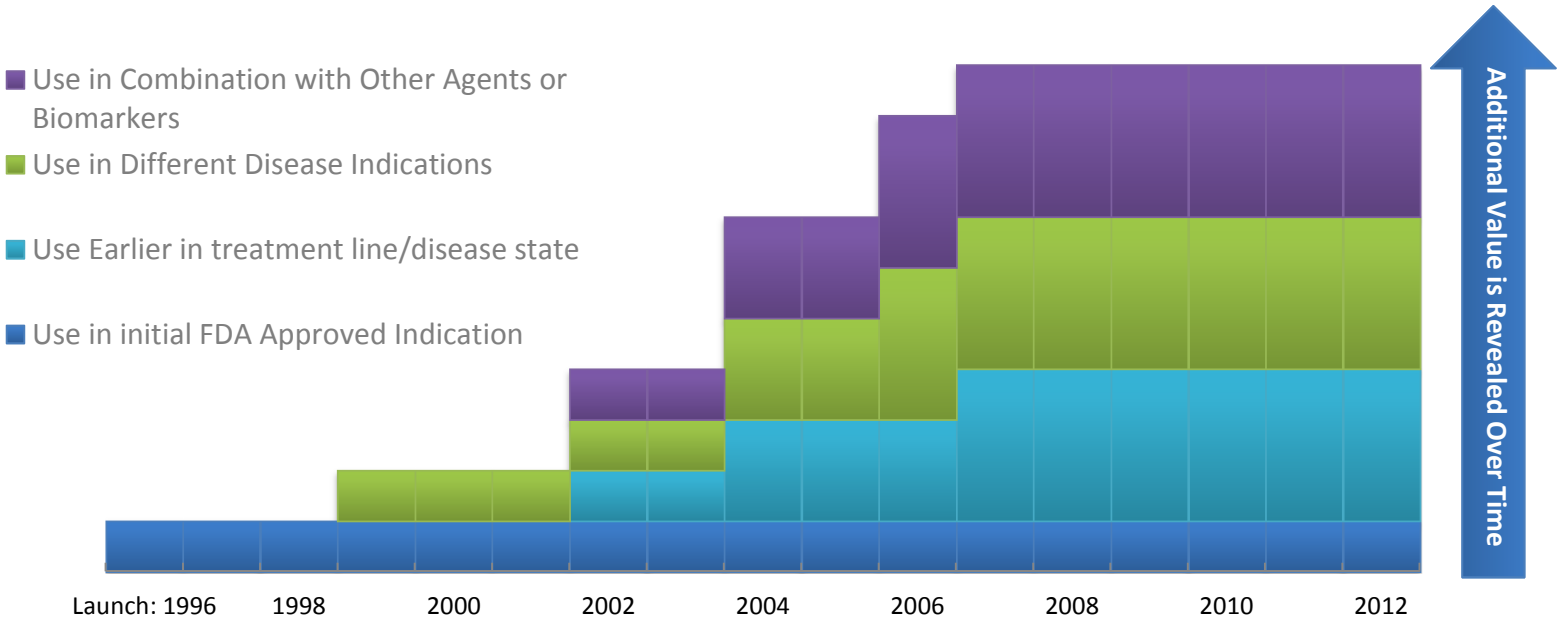
- After initial approval, continued research revealed that imatinib had an even greater impact when initiated earlier in the progression of the disease.
- Further research also revealed that imatinib was effective in combating other devastating forms of leukemia, as well as gastrointestinal stromal tumors.
- Today, survival rates have improved dramatically and **CML patients are living close to normal life spans.**



Clinical Value Evolves Over Time: Trastuzumab

Trastuzumab was originally approved by the FDA in 1998 to treat metastatic breast cancer in patients whose tumors expressed the HER2 protein. Approval was based on data showing that treatment slowed disease progression but long term survival data was unknown.

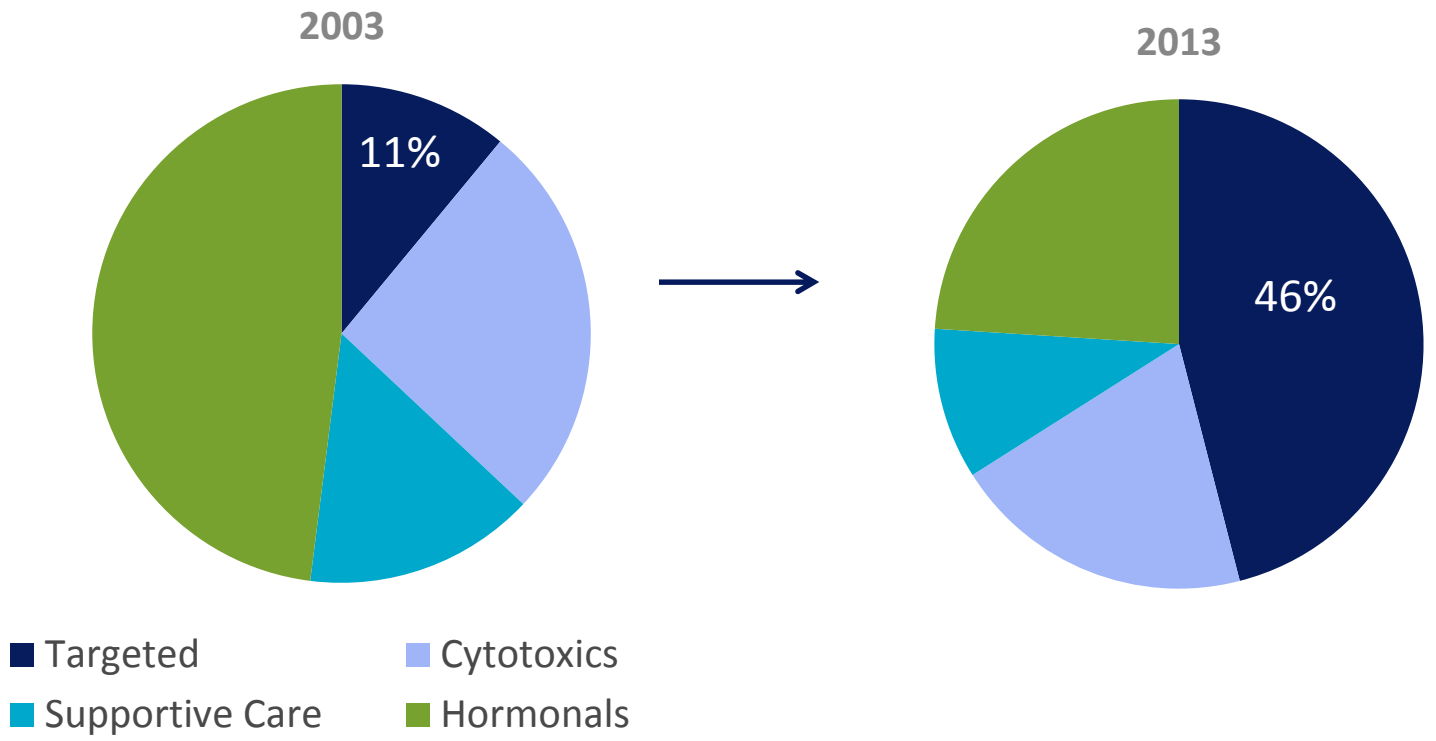
- 2001 data showed that median overall survival increased by 25% with the addition of trastuzumab.
- In 2008, the benefit of earlier treatment initiation was proven, giving trastuzumab an additional indication as early adjuvant therapy for breast cancer.
- Subsequent indications for use in combination to treat other types of gastric and gastroesophageal cancers followed.



The Role of Personalized Medicines Has Grown in the Last Decade

Personalized medicines provide effective and efficient care by targeting the right medicine to the right patient.

Oncology Treatment Modalities in Top Pharmaceutical Markets, 2003-2013



2 MEDICINES IN DEVELOPMENT

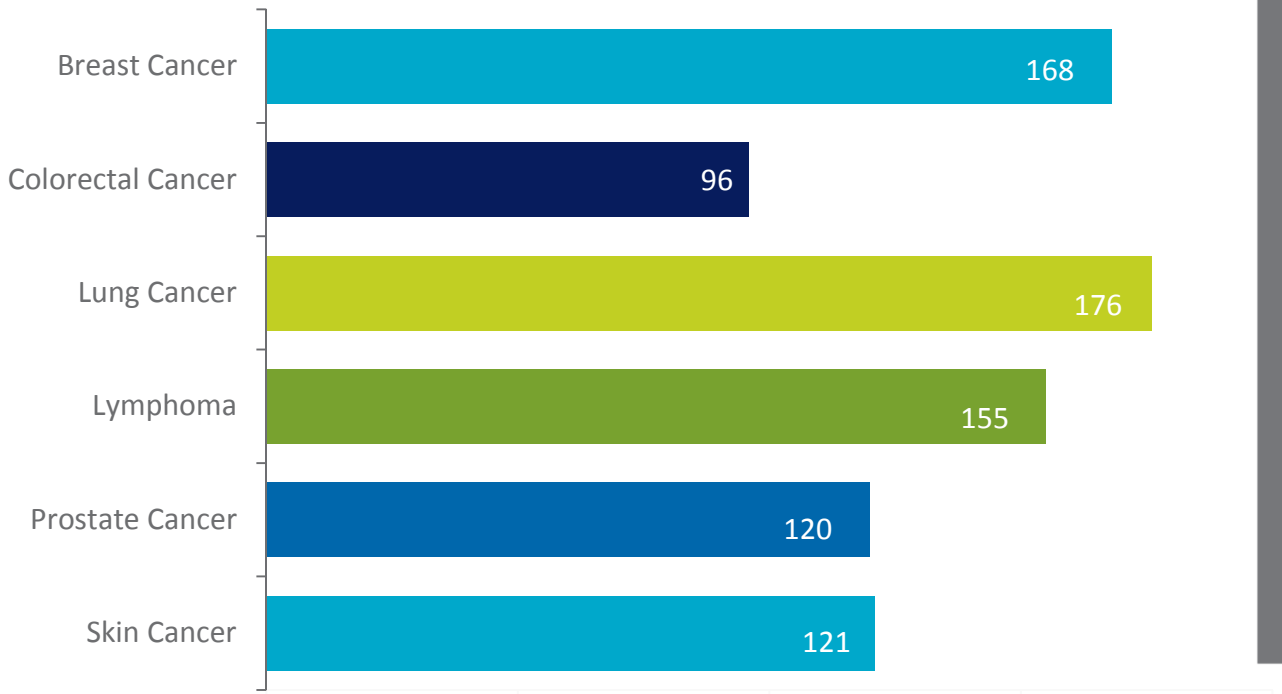
Discovery and Development of Cancer Medicines

The R&D process remains challenging and expensive but advances in basic science have opened new doors for biopharmaceutical researchers.

More than 1,000 new cancer medicines are in clinical development in the U.S. These medicines represent a wide range of novel approaches. Patients today have more reason to hope than ever before.

More than 1,000 Medicines in Development for Various Cancers

Number of Medicines in Development in 2014, Selected Cancer Types*



"Scientifically, we have never been in a better position to advance cancer treatment. … We now understand many of the cellular pathways that can lead to cancer. We have learned how to develop drugs that block these pathways."

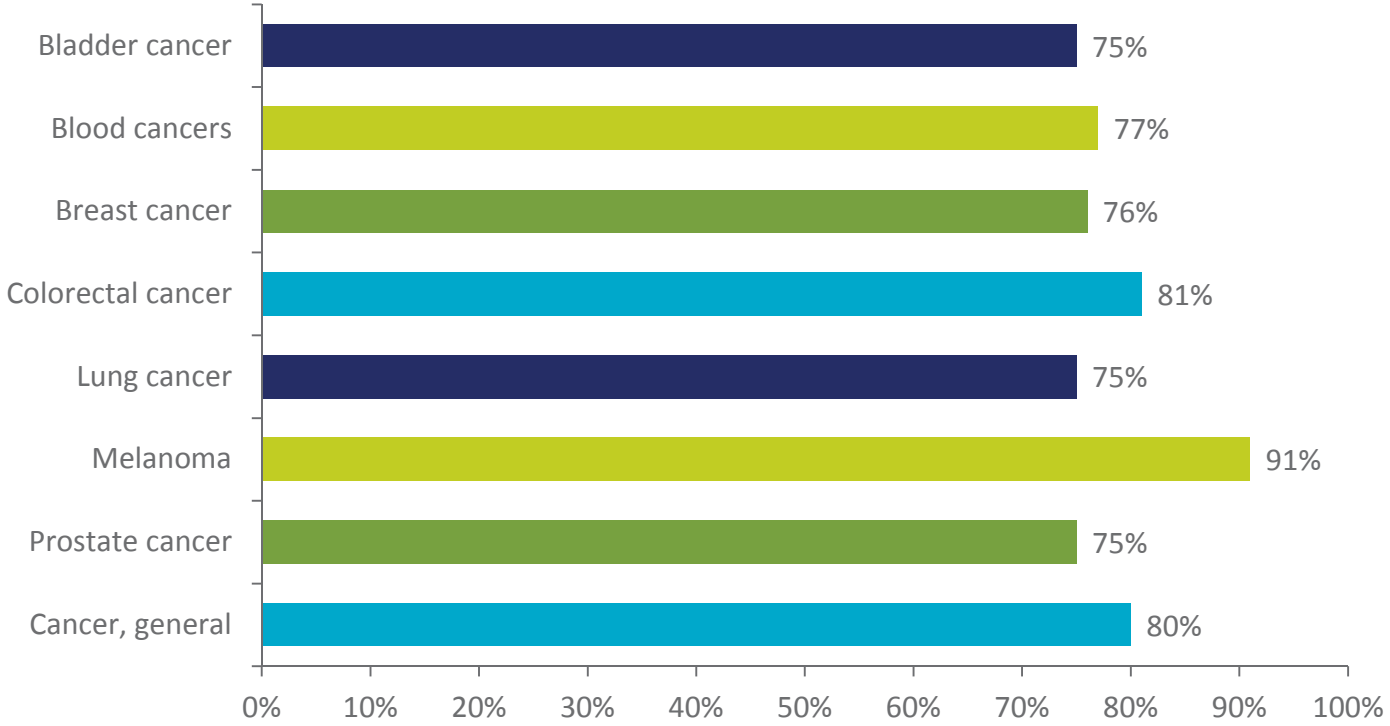
— Richard L. Schilsky, MD, Professor, University of Chicago

*Some medicines are being explored in more than one therapeutic category.

The Majority of Cancer Medicines in the Pipeline Would be New Approaches to Targeting Selected Cancers

Researchers are using novel approaches to attack cancer at the molecular level. An average of 80% of drugs in the oncology pipeline may be first-in-class medicines.

Percentage of Projects in Development that Are Potentially Novel Approaches in Selected Cancer Areas, 2011



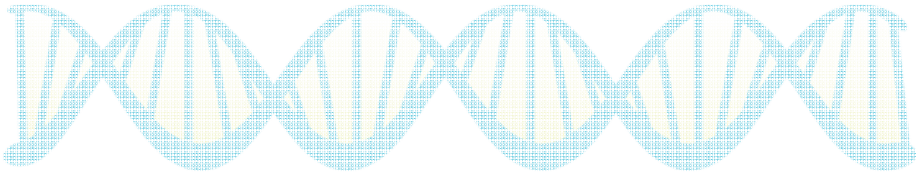
Personalized Medicine is Transforming Cancer Care

Biopharmaceutical companies are focused in this area resulting in several recent approvals and a growing number of new medicines in the pipeline.

12-50%

Of new drugs in the pipeline are reportedly personalized medicines (across all diseases)

> *"Oncology is on fire with [personalized medicine], with treatment selections based on individual molecular characteristics. This is also happening with chronic infectious diseases, and genetic diseases are not far behind."*
>
> — Janet Woodcock, Director
> Center for Drug Evaluation and Research,
> U.S Food and Drug Administration



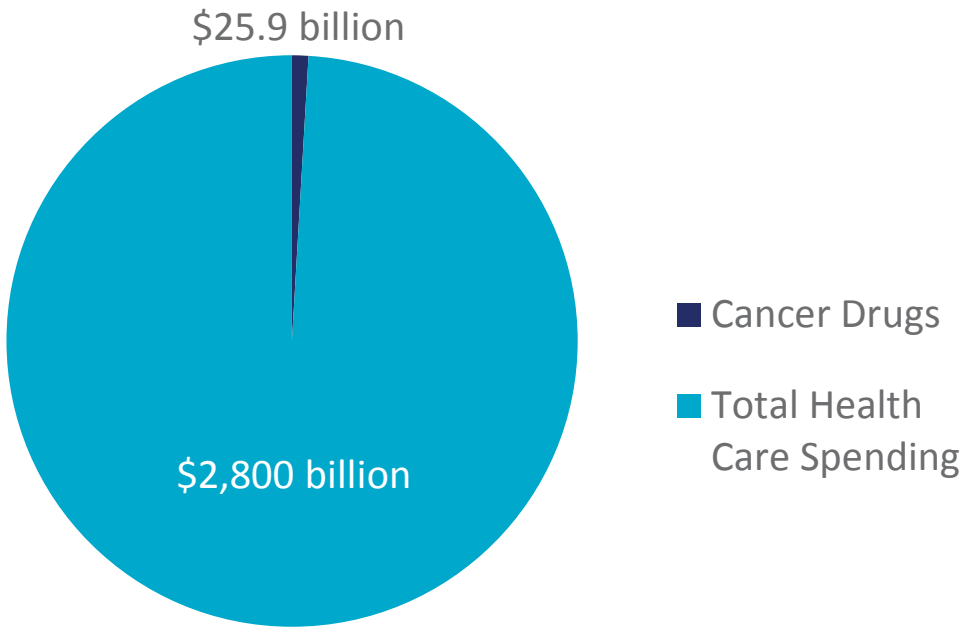
3 VALUE AND SPENDING

Understanding the Value of Cancer Medicines

New cancer medicines bring great value to patients and the health care system. It is important to view cancer spending in the context of health care spending overall. Likewise, cancer medicines are a crucial part of oncology treatment but represent just a small portion of cancer costs.

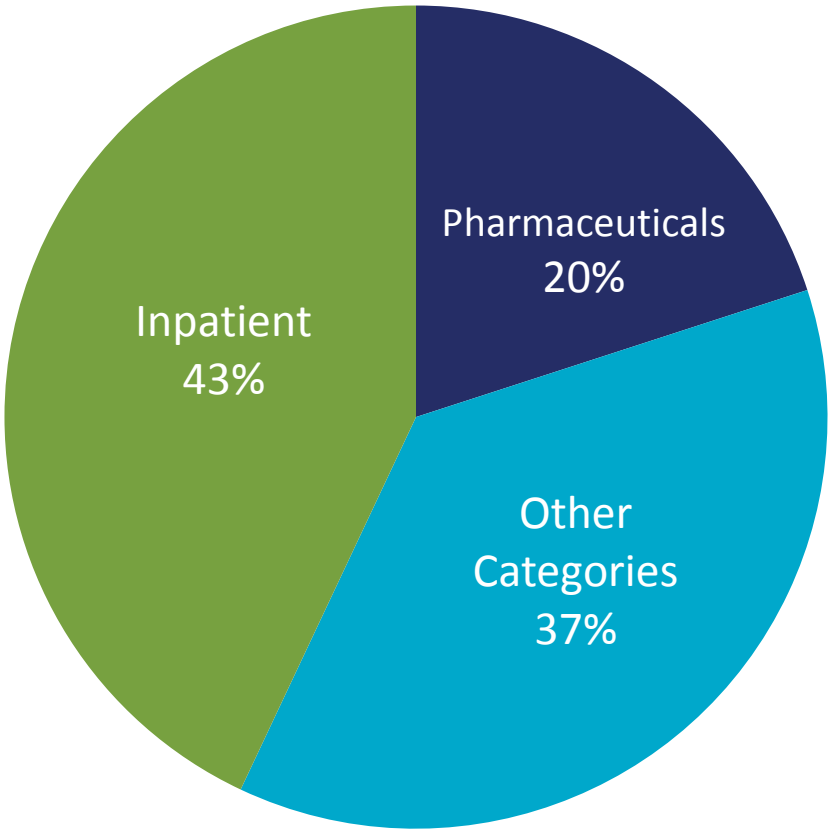
Spending on Cancer Medicines Represents Less Than 1% of Overall Health Care Spending

Cancer Medicines as a Portion of Total U.S. Health Care Spending, Billions, 2012



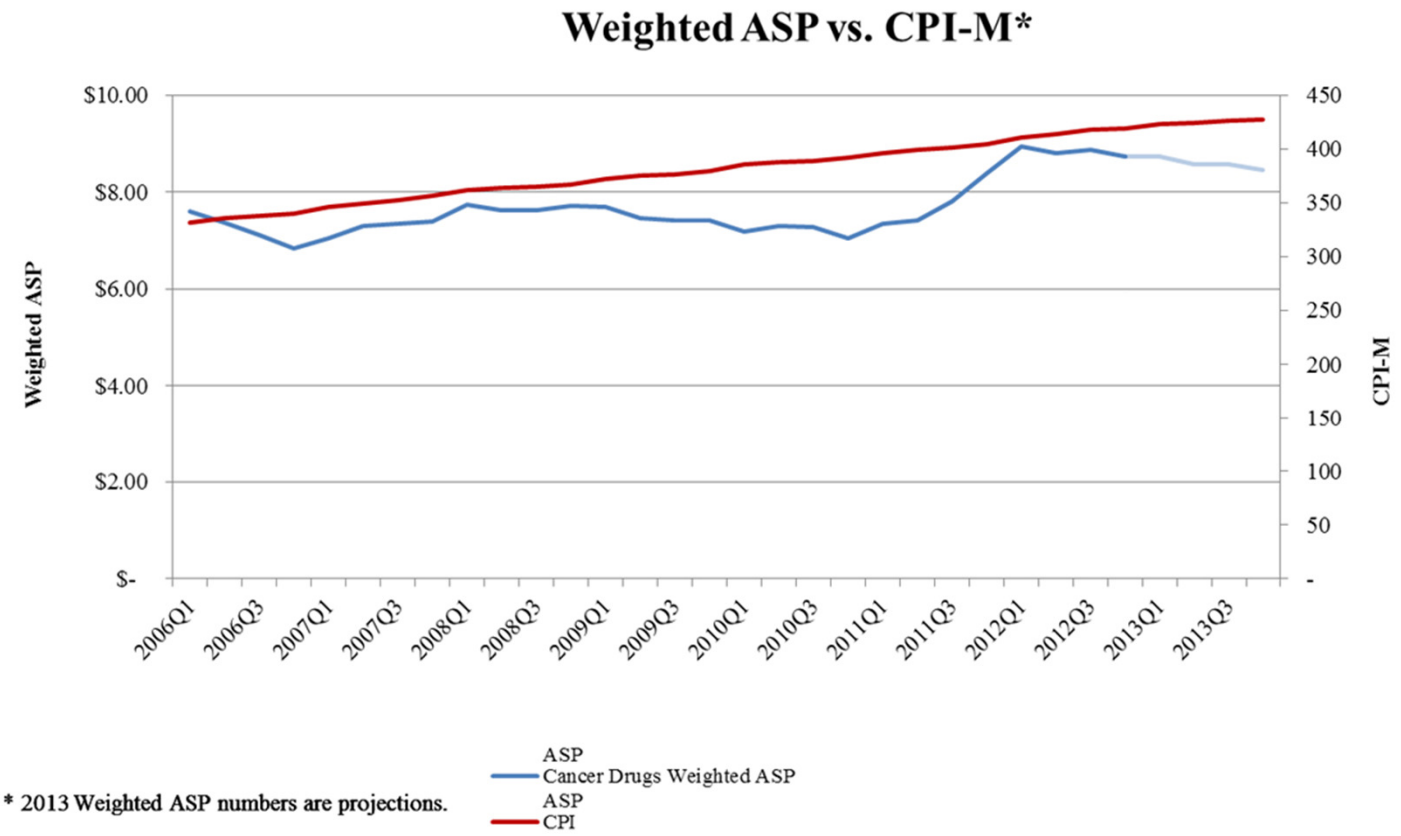
Cancer Medicines Represents 1/5 of Total Spending on Cancer Treatment

Total U.S. Cancer Care Spending, 2011



Average Price Growth of Cancer Drugs in Medicare Part B Less than Medical Inflation

The trend of volume-weighted Average Sales Price (ASP) for cancer drugs administered through Medicare Part B has been growing more slowly than overall medical inflation

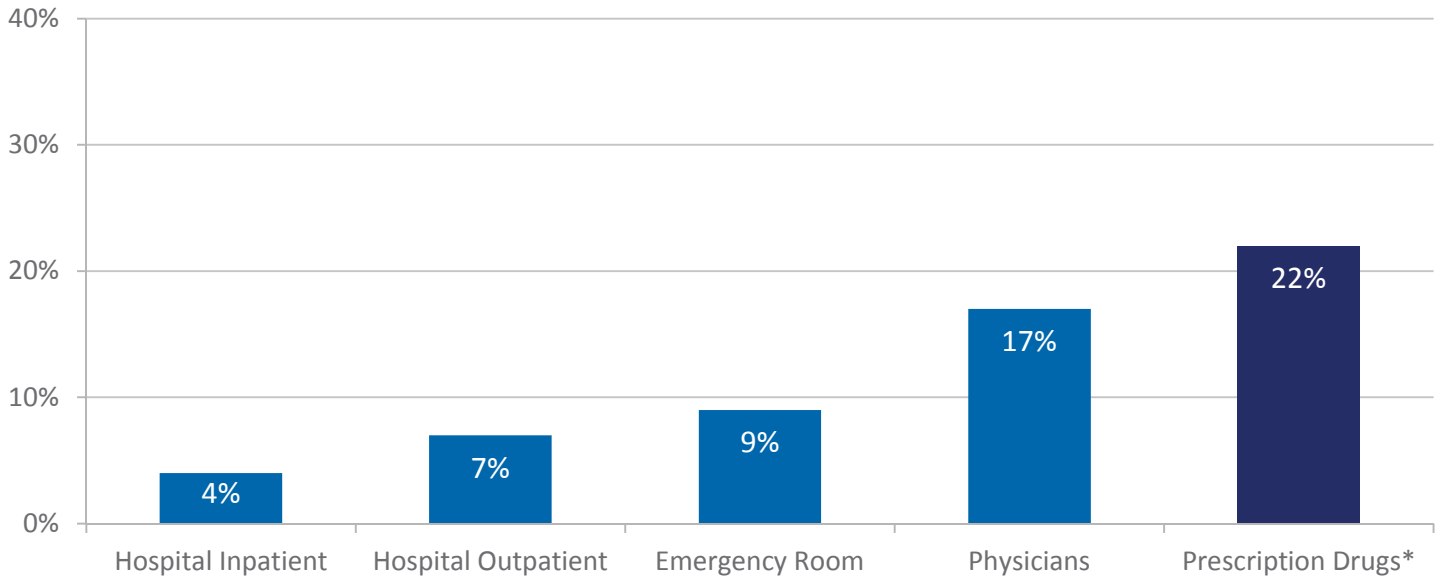


*Total retail sales including brand medicines and generics.

Insurance Covers a Lower Share of Prescription Drug Costs than of Other Medical Services

On average, privately insured consumers pay out-of-pocket more than 20% of their total prescription drug spending, compared to 4% of spending for inpatient hospital care and 7% on hospital outpatient care.

Average Patient Cost-Sharing by Type of Service in the Commercial Market



*Includes brand & generic.

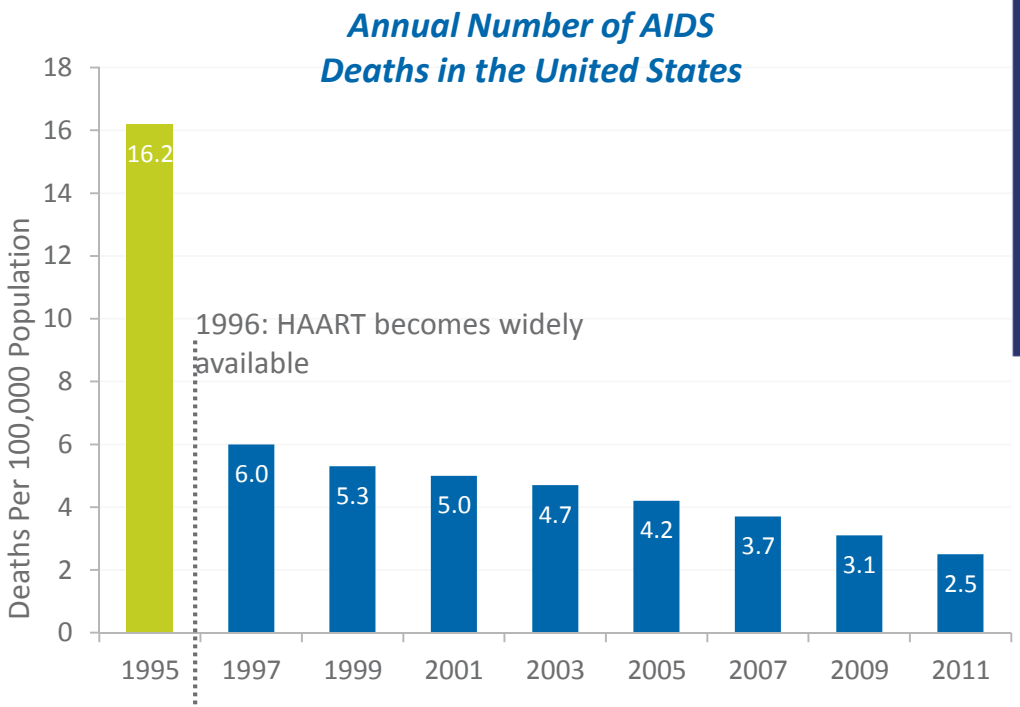
Substantial Gains to Society from Advances in Cancer Treatment

23 MILLION = Years of life saved due to cancer treatment advances, 1988-2000

$1.9 TRILLION = Value of improved cancer treatment to society based on improved productivity, extended life and other factors, 1988-2000

Innovations in Cancer Treatment Could Echo HIV/AIDS Successes

As HIV/AIDS treatments improved, spending became more sustainable.



"We used to think HIV costs would overwhelm us….but we figured it out and let drug development progress… similarly, cancer care will evolve."

— Ira Klein, M.D., M.B.A., FACP, Aetna

"Remember HIV?.... thanks to a wave of new discoveries that came both from academic centers and the pharmaceutical industry, the HIV crisis was transformed into a stable condition which is managed very differently by society where good drugs are available. They are controlling the disease, and society has been saving an enormous amount of money as a result of these innovative drugs by providing better care out of hospitals."

— Hervé Hoppenot, President, Incyte Pharmaceuticals

Fostering Innovation

At a time when the scientific promise is greater than ever, we must find ways to promote innovation while also addressing health care costs.

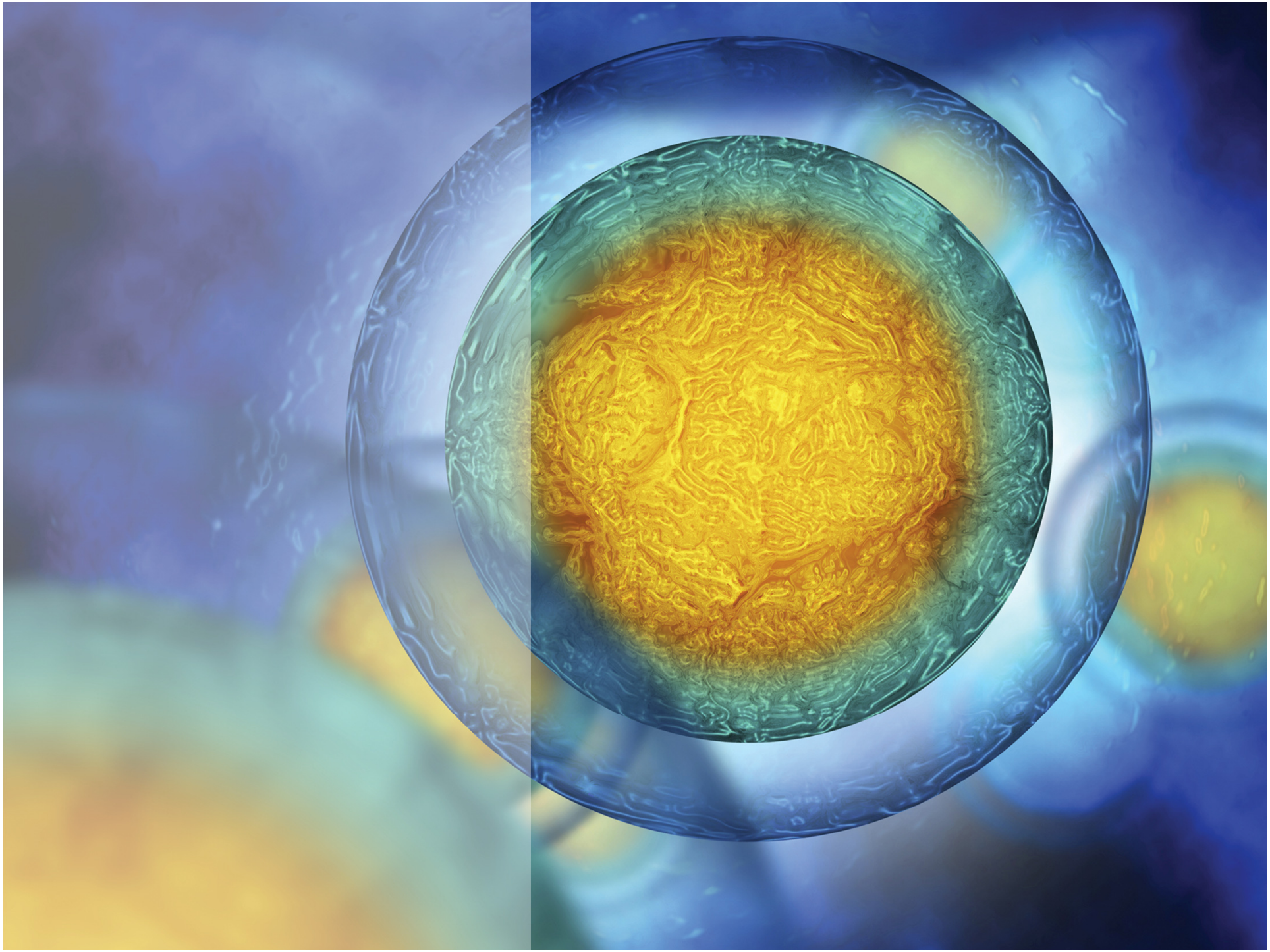
- Coverage and payment policies that support and encourage medical innovation
  - Value assessments that align with innovation
- A well-functioning, science-based regulatory system
- Strong intellectual property protections

"By bringing the stakeholder community together, stimulating dialogue, and communicating with policymakers, we are optimistic that continued scientific progress toward solutions to long-term human and economic challenges can be realized within regulatory and policy frameworks that incentivize innovation and value in healthcare."

— A. Abernethy, et al.
Clinical Cancer Research

"There is no question that when we talk about turning the tide against cancer, the most exciting opportunities, the new opportunities in fact, are understanding the biology and applying that biology to new treatments. We are certainly at a turning point."

— J. Leonard Lichtenfeld, M.D., American Cancer Society



Pharmaceutical Research and Manufacturers of America
950 F Street, NW, Suite 300
Washington, DC 20004

www.phrma.org

PhRMA
RESEARCH • PROGRESS • HOPE