



THE VALUE OF



MEDICAL INNOVATION  
IN CANCER



*Committed to  
improving the lives  
of patients worldwide®*





“ Just as **energy** is the **basis of life** itself, and **ideas** the source of **innovation**, so is innovation the vital **spark** of all human change, improvement and progress.”

American Economist  
**Theodore Levitt**



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# INTRODUCTION

Medical innovation is the greatest source of longer life and economic prosperity. In the 21<sup>st</sup> century, medical innovation will dramatically improve health outcomes, reduce the overall cost of healthcare and stimulate the growth of the global economy, producing a world that can be free from cancer. This virtuous cycle of innovation in turn stimulates investment in biomedical research to further improve health and create economic value throughout the world.





# 1

## VIRTUOUS CYCLE OF INNOVATION



### MEDICAL INNOVATION IS THE SOURCE OF LONGER LIFE AND BETTER HEALTH.

We will show that among the technological innovations of the 20<sup>th</sup> century, medical innovation has contributed more to our ability to live longer, healthier and more prosperous lives than anything else.

Medical innovation is turning knowledge about disease mechanisms at the genetic and cellular level into breakthrough therapies that cure or prevent illness. We will demonstrate that medical innovation in particular brings about a virtuous cycle of better health, longer life and greater prosperity that in turn stimulates additional investment in even more advanced innovations for preventing and treating disease.





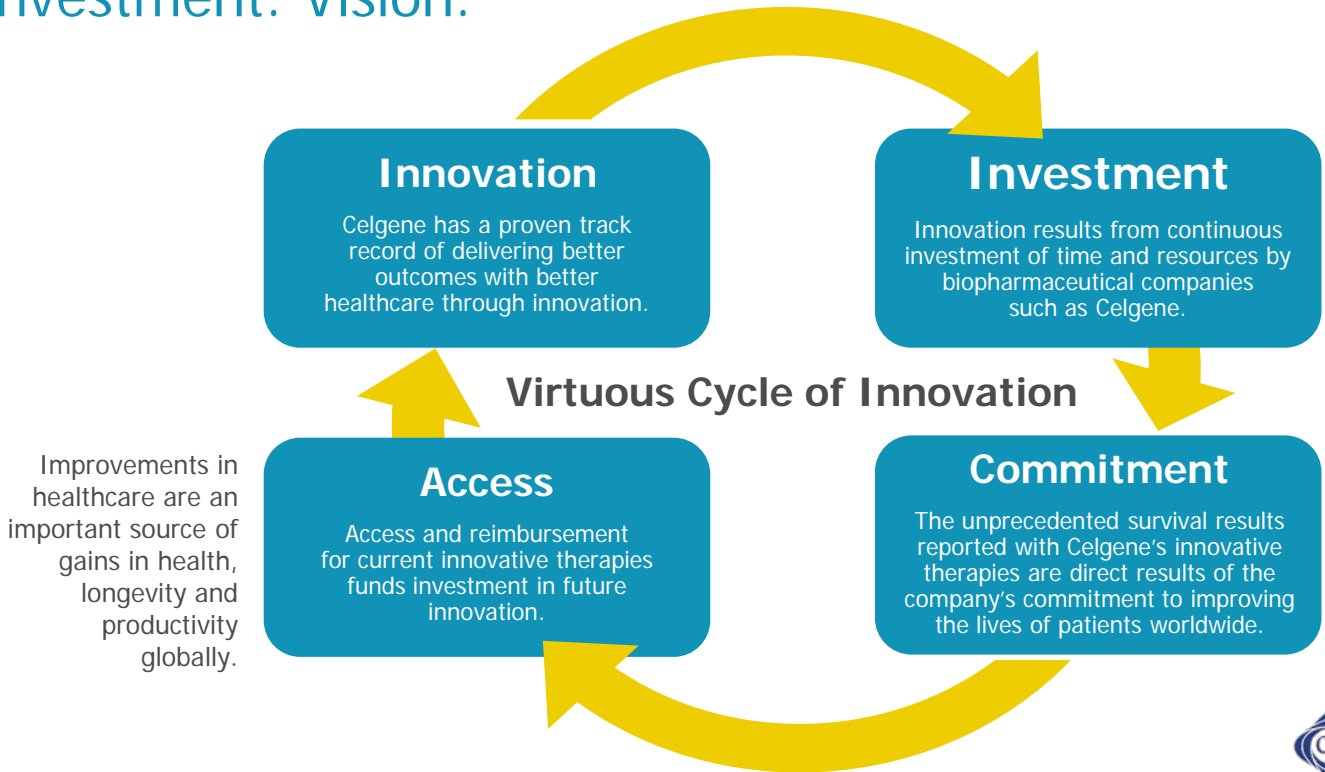


“ This is the **most exciting time** in the history of medicine. If we can make some **radical changes** to accommodate the enormous opportunities, there will be **better health** at **lower costs** for many generations to come. ”

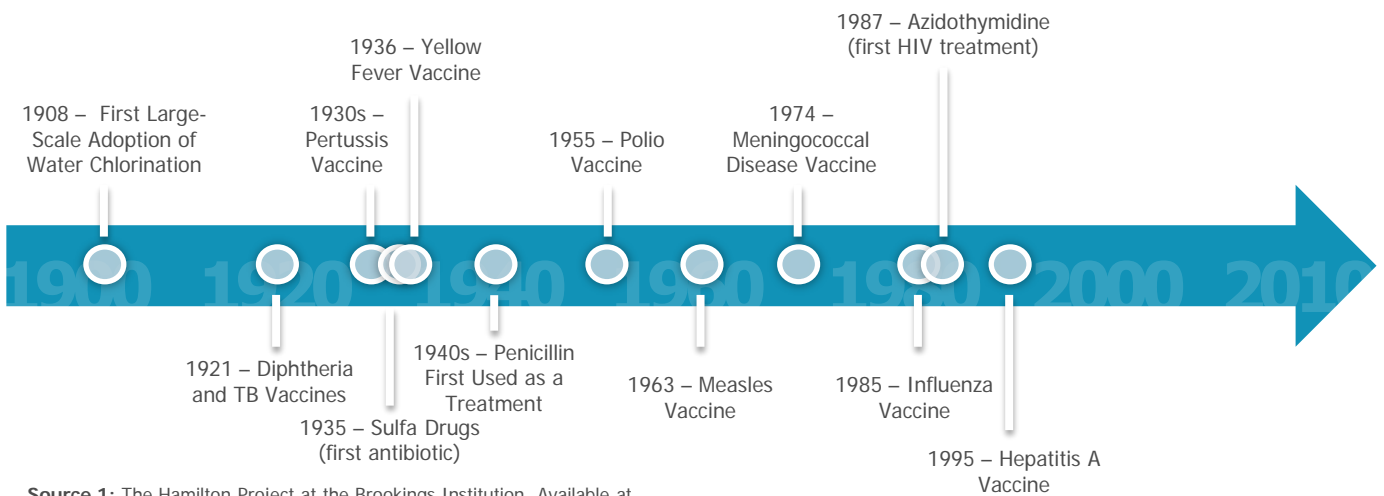
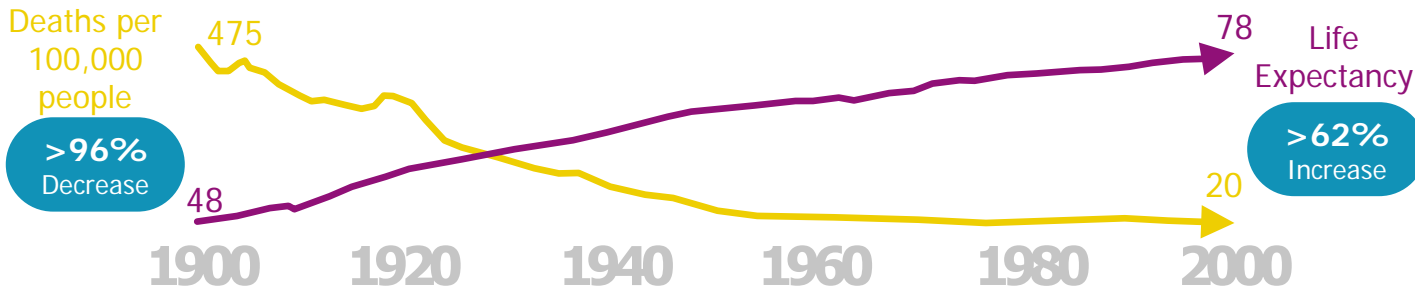
*The Creative Destruction of Medicine* Author  
**Eric Topol, MD**

# 1 VIRTUOUS CYCLE

## Innovation is a Virtuous Cycle. Requiring Commitment. Investment. Vision.



# Longer Life Through Better Medicines 1900-2010



Source 1: The Hamilton Project at the Brookings Institution. Available at [http://www.hamiltonproject.org/multimedia/charts/deaths\\_from\\_major\\_infectious\\_disease/](http://www.hamiltonproject.org/multimedia/charts/deaths_from_major_infectious_disease/). Accessed on 11/1/12.

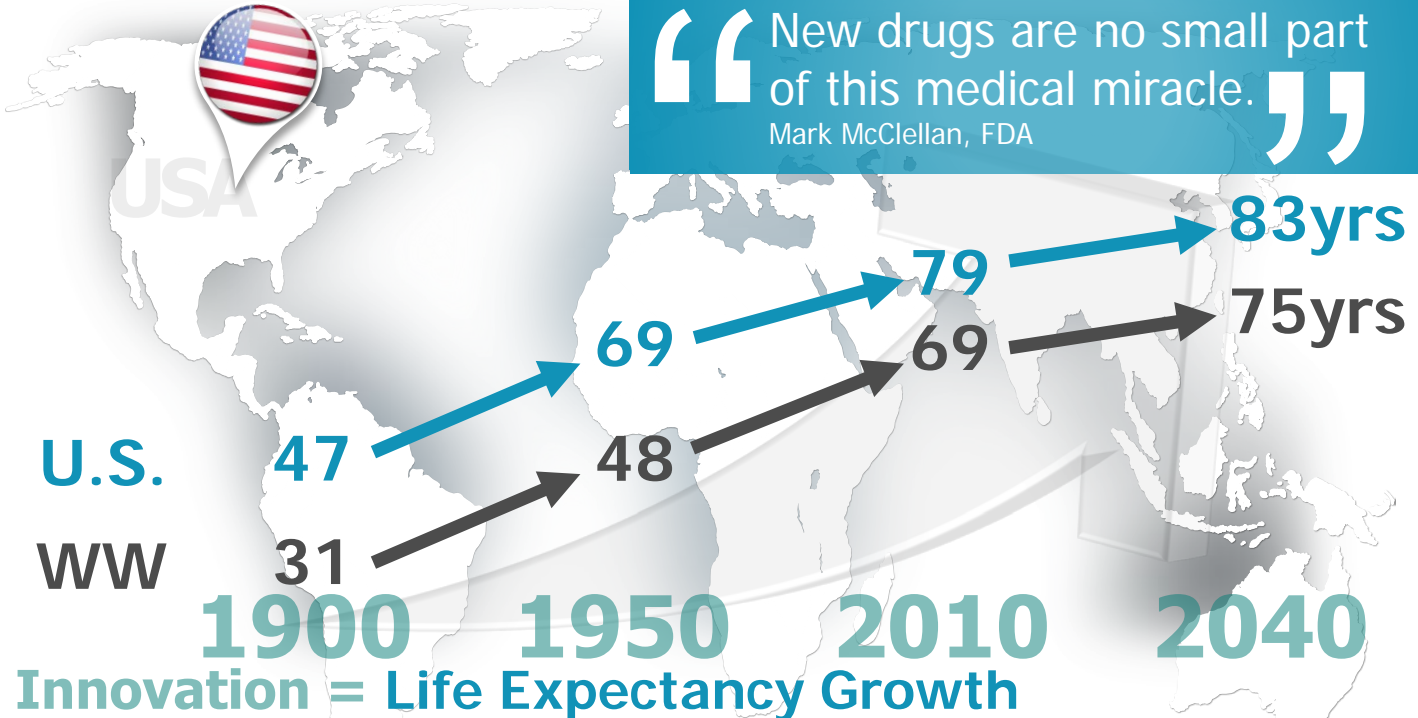
## The Impact of Medical Innovation



In July 1999, the U.S. Centers for Disease Control and Prevention's *Morbidity and Mortality Weekly Report* noted the death of a 90-year-old woman. She had been deathly ill in 1942, but as a last resort, doctors treated her with what was then called “an obscure, experimental drug.” And this 33-year-old woman went from death’s door to get married, raise a family and live nearly 60 more years. In fact, she was the first U.S. civilian whose life was saved by ...

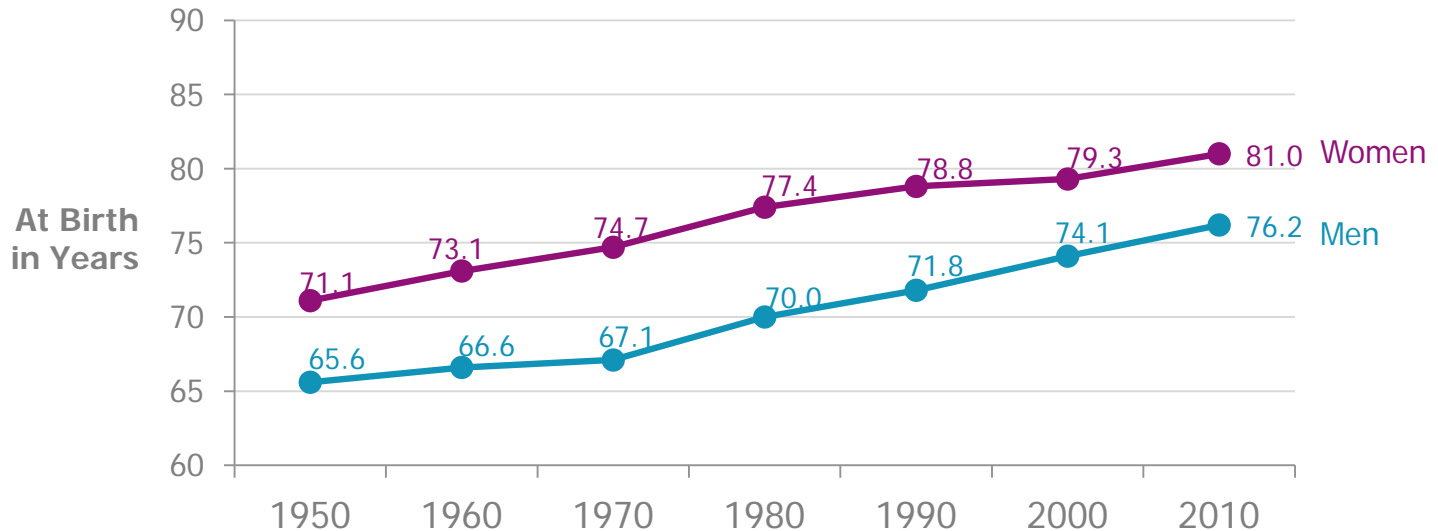
*penicillin.*

# The Gift of Life



**Source 3:** United Nations Development Program. World Population Prospects The 2010 Revision. Volume 1: Comprehensive Tables. Available at [http://esa.un.org/wpp/Documentation/pdf/WPP2010\\_Volume-I\\_Comprehensive-Tables.pdf](http://esa.un.org/wpp/Documentation/pdf/WPP2010_Volume-I_Comprehensive-Tables.pdf). Accessed 10/30/13. U.S. Food and Drug Administration. Centers for Disease Control and Prevention and National Center for Health Statistics. Health, United States, 2011: With Special Feature on Socioeconomic Status and Health. Available at <http://www.cdc.gov/nchs/index.htm>. Accessed 11/12/12. Prentice T. Health, History and Hard Choices: Funding Dilemmas in a Fast-Changing World. Nonprofit and Voluntary Sector Quarterly. 2008: Supplement to vol.37; no 1: 63S-75S.

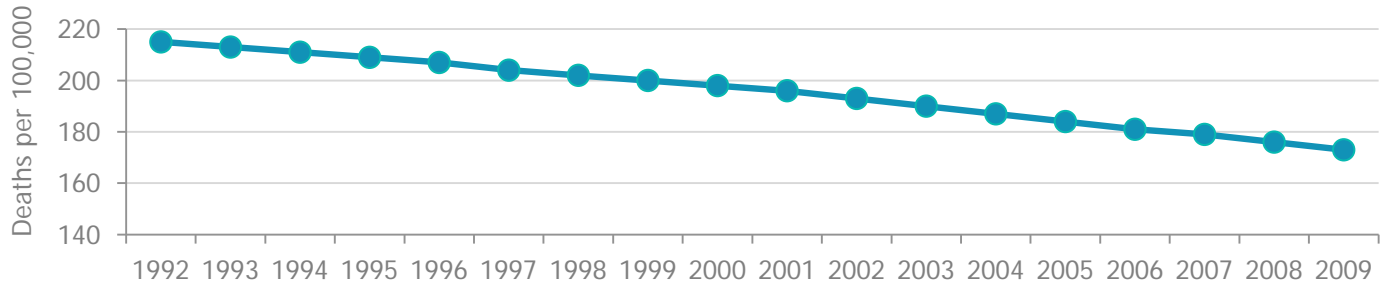
## Life Expectancy Gains Are Clear in the U.S.



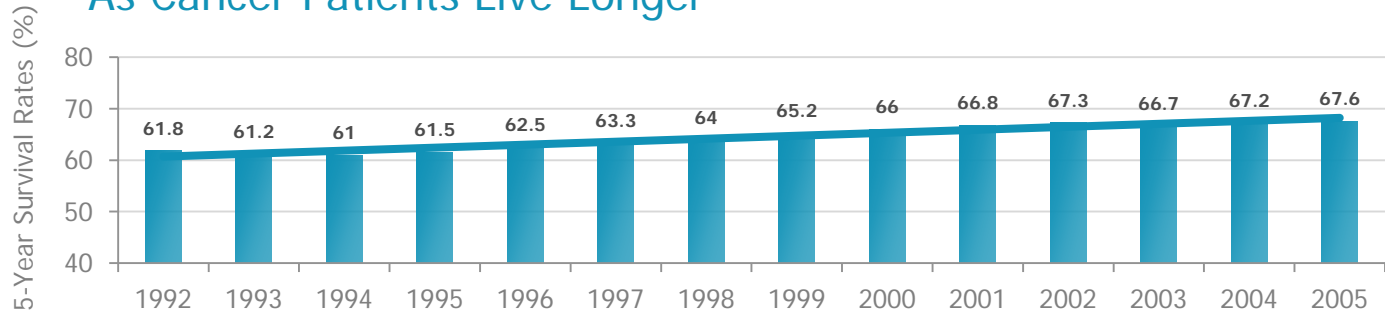
**Source 4:** U.S. Department of Health and Human Services, Centers for Disease Control and Prevention, National Center for Health Statistics, Health, United States, 2008 With Chartbook (Hyattsville, MD: HHS, 2009); 1950-2006 data from Heron M, et al. Deaths: Final Data for 2006. National Vital Statistics Reports 57, no.14, (Hyattsville, MD: National Center for Health Statistics, August 2009): 5. Available at [http://www.cdc.gov/nchs/data/nvsr/nvsr57/nvsr57\\_14.pdf](http://www.cdc.gov/nchs/data/nvsr/nvsr57/nvsr57_14.pdf). Accessed June 2010]; 2007 data from Xu J, et al. Deaths: Final Data for 2007. National Vital Statistics Reports 58, no.19. (Hyattsville, MD: National Center for Health Statistics, May 2010): 13. Available at [http://www.cdc.gov/nchs/data/nvsr/nvsr58/nvsr58\\_19.pdf](http://www.cdc.gov/nchs/data/nvsr/nvsr58/nvsr58_19.pdf). Accessed June 2010. 2008-2009 data from K. Kochanek, et al. Deaths: Preliminary Data for 2009. National Vital Statistics Reports 59, no.4, (Hyattsville, MD: National Center for Health Statistics, March 2011): 28. Available at [http://www.cdc.gov/nchs/data/nvsr/nvsr59/nvsr59\\_04.pdf](http://www.cdc.gov/nchs/data/nvsr/nvsr59/nvsr59_04.pdf). Accessed August 2011. 2010 data from K. Kochanek, et al. Deaths: Final Data for 2010. National Vital Statistics Reports 61, no.4, (Hyattsville, MD: National Center for Health Statistics, May 2013): 12. Available at [http://www.cdc.gov/nchs/data/nvsr/nvsr61/nvsr61\\_04.pdf](http://www.cdc.gov/nchs/data/nvsr/nvsr61/nvsr61_04.pdf). Accessed July 2013.

# Medical Innovation is Increasing Patient Survival

## Cancer-Related Death Rates Have Declined Since 1990



## As Cancer Patients Live Longer



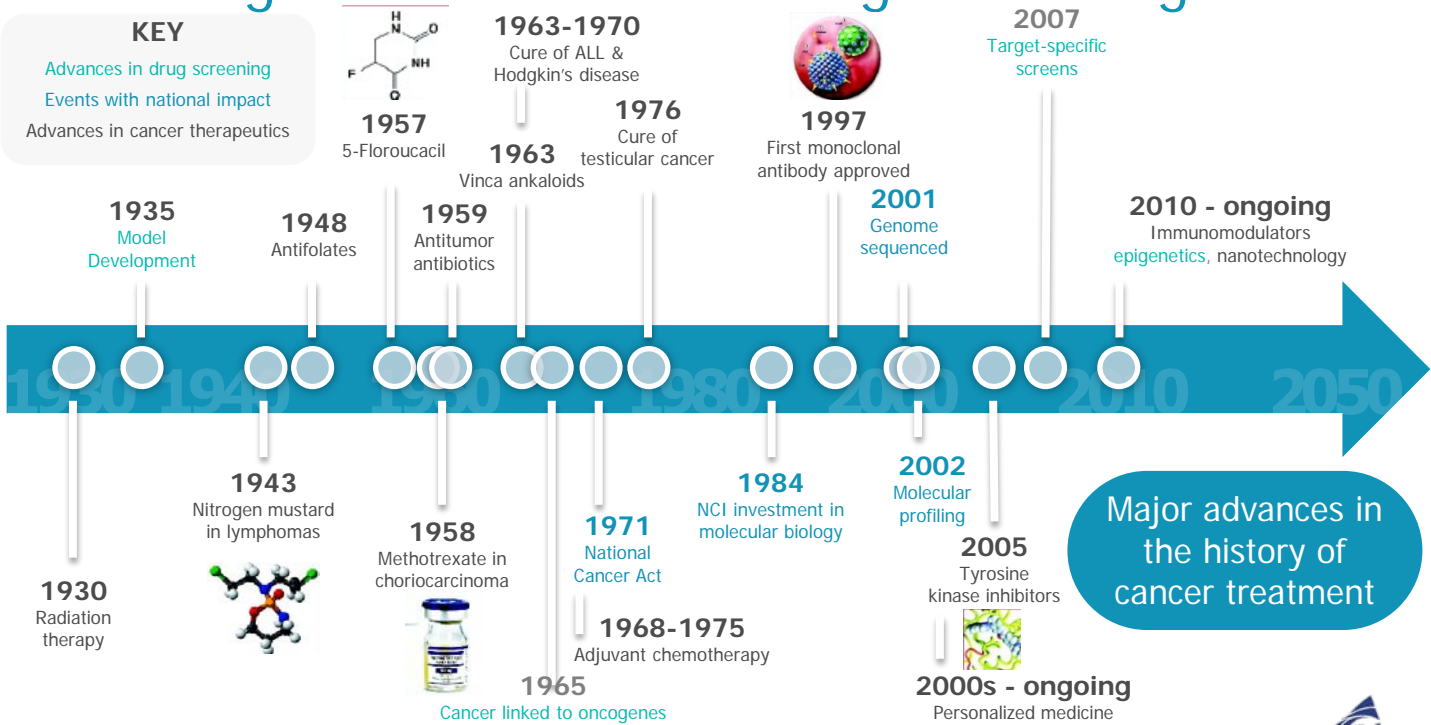
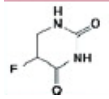
**Source 5:** National Cancer Institute, Surveillance Epidemiology and End Results (SEER). Available at <http://seer.cancer.gov/faststats/selections.php>. Accessed 11/14/13. Center for Disease Control (CDC) Health, United States, 2012 Table 28. Death rates for malignant neoplasms, by sex, race, Hispanic origin, and age: United States, selected years 1950–2010. Available at <http://www.cdc.gov/nchs/hus/contents2012.htm#028>. Accessed 11/14/13.

# 1 VIRTUOUS CYCLE

## Cancer Continuum: Advancing Patients from Surviving to Thriving

### KEY

- Advances in drug screening
- Events with national impact
- Advances in cancer therapeutics



Major advances in the history of cancer treatment





# New Therapies are the Greatest Contributor to Increased Life Expectancy



1986 - 2000

New therapies account for 40% of the increase in life expectancy

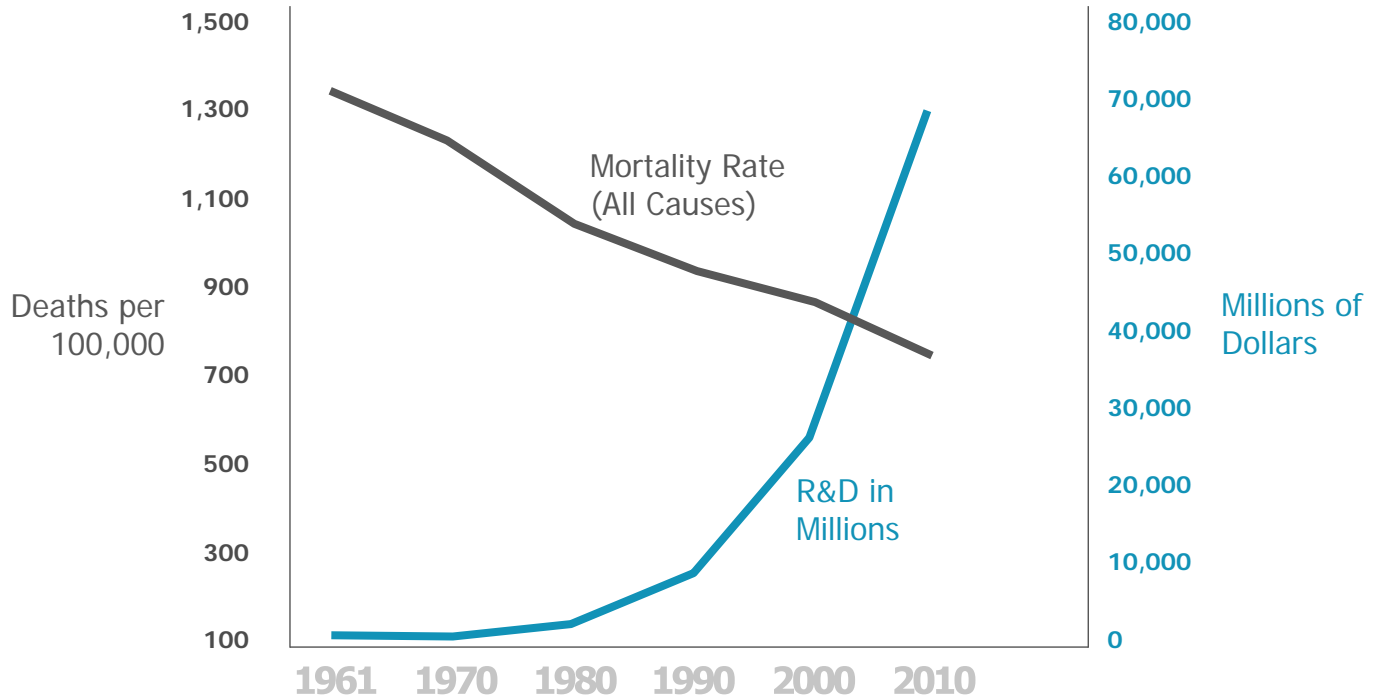


2000 - 2009

New therapies account for 73% of the increase in life expectancy

**Source 7:** Lichtenberg FR. NBER Working Paper No. 18235. Pharmaceutical innovation and longevity growth in 30 developing and high-income countries, 2000-2009. Available at <http://www.nber.org/papers/w18235>. Accessed 10/30/13. Lichtenberg FR. NBER Working Paper No. 9754. The impact of new drug launches on longevity: evidence from longitudinal disease-level data from 52 countries, 1982-2001. Available at <http://www.nber.org/papers/w9754>. Accessed 11/12/13.

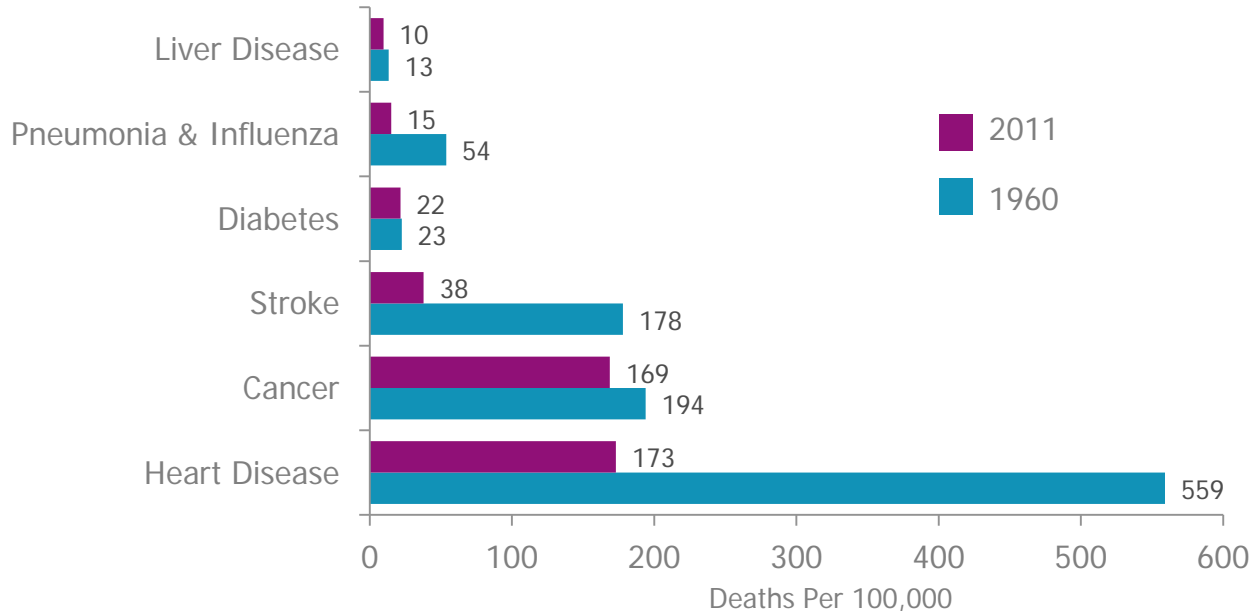
# Impact of New Therapies on Death Rates



**Source 8:** Centers for Disease and Prevention's National Center for Health Statistics. Health, United States, 2011: With Special Feature on Socioeconomic Status and Health. Available at <http://www.cdc.gov/nchs/>. Accessed 11/14/13. Pharmaceutical Research and Manufacturers of America, PhRMA Annual Membership Survey, 2013. Available at [www.phrma.org](http://www.phrma.org). Accessed 11/14/13.



# Decline in Death Rates from Leading Causes in 1960 vs. 2011



**Source 9:** Centers for Disease Control and Prevention and National Center for Health Statistics. National Vital Statistics System. 2011. Available at [http://www.cdc.gov/nchs/data/nvsr/nvsr61/nvsr61\\_06.pdf#page1](http://www.cdc.gov/nchs/data/nvsr/nvsr61/nvsr61_06.pdf#page1). Accessed 11/12/12.

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3. United Nations Development Program. World Population Prospects The 2010 Revision. Volume 1: Comprehensive Tables. Available at [http://esa.un.org/wpp/Documentation/pdf/WPP2010\\_Volume-I\\_Comprehensive-Tables.pdf](http://esa.un.org/wpp/Documentation/pdf/WPP2010_Volume-I_Comprehensive-Tables.pdf). Accessed 10/30/13. U.S. Food and Drug Administration. Centers for Disease Control and Prevention and National Center for Health Statistics. Health, United States, 2011: With Special Feature on Socioeconomic Status and Health. Available at <http://www.cdc.gov/nchs/index.htm>. Accessed 11/12/12. Prentice T. Health, History and Hard Choices: Funding Dilemmas in a Fast-Changing World. Nonprofit and Voluntary Sector Quarterly. 2008; Supplement to vol 37; no 1: 63S-75S.
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6. DeVita VT, Chu E. A History of Cancer Chemotherapy. *Cancer Res* 2008;68:8643-8653. U.S. Food and Drug Administration. Available at [www.fda.gov](http://www.fda.gov). Accessed 11/1/12. ©2008 by American Association for Cancer Research
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8. Centers for Disease and Prevention's National Center for Health Statistics. Health, United States, 2011: With Special Feature on Socioeconomic Status and Health. Available at <http://www.cdc.gov/nchs/>. Accessed 11/14/13. Pharmaceutical Research and Manufacturers of America, PhRMA Annual Membership Survey, 2013. Available at [www.phrma.org](http://www.phrma.org). Accessed 11/14/13.

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9. Centers for Disease Control and Prevention and National Center for Health Statistics. National Vital Statistics System. 2011. Available at <http://www.cdc.gov/nchs/nvss.htm>. Accessed 11/12/12.





# 2

## PROGRESS & PROSPERITY



### MEDICAL INNOVATION LEADS TO GREATER ECONOMIC GROWTH AND PROSPERITY.

We will demonstrate that medical innovation leads to improved health outcomes, lower costs, higher productivity, reduced disability, greater economic value to society increased wealth and expanding gross domestic product (GDP).

We will show through specific examples (polio, infectious disease, diabetes, heart disease, HIV, and in recent years, cancer) how innovations have reduced the cost of care and increased economic well-being.

The value of increasing the quality and length of life is substantial; longer life and greater well-being leads to greater productivity and economic growth.





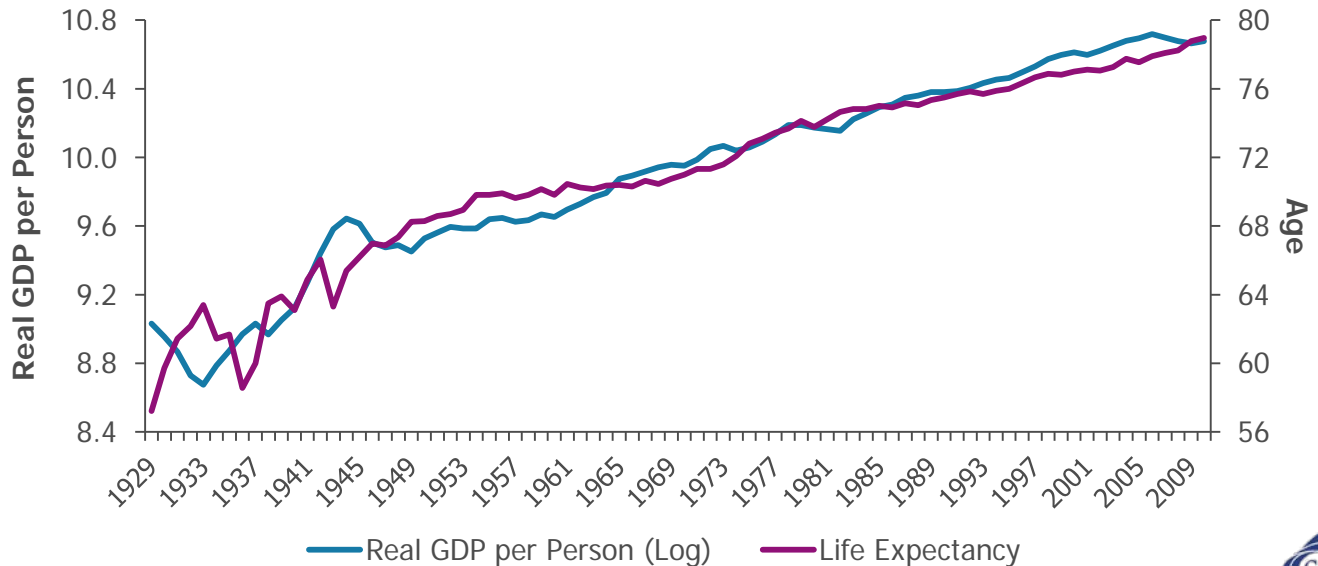


“Over the last half century, improvements in **health** have been as **valuable** as all other sources of **economic growth** and **productivity** combined.”

University of Chicago Economists  
**Kevin Murphy, Ph.D., and Robert Topel, Ph.D.**

# Increasing Life Expectancy Sustains Economic Growth

## U.S. Life Expectancy vs. Real GDP per Person 1929 to 2010



# Tremendous Contributions to Economic Growth



Over the past 50 years, medical innovation  
has been the source of more than

$\frac{1}{2}$

of **all** economic growth

Source 2: Murphy KM and Topel RH. The Value of Health and Longevity. *J Polit Econ*. 2006; 114(4); 871-904.

**2** PROGRESS &  
PROSPERITY

## Medical Innovation Adds Value, Reduces Costs and Saves Lives



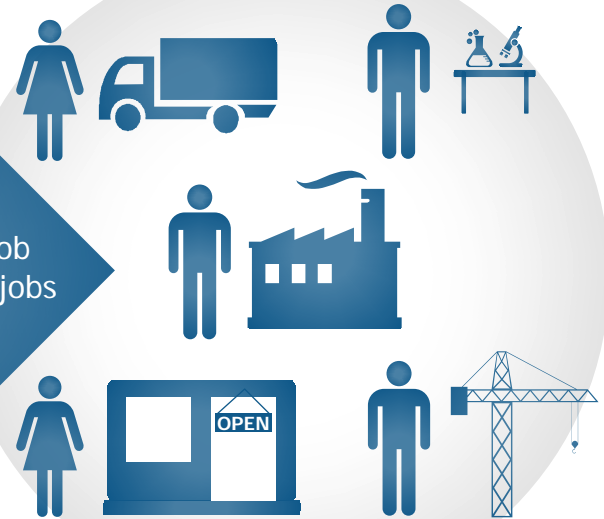
# Greatest Source of Jobs in 21<sup>st</sup> Century



## BIOPHARMA JOBS

More than 810,000 jobs in the U.S. biopharmaceutical sector

Each direct biopharmaceutical job supports 5 additional jobs in other sectors



## TOTAL JOBS SUPPORTED

3.4 million total U.S. jobs supported by the biopharmaceutical sector

**Source 4:** Battelle Technology Partnership Practice. The U.S. Biopharmaceuticals Sector: Economic Contribution to the Nation. July 2013. Available at <http://phrma.org/sites/default/files/pdf/The-Economic-Impact-of-the-US-Biopharmaceutical-Industry.pdf>. Accessed 7/23/12.

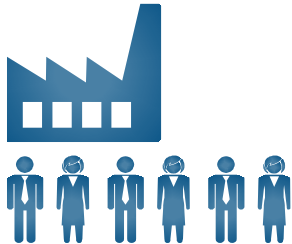
# Human Genome Project

## Why Incentivize the Innovators?

1990

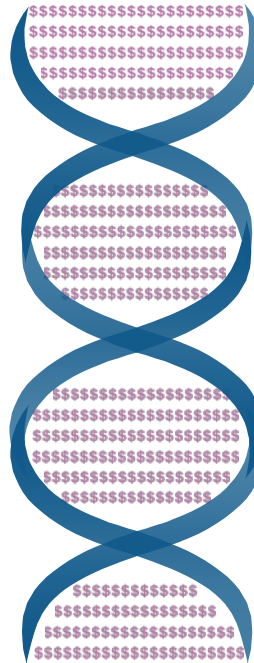
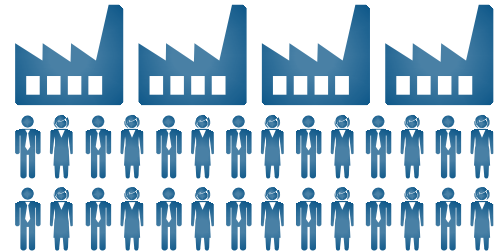
**\$3.8 billion**

U.S. Investment in the Human Genome Project

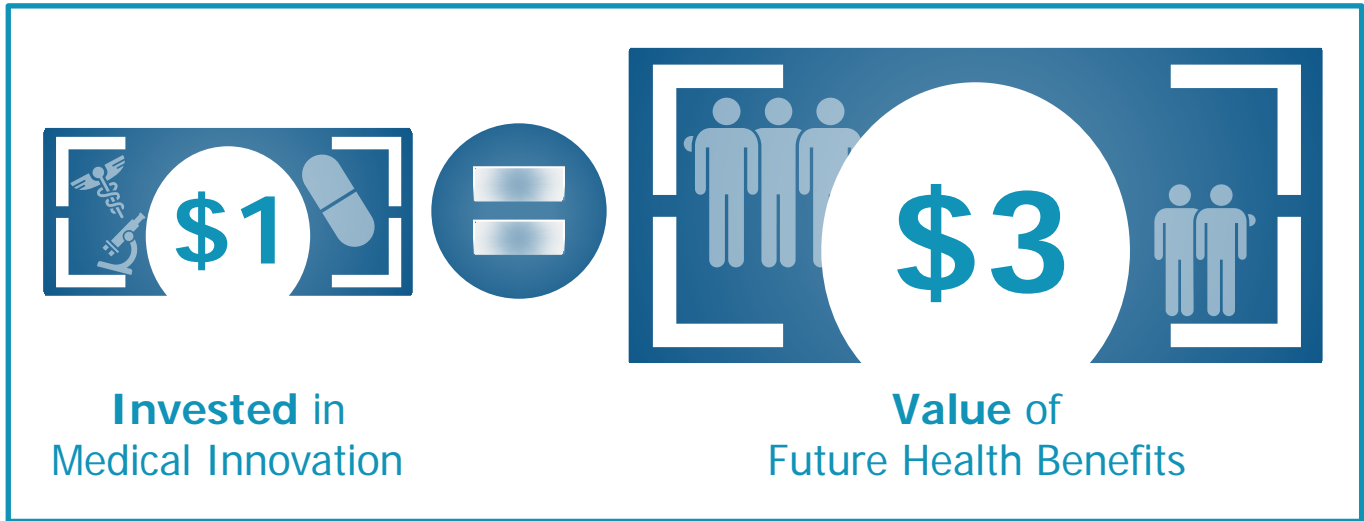


2003

ROI on Human Genome Project: **\$796 billion**  
**310,000 jobs**



# Investments in Medical Innovation Yield Significant Future Health Benefits to Society



**Source 6:** Hughes JW, Moore MJ and Snyder EA. "Napsterizing" Pharmaceuticals: Access, Innovation, and Consumer Welfare. Published 1/19/11. Available at <http://www.ftc.gov/os/comments/intelpropertycomments/snydermoorehughes.pdf>. Accessed 11/15/13.

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3. Lichtenberg FR. Benefits and Costs of Newer Drugs: An Update. National Bureau of Economic Research, Working Paper No. 8996. June 2007. Available at <http://www.nber.org/papers/w8996.pdf>. Accessed 11/14/13.
4. Battelle Technology Partnership Practice. The U.S. Biopharmaceuticals Sector: Economic Contribution to the Nation. July 2013. Available at <http://phrma.org/sites/default/files/pdf/The-Economic-Impact-of-the-US-Biopharmaceutical-Industry.pdf>. Accessed 7/23/12.
5. Celebration of Science. Available at [www.celebrationofscience.org/about](http://www.celebrationofscience.org/about). Accessed 9/10/12.
6. Hughes JW, Moore MJ and Snyder EA. "Napsterizing" Pharmaceuticals: Access, Innovation, and Consumer Welfare. Published 1/19/11. Available at <http://www.ftc.gov/os/comments/intelpropertycomments/snydermoorehughes.pdf>. Accessed 11/15/13.



**2** PROGRESS &  
PROSPERITY





# 3

## LIVING LONGER, BETTER AND HEALTHIER



RECENT MEDICAL INNOVATIONS IN CANCER ARE LEADING TO LONGER LIFE, BETTER HEALTH, MORE PRODUCTIVITY AND LOWER HEALTH CARE COSTS.

This section will show what has been accomplished in the last 20 years. We have made substantial progress in helping people become free from cancer, allowing them to live longer, healthier lives.

Innovative cancer treatments that produce longer and better lives also reduce the cost and complexity of cancer care. As with other diseases discussed, people treated for cancer can live and prosper as well as someone without the illness. Much of this progress has occurred in just the past five to 10 years.

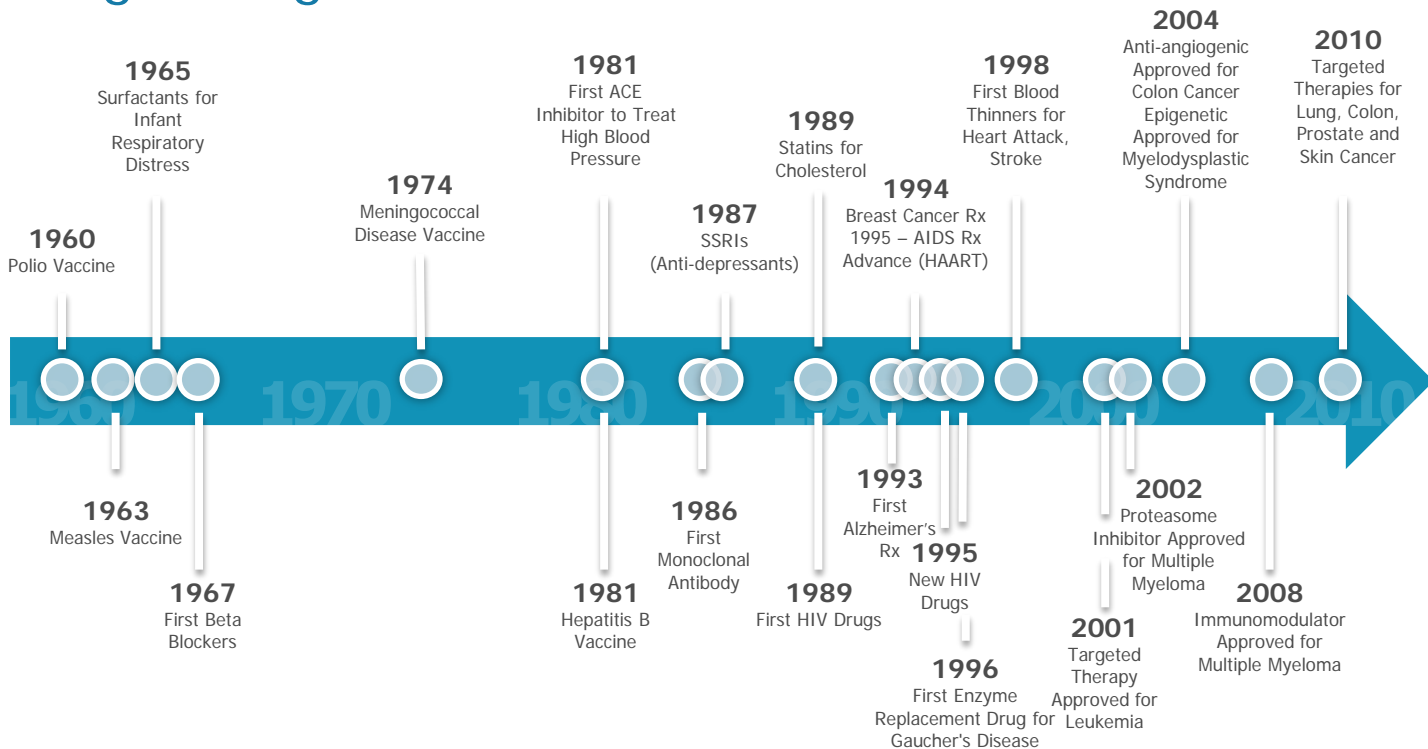


“

In 2005, a man diagnosed with **multiple myeloma** asked me if he would **be alive** to watch his daughter **graduate from high school** in a few months. In 2009, bound to a **wheelchair**, he watched his daughter graduate from college. The wheelchair had **nothing to do with his cancer**. The man had fallen down while coaching his youngest son's baseball team.”

*The Emperor of All Maladies* Author  
**Dr. Siddhartha Mukherjee**

# Progress Against Diseases 1960-2012



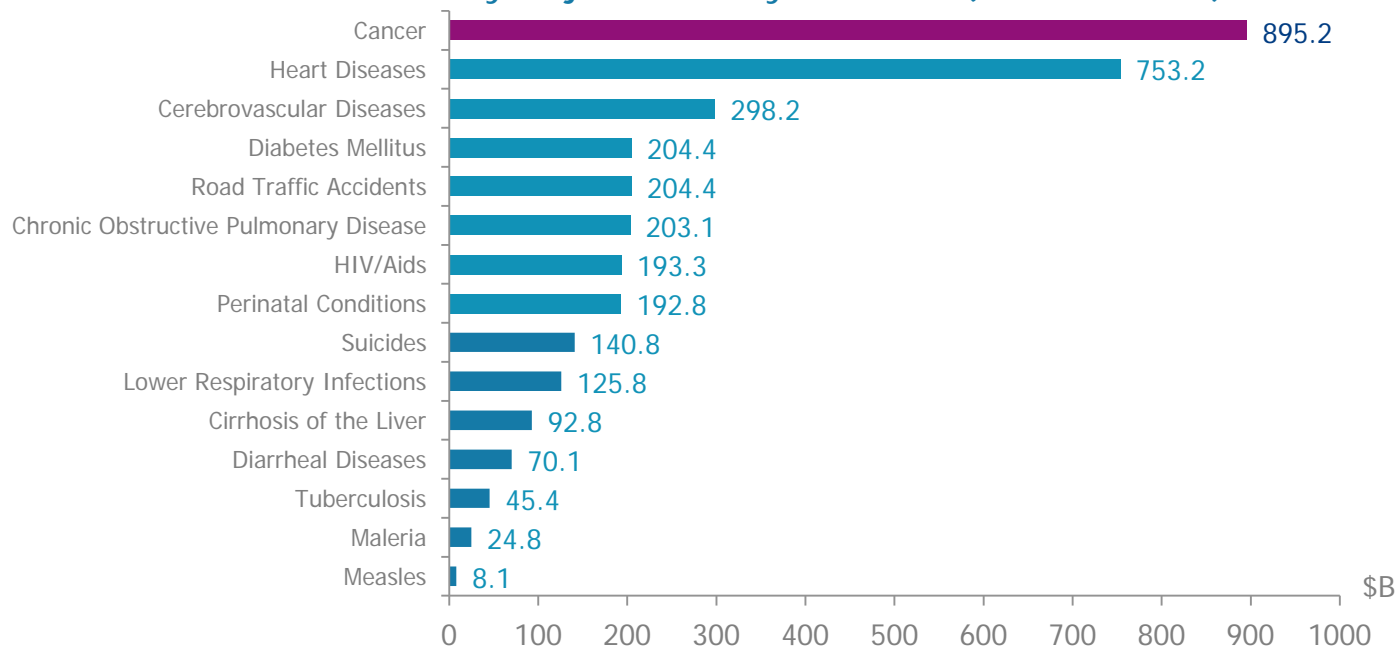
**Source 1:** American Society of Clinical Oncology's CancerProgress.net. Available at [www.cancerprogress.net](http://www.cancerprogress.net). Accessed 11/12/12. Müller DC, Ellen MA, Duffy MC, et al. Timeline: 200 Years of the New England Journal of Medicine. N Engl J Med 2012; 366:e 3January 5, 2012DOI: 10.1056/NEJMp1114819. Available at <http://www.nejm.org/doi/full/10.1056/NEJMp1114819>. Accessed 11/12/13. U.S. Food and Drug Administration. Hematology/Oncology (Cancer) Approvals & Safety Notifications. Available at <http://www.fda.gov/drugs/informationondrugs/approveddrugs/ucm279174.htm>. Accessed 11/12/13.



# The Worldwide Cost of Cancer



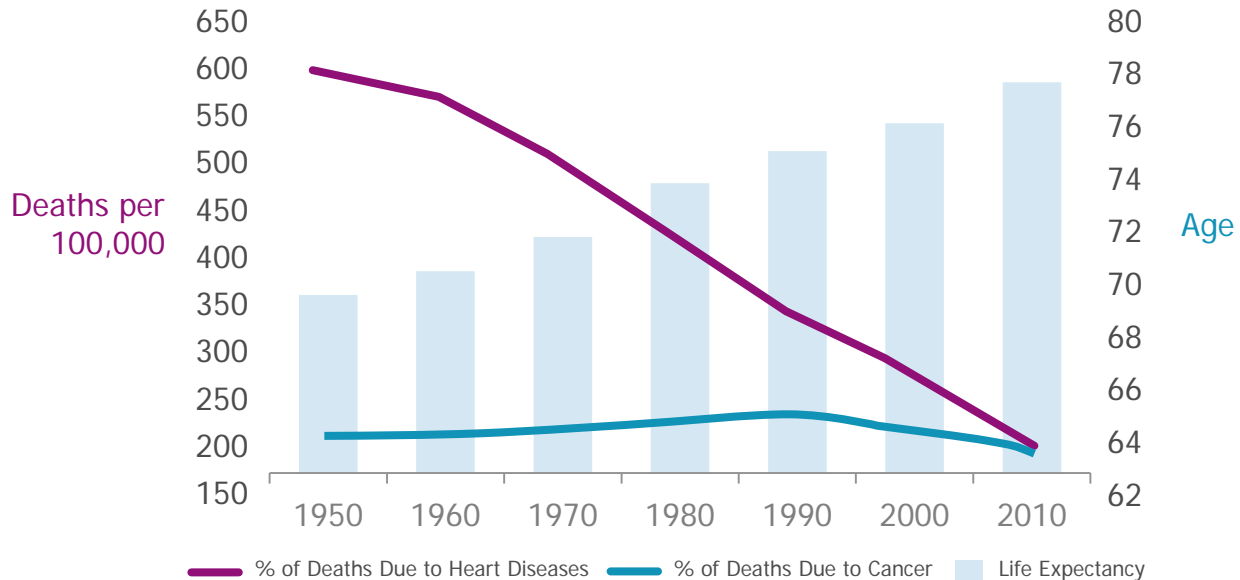
## Economic value of disability-adjusted life-years lost (U.S. \$ billion) in 2008



**Source 2:** Soerjomataram I, et al Global burden of cancer in 2008: a systematic analysis of disability-adjusted life-years in 12 world regions. Lancet 2012; 10.1016/S0140-6736(12)60919-2.

**3** LIVING LONGER,  
BETTER & HEALTHIER

## Increased Life Expectancy Driven by Medical Progress, Including in Heart Disease and Cancer

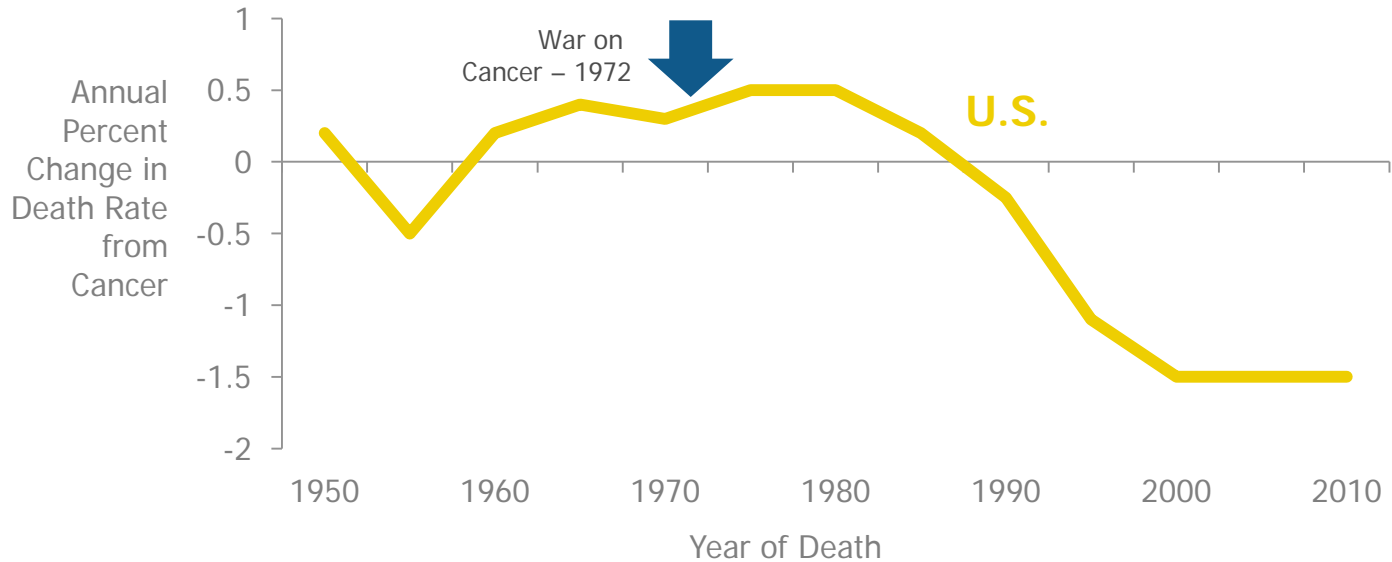


Source 3: Centers for Disease Control and Prevention's National Vital Statistics System. Available at [www.cdc.gov/nchs/nvss.htm](http://www.cdc.gov/nchs/nvss.htm). Accessed 11/15/13.  
 40 Centers for Disease Control and Prevention and National Center for Health Statistics. Health, United States, 2011: With Special Feature on Socioeconomic Status and Health. Available at <http://www.cdc.gov/nchs/index.htm>. Accessed 11/12/13.





# Cancer Innovation Tells the Story

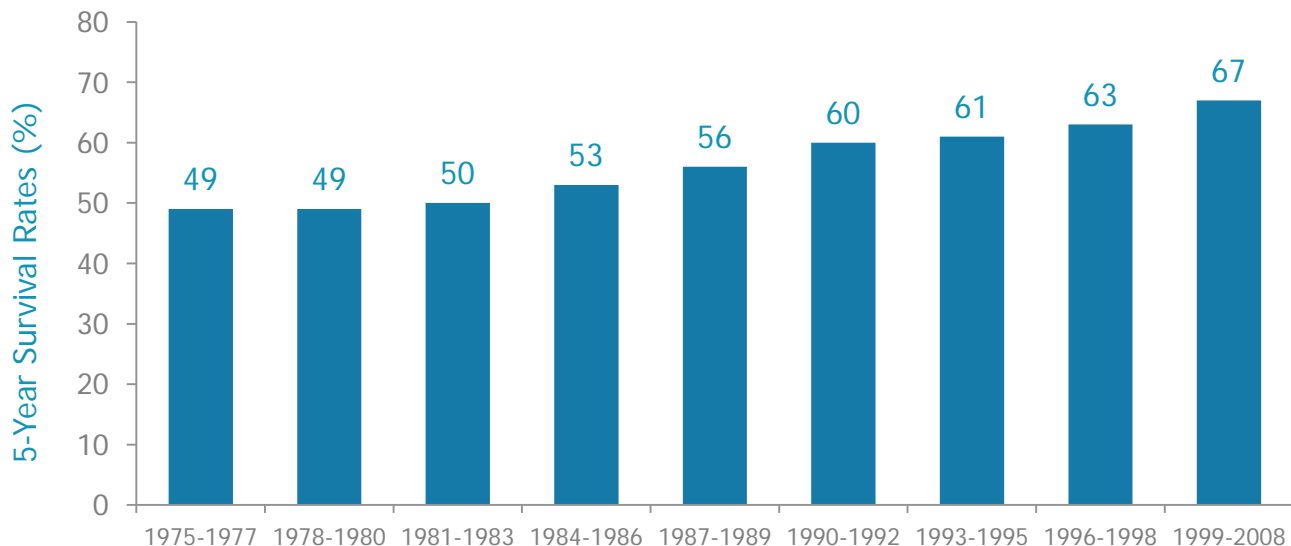


Death Rate from Cancer Has Been on the Decline for Over 30 Years

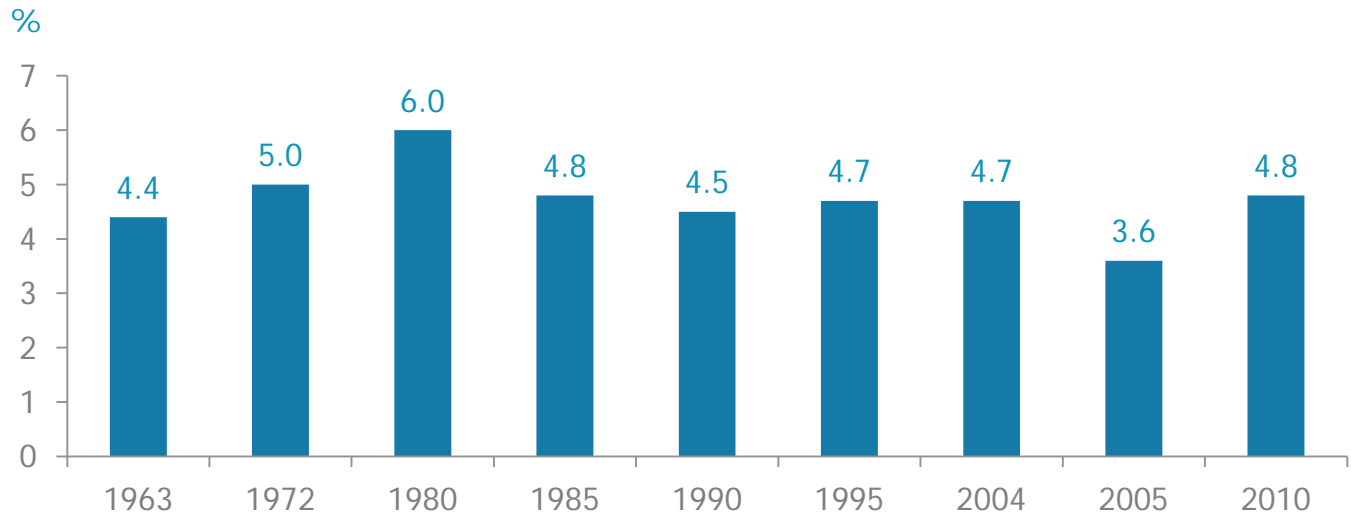
**Source 4:** Kort EJ, Paneth N, Vande Woude GF. *Cancer Res.* 2009;69:6500-6505. National Cancer Institute, Surveillance Epidemiology and End Results (SEER). Available at [http://seer.cancer.gov/csr/1975\\_2009\\_pops09/browse\\_csr.php?section=18&page=sect\\_18\\_table09.html](http://seer.cancer.gov/csr/1975_2009_pops09/browse_csr.php?section=18&page=sect_18_table09.html). Accessed 7/25/13.

**3** LIVING LONGER,  
BETTER & HEALTHIER

## Five-Year Survival Rates for People with Cancer Continue to Climb

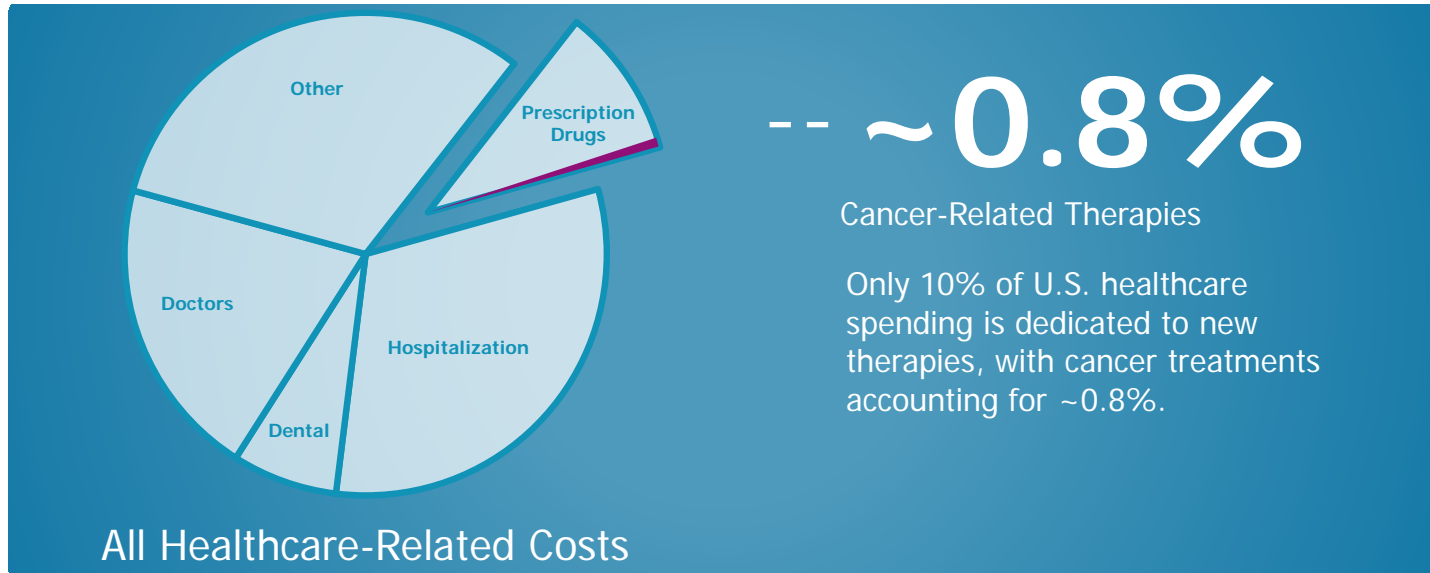


# Cost of Treating Cancer Remains Small Portion of Total Health Care Expenditures



**Source 6:** U.S. Department of Health and Human Services. Centers for Medicare and Medicaid Services. Available at <http://www.cms.gov/>. Accessed 11/14/13. Mariotto AB, Yabroff KR, Shao Y, et al. Projections of the Cost of Cancer Care in the United States: 2010–2020. *J Natl Cancer Inst.* 2011. 103 (2): 117.

## New Cancer Therapies: Small Cost, Large Impact on Society



-- ~0.8%  
Cancer-Related Therapies

Only 10% of U.S. healthcare spending is dedicated to new therapies, with cancer treatments accounting for ~0.8%.

Source 7: Centers for Medicare and Medicaid Services. National Health Expenditure Data. Available at <https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/Downloads/Proj2011PDF.pdf>. 2011. Accessed 11/15/13. Statista. Statistics and facts about cancer in the U.S. Available at <http://www.statista.com/topics/1192/cancer-in-the-us/>. Accessed 11/15/13.

# Living Longer, Better and Healthier Benefits Society



**REDUCTION**  
in **CANCER**-related  
**DEATHS** in the U.S.

Improved  
quality of life

Stimulates the  
economy\*

**\$500 Billion**  
**In Societal Value**

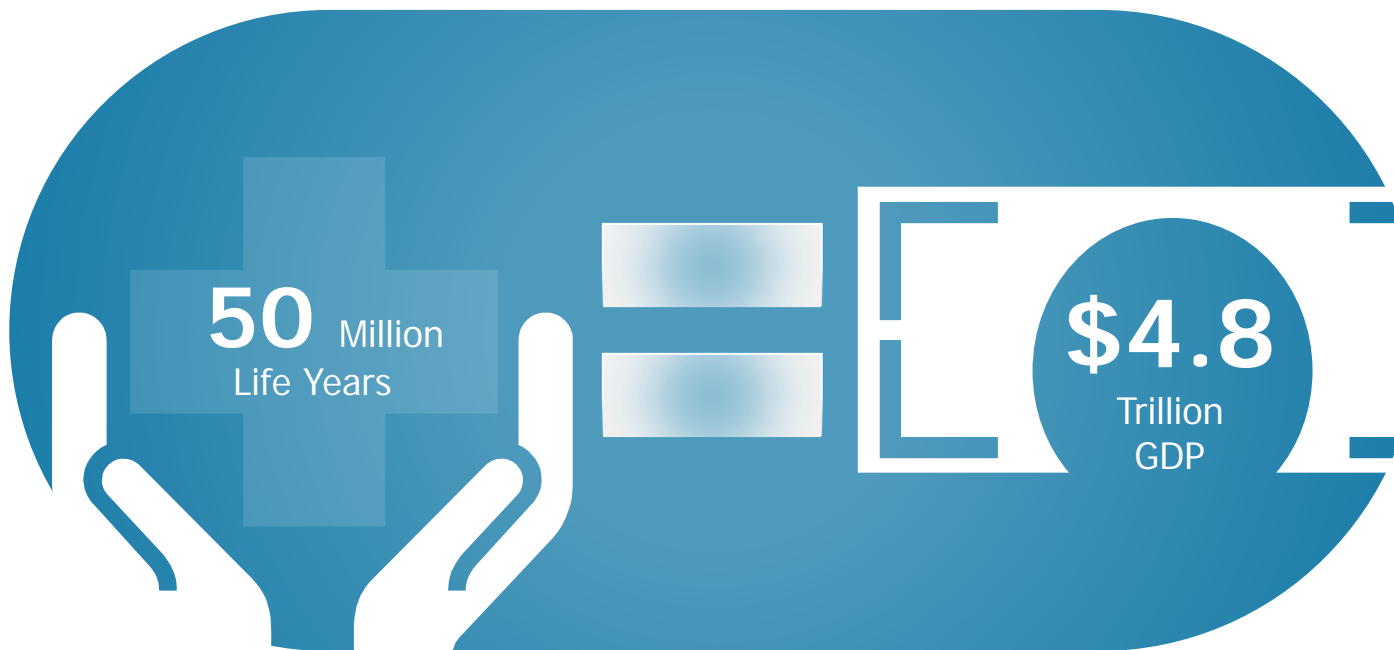
Maximized life  
expectancy

\*Extended survival contributes to economic stimulus by affording people more time to purchase and enjoy leisure activities

Source 8: Murphy KM and Topel RH. The Value of Health and Longevity. *J Political Econ* 2006: 114; 5; 871-904.

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BETTER & HEALTHIER

## Estimated Value of Additional Life Years Gained from Innovative Cancer Treatments 1990-2013



**Source 9:** Estimate derived from the National Bureau of Economic Research. Sun EC, Jena AB, Lakdawalla DN, et al. An Economic Evaluation of the War on Cancer. NBER Working Paper No. 15574. Issued in December 2009. Available at <http://www.nber.org/papers/w15574>. Accessed 11/16/12. Updated 7/22/13. Methodology discussed at <http://valueofinnovation.org/about-the-clock.html> and <http://imgsrv.wben.com/image/wben2/UserFiles/File/Philipson%20FF.pdf>.

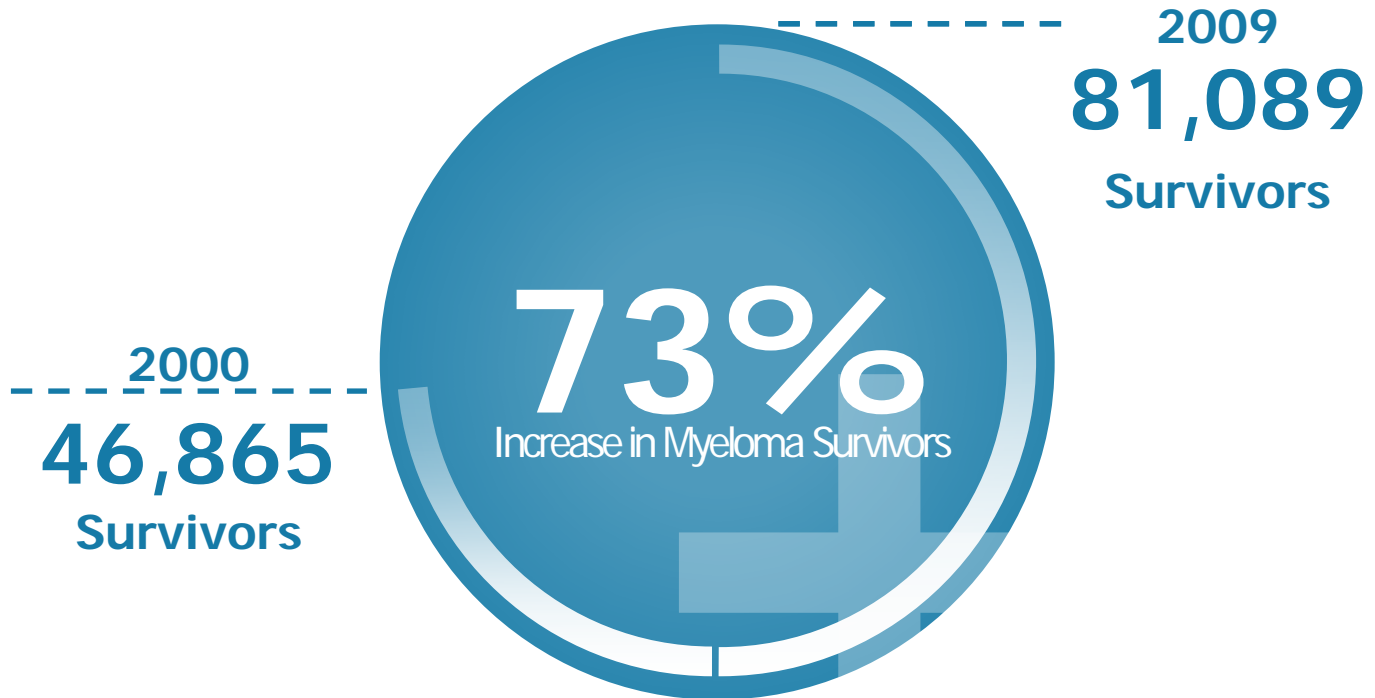


“ I planted a **new rose bush** every time I **underwent a treatment**. Now, I've got a **regular garden**. The bees love them. So do I. Reminds me of **all I've gone through to get here.** ”

– Multiple Myeloma Patient

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## Myeloma Survivorship Rapidly Increasing





# Advances Made in Myeloma Point to a Brighter Future



## Dr. Brian Durie

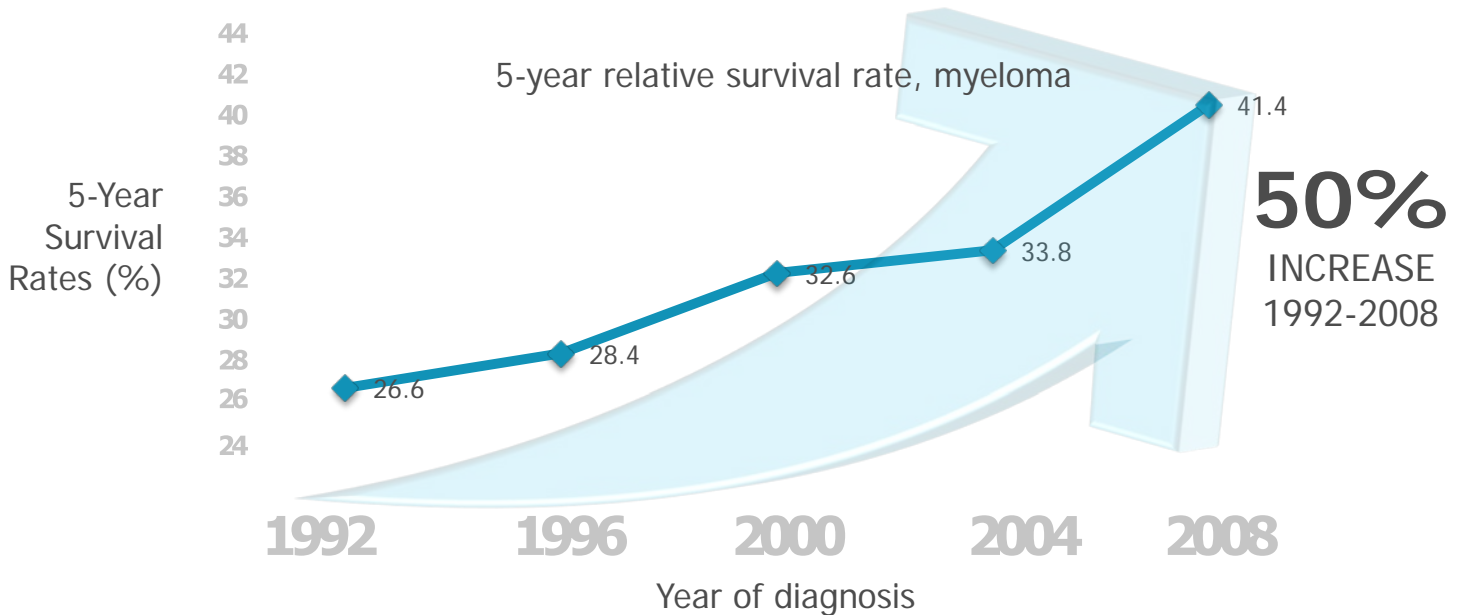
International Myeloma Foundation Chair,  
Professor of Medicine, Hematologist/Oncologist,  
Cedars-Sinai Outpatient Cancer Center,  
Los Angeles

*“With the novel therapies we're seeing a quantum leap in two-year survival from 50% to now 93%, which is just 3% short of what a healthy person of a comparable age could expect.”*



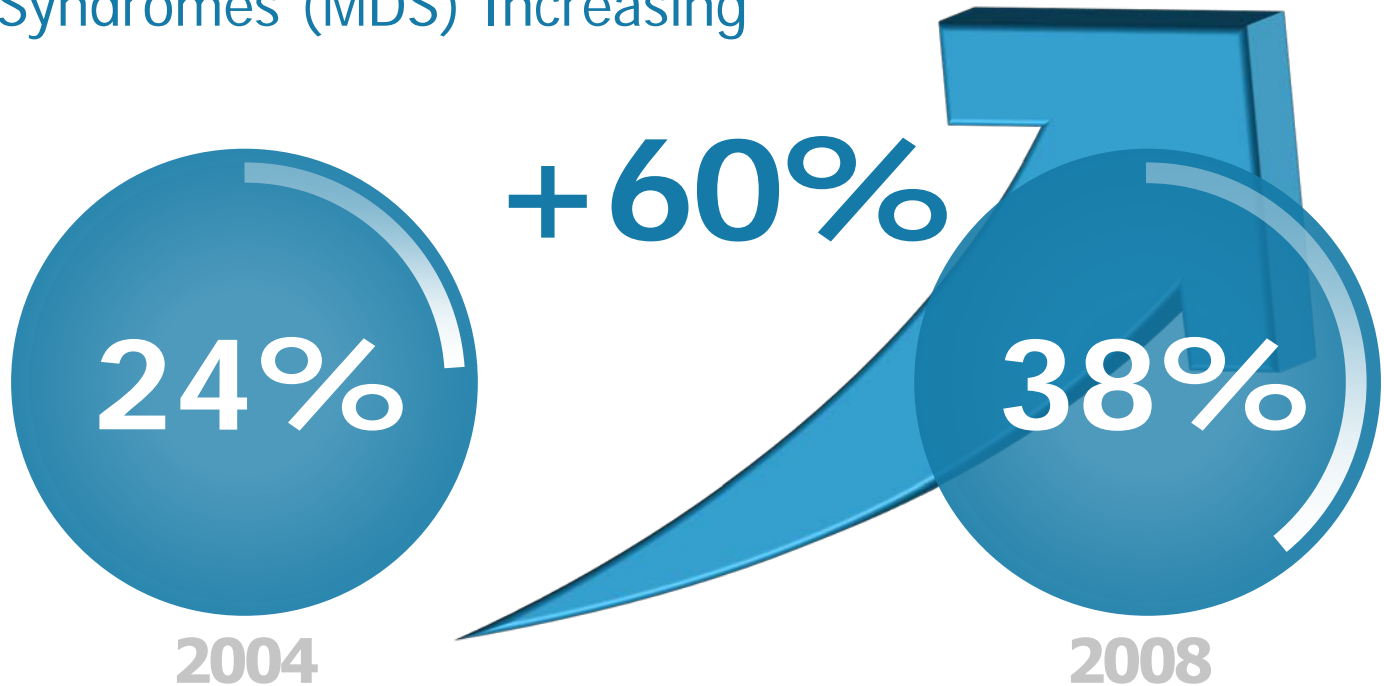
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## Survival Rate for Myeloma Patients Soar with New Innovative Therapies



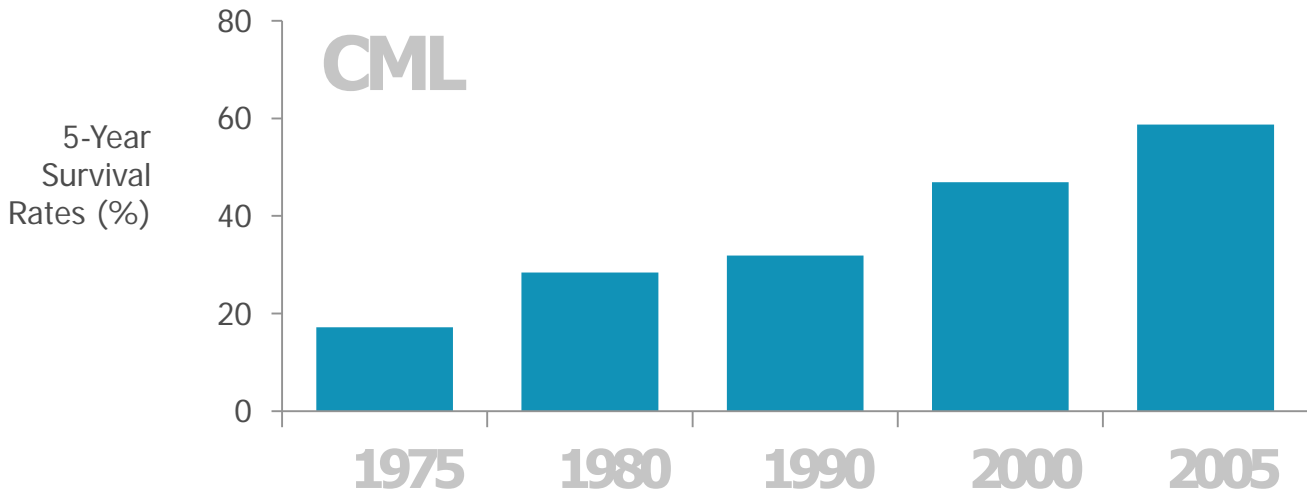
**Source 11:** National Cancer Institute. Surveillance Epidemiology and End Results (SEER). SEER 18 Regs Research Data + Hurricane Katrina Impacted Louisiana Cases, Nov 2011 Sub, Vintage 2009 Pops (2000-2009) <Katrina/Rita Population Adjustment. Analysis based on seer.cancer.gov/seerstat. Version 8.1. Accessed 10/31/13.

# Five-Year Survival from Myelodysplastic Syndromes (MDS) Increasing



Source 12: Smout R, Horn S, Goldberg R. Age Period Cohort Analysis of Cancer Survival In SEER 18 Registry. Center for Medicine in the Public Interest.

## Chronic Myeloid Leukemia (CML) Patients Benefiting from Medical Innovation

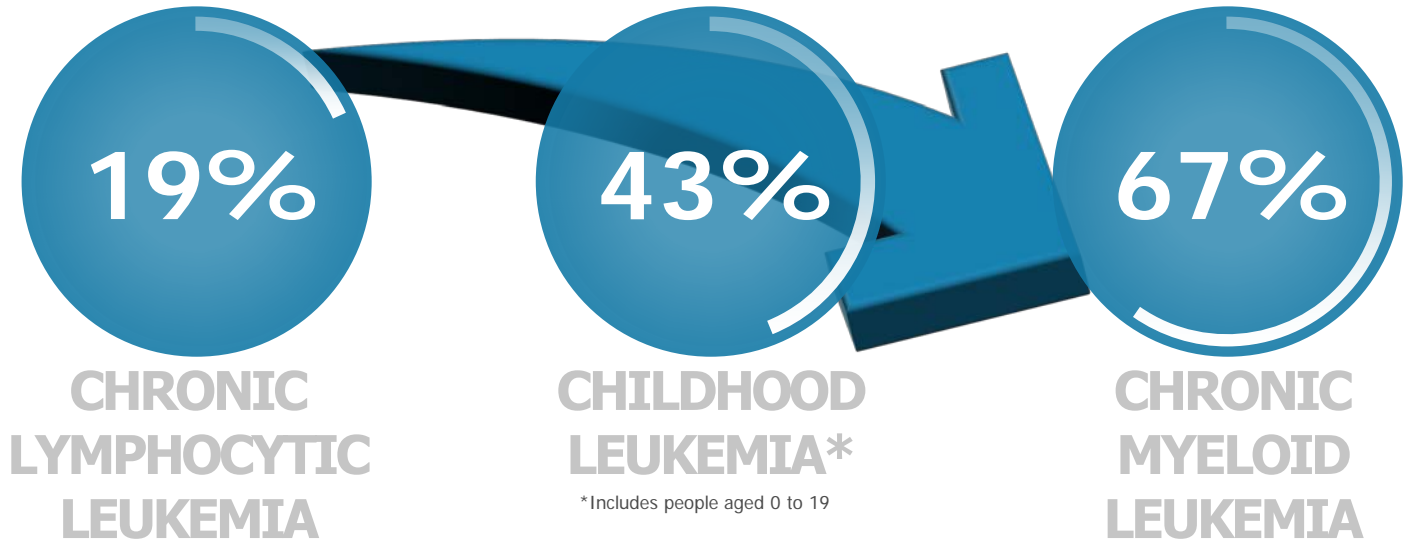


Source 13: SCB Analysis, National Cancer Institute, Surveillance Epidemiology and End Results (SEER). Available at <http://seer.cancer.gov/faststats/selections.php?> Accessed 7/31/13.

# Significant Strides Made in Reducing Mortality Rates Among Hard-to-Treat Blood Cancers



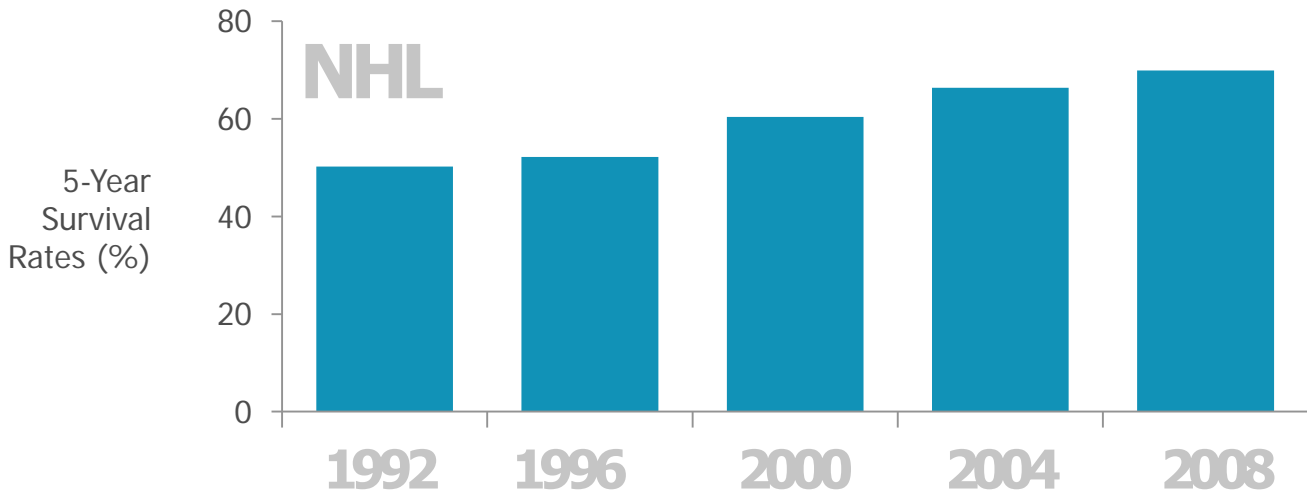
MORTALITY RATE PER 100,000 PEOPLE FROM 1992-2010



Source 13: National Cancer Institute, Surveillance Epidemiology and End Results (SEER). Available at <http://seer.cancer.gov/>. Accessed 7/31/13.

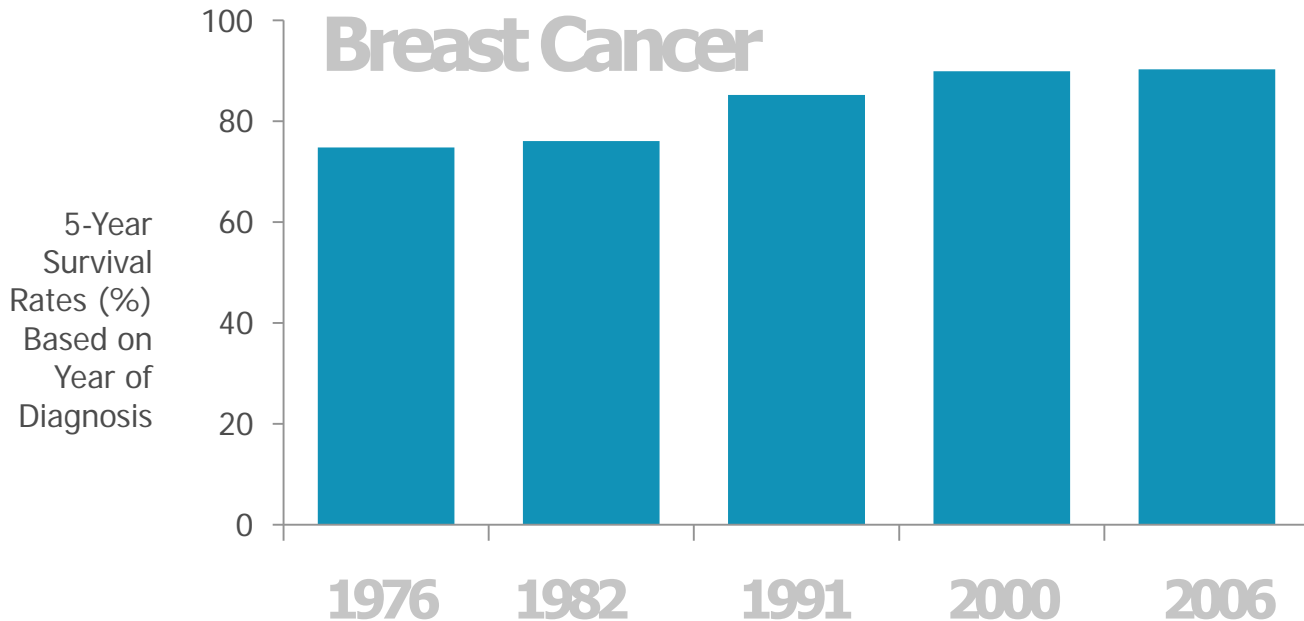
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## Improving Non-Hodgkin Lymphoma (NHL) Diagnosis and Survival



Source 13: SCB Analysis, National Cancer Institute, Surveillance Epidemiology and End Results (SEER). Available at <http://seer.cancer.gov/faststats/>.

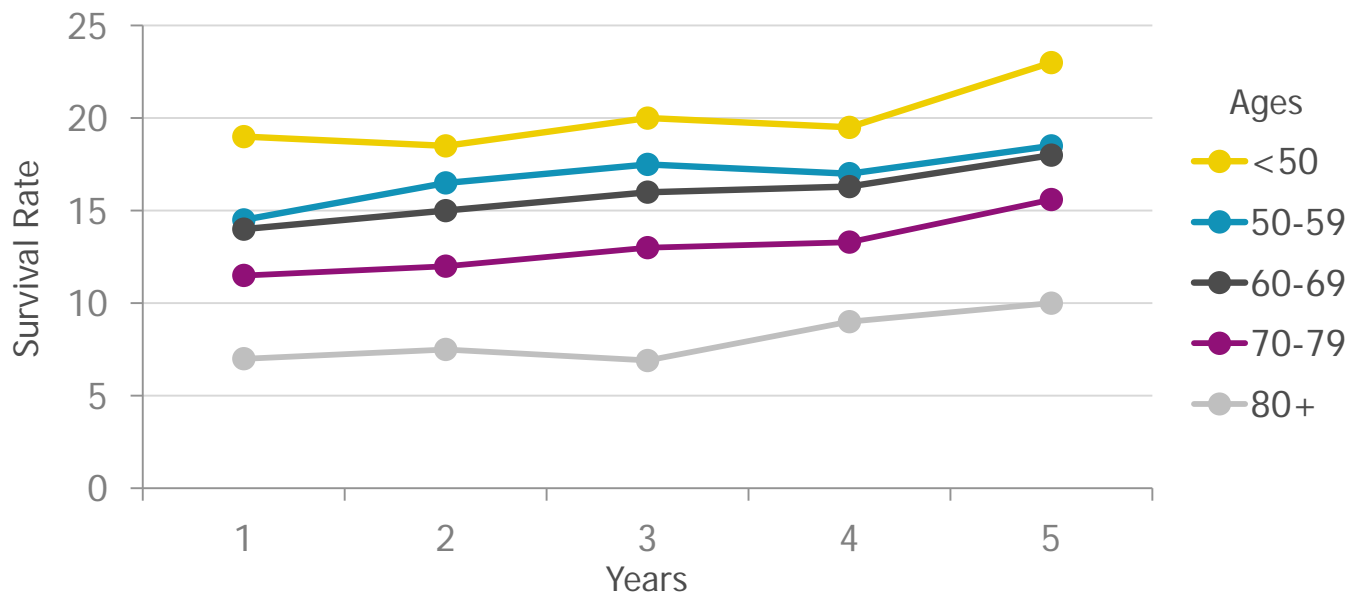
# Advances Continue in Breast Cancer



**Source 13:** SCB Analysis, National Cancer Institute, Surveillance Epidemiology and End Results (SEER). Available at <http://seer.cancer.gov/faststats/>. Accessed 7/31/13.

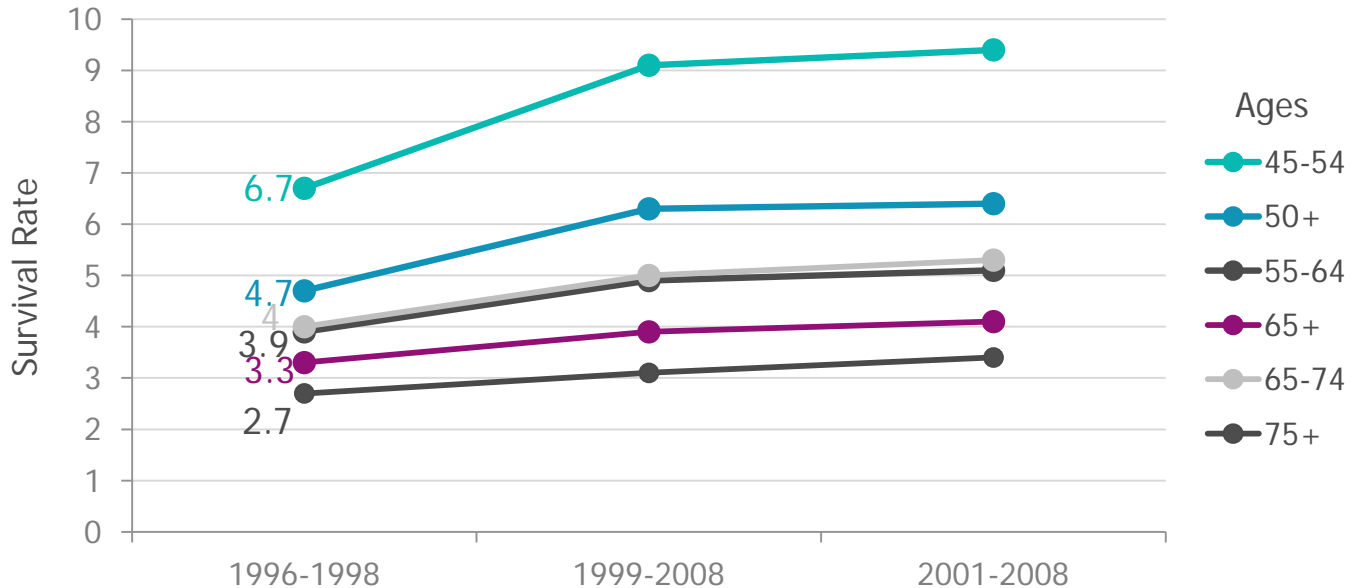
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## Lung Cancer 5-Year Survival Rate on the Rise





# Hopeful Signs in Extending 5-Year Survival Among Patients with Pancreatic Cancer



Source 12: Smout R, Horn S, Goldberg R. Age Period Cohort Analysis of Cancer Survival In SEER 18 Registry. Center for Medicine in the Public Interest.

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8. Murphy KM and Topel RH. The Value of Health and Longevity. J Political Econ 2006; 114; 5: 871-904.
9. Estimate derived from the National Bureau of Economic Research. Sun EC, Jena AB, Lakdawalla DN, et al. An Economic Evaluation of the War on Cancer. NBER Working Paper No. 15574. Issued in December 2009. Available at <http://www.nber.org/papers/w15574>. Accessed 11/16/12. Updated 7/22/13. Methodology discussed at <http://valueofinnovation.org/about-the-clock.html> and <http://imgsrv.wben.com/image/wben2/UserFiles/File/Philipson%20FF.pdf>.
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12. Smout R, Horn S, Goldberg R, Age Period Cohort Analysis of Cancer Survival In SEER 18 Registry. Center for Medicine in the Public Interest.
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# 4

## BETTER HEALTHCARE, BETTER OUTCOMES



CONTINUED MEDICAL INNOVATION CAN LEAD TO A FUTURE FREE FROM CANCER AS WE KNOW IT TODAY.

As with HIV, medical innovation is transforming cancer from a leading cause of death to a long-term, manageable disease.

This section will describe how medical innovation that can change the treatment paradigm for cancer care will be personalized. There will be no one type of cancer and no single treatment. Increasingly, cancer will be managed, prevented or cured by therapies that detect the disease before it spreads, and stops it as quickly as possible – without chemotherapy or surgery.

We will show how such innovations will replace expensive and inconvenient cancer modalities, just as the polio vaccine and HIV treatments replaced iron lungs and expensive inpatient care. Cancer care in the future could be free of toxic chemotherapy, surgery and transfusions.



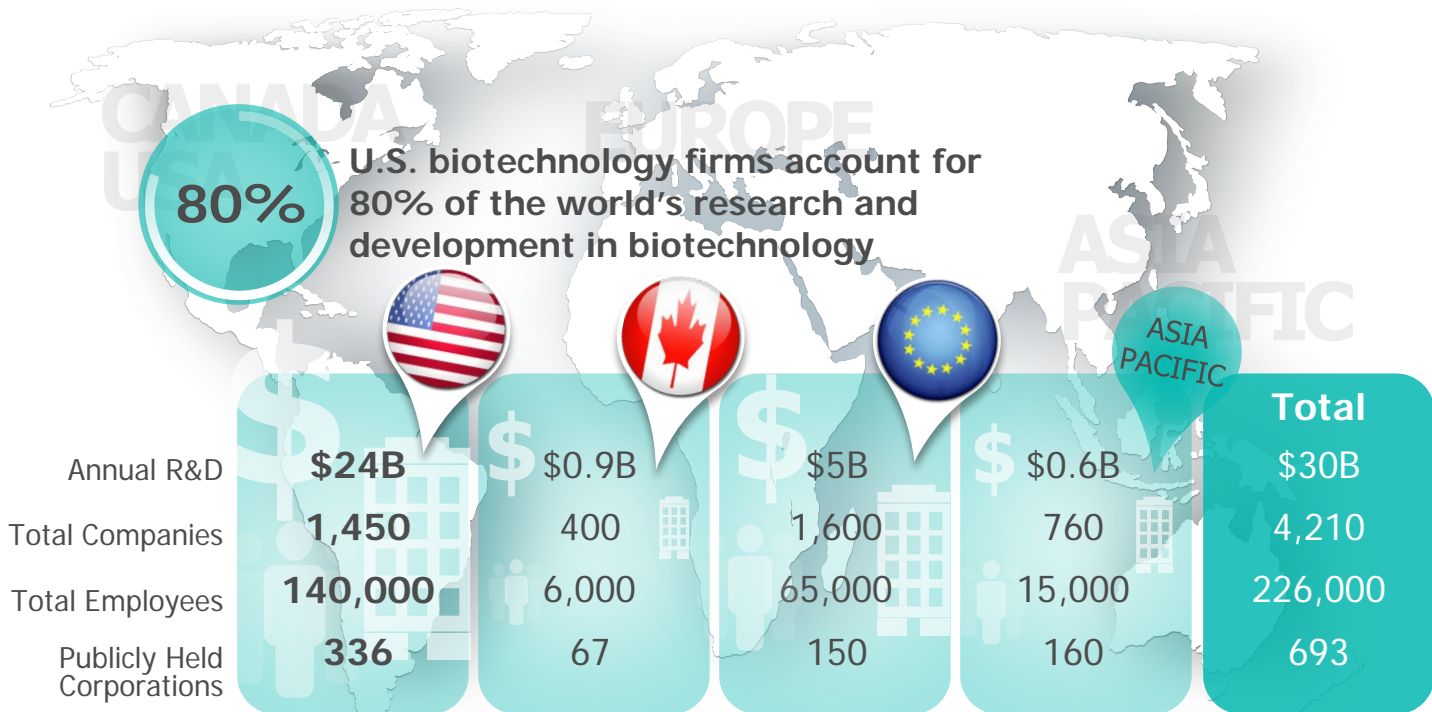


“The **pharmaceutical industry** is one of the **most research-intensive** industries in the United States. Pharmaceutical firms invest as much as **five times more** in research and development, relative to their sales, than the **average U.S. manufacturing firms.**”

October 2006

**Congressional Budget Office**

# U.S. Sets Pace with Bold Pursuits in Science



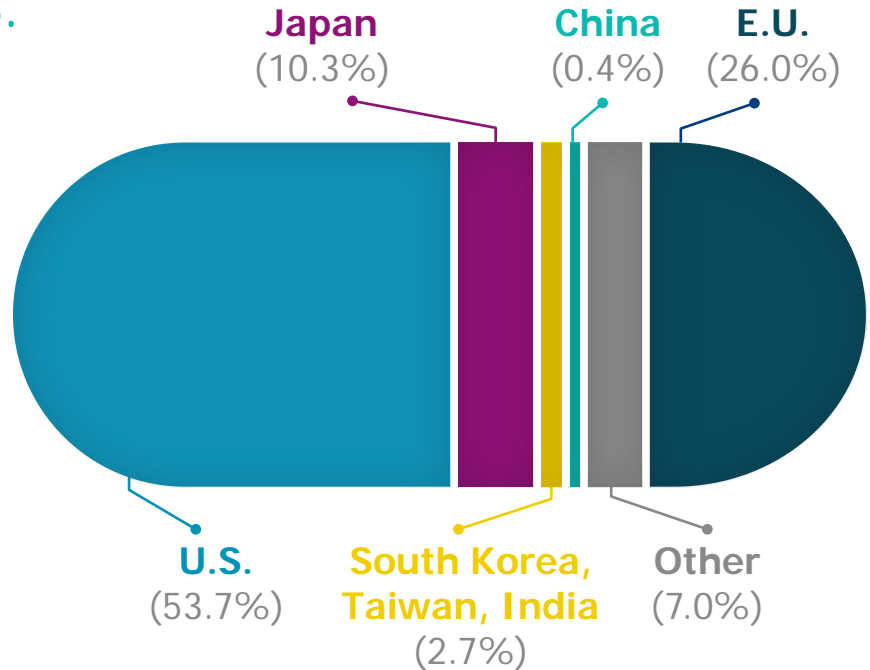
Biotechnology companies are defined as those whose primary activity is to use biological processes to develop health care products, and other companies whose primary activity is to supply health biotechnology companies with technology-based research products.



# More than ½ of All Biopharmaceutical Innovation Originates in the U.S.

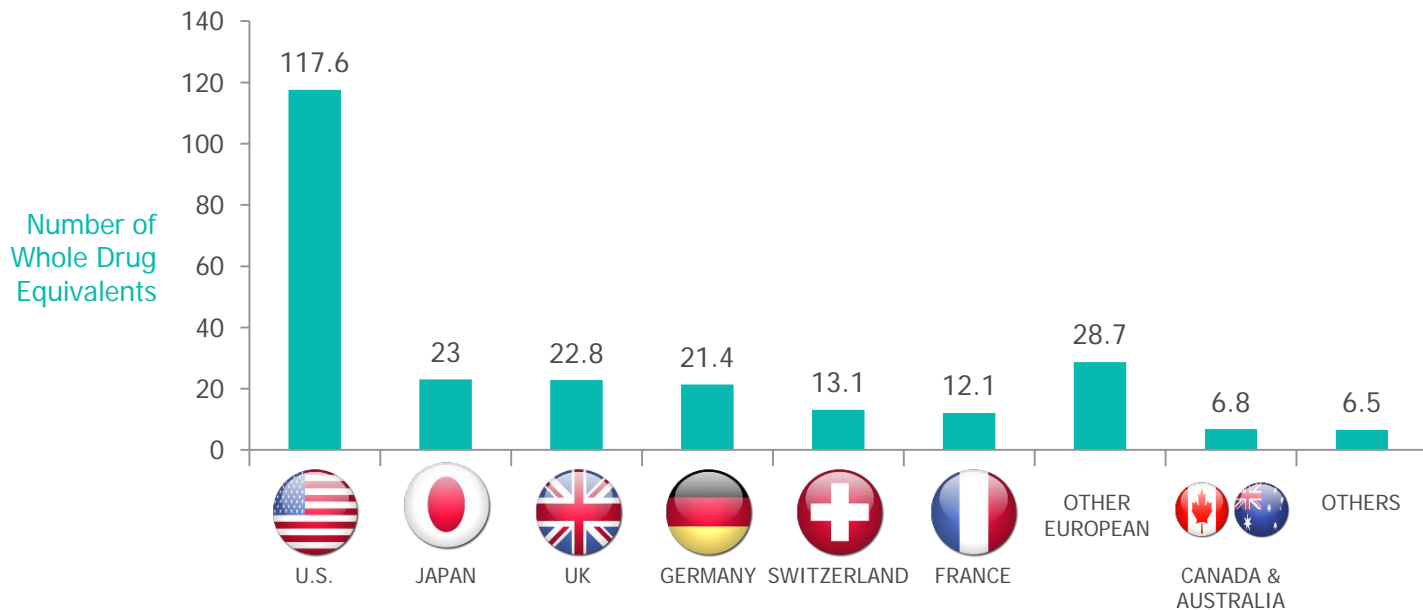


The U.S. produces more than ½ of all new innovative therapies and biotech patents in the world



**Source 2:** Macher JT and Mowry DC. Innovation in Global Industries: U.S. Firms Competing in a New World (Collected Studies), Washington DC: The National Academies Press, 2008. PhRMA analysis based on National Science Board. "Science and Engineering Indicators 2012." Arlington, VA: National Science Foundation (NSB12-01), 2012.

# U.S. Leadership in Sustaining Cancer Innovation



Allocation of the 252 new therapies approved by the U.S. Food and Drug Administration between 1998 and 2007 among drug-discovering countries. The numbers represent whole drug equivalents.

**Source 3:** Kneller R. The importance of new companies for drug discovery: origins of a decade of new drugs. *Nature Reviews Drug Discovery*. 2010; 9, 867-882. Available at <http://www.nature.com/nrd/journal/v9/n11/full/nrd3251.html>. Accessed 10/31/13.

# Maximizing the Promise of Science: 5,000+ Medicines in Development in 2011

# 3,436

## Cancer

- 142 Lung Cancer
- 95 Breast Cancer
- 383 Blood Cancers
- 63 Colorectal Cancer
- 105 Skin Cancer

## 1,795 Rare Diseases

## 1,586 Infectious Diseases

## 1,247 Neurological Disorders

## 650 Cardiovascular Disorders

## 731 Immunological Conditions

## 412 Diabetes Mellitus

## 204 HIV/AIDS and Related Conditions

## 454 Musculoskeletal Diseases

## 69 Liver Disease and Related Conditions

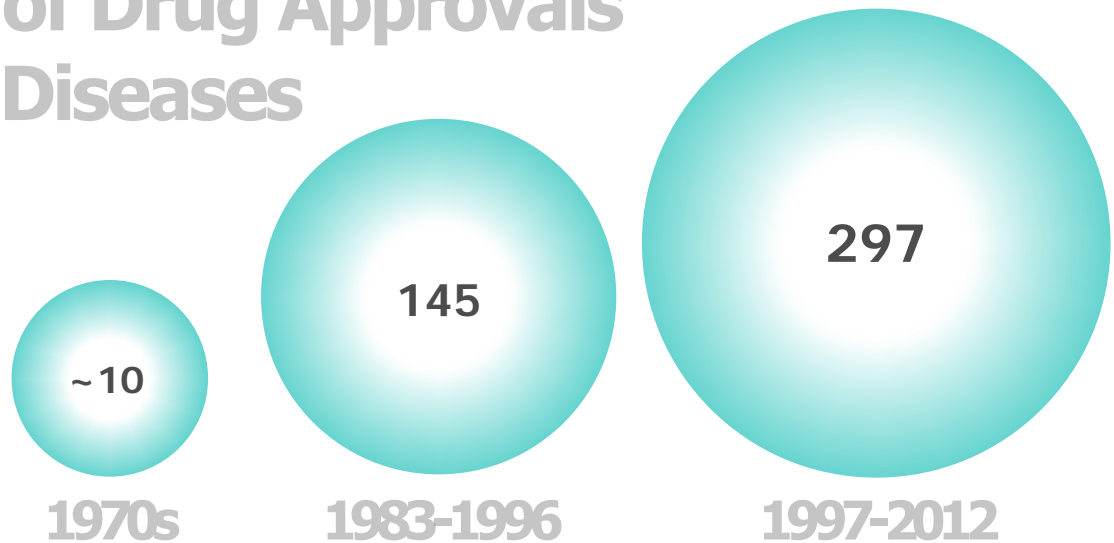
**Source 4:** Innovation in the Biopharmaceutical Pipeline: A Multi-Dimensional View. Available at <http://www.phrma.org/sites/default/files/pdf/2013innovationinthebiopharmaceuticalpipeline-analysisgroupfinal.pdf>. Accessed 11/12/13.

Reflects compounds in all phases of development, including having been filed with the FDA, or approved by the FDA, but not yet on the market in the U.S. as of January 2013. Medicines with multiple indications may appear in more than one category, but in the total number (5,000+ medicines), only the initial indication is counted.

**4** BETTER HEALTHCARE,  
BETTER OUTCOMES

## U.S. Orphan Drug Act (1984) Impact on Innovation

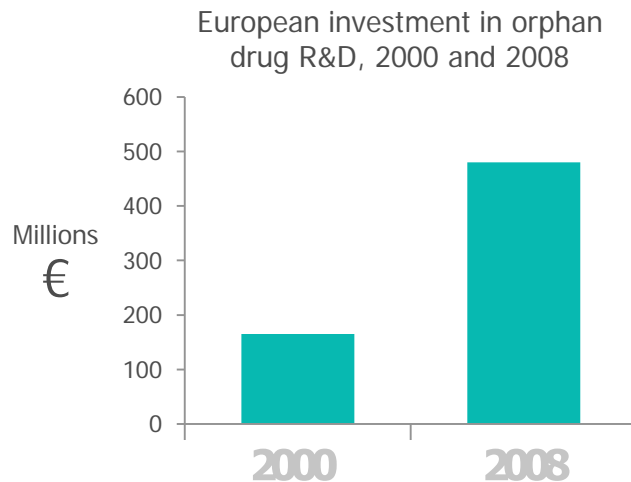
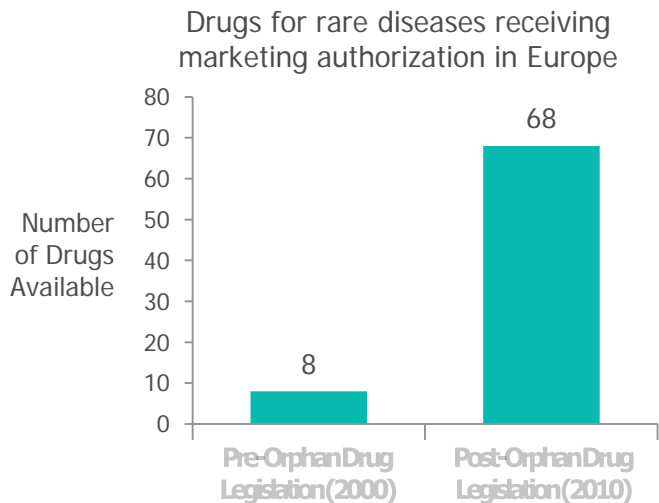
### Number of Drug Approvals for Rare Diseases



Rare diseases are those that affect **200,000 or fewer** people in the U.S.  
There are between **6,000-7,000 rare diseases** affecting 25 million Americans.

**Source 5:** U.S. Food and Drug Administration, Office of Orphan Product Development, Orphan Drug Designations and Approvals Database, 1983-2012. Available at <http://www.accessdata.fda.gov/scripts/opdlisting/oopd/index.cfm>. U.S. Food and Drug Administration. Developing Products for Rare Diseases & Conditions, 1970s. Available at <http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/default.htm>. Accessed 7/17/12.

# European Commission's Orphan Drug Regulation



The Orphan Drug Regulation (EC) 141/2000, together with national incentives, have contributed to the discovery and development of much needed treatments. Orphan drug expenditures are expected to account for less than 5% of total European pharmaceutical expenditures by 2020, confirming both the affordability of orphan drugs and the sustainability of this new model for health care systems.

**Source 6:** Office of Health Economics. Assessment of the Impact of OMPs on the European Economy and Society. Consulting Report November 2010. Available at <http://www.ohe.org/publications/article/assessment-of-the-impact-of-orphan-medicinal-products-on-europe-15.cfm>. Accessed 12/13/12.

# Biopharmaceutical Sector Invests Ten Times More Per Employee on R&D than All U.S. Manufacturing

Total Global R&D Spend,  
Biopharmaceutical Sector (2006)

\$56.1 Billion

Total U.S. R&D Spend,  
Biopharmaceutical Sector (2006)

\$44.9 Billion

Average U.S. R&D Spend per Employee,  
Biopharmaceutical Sector (2006)

\$65,381

Average U.S. R&D Spend per Employee,  
All Manufacturing Sectors (2000-2004)

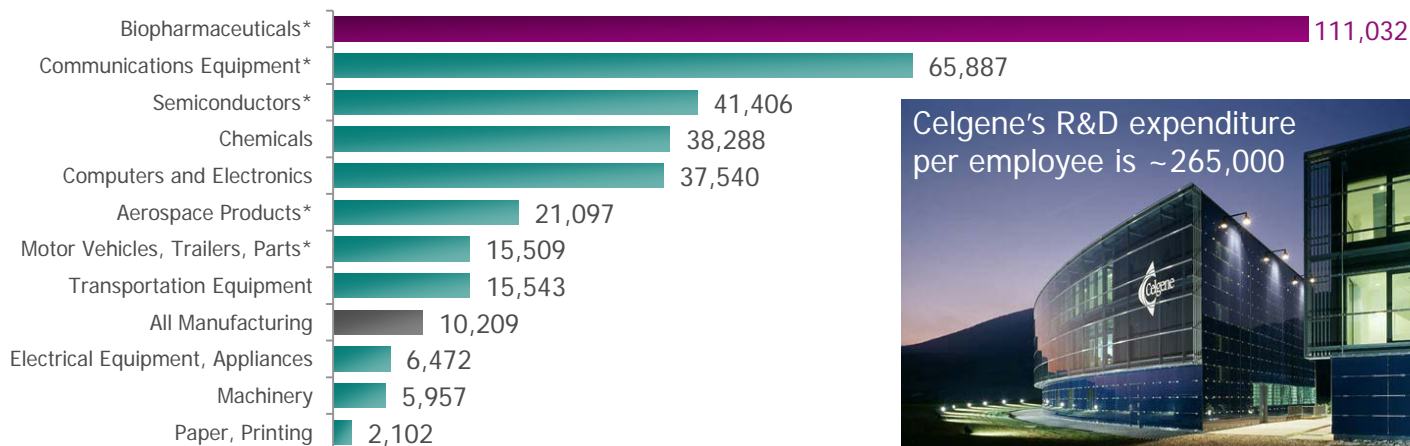
\$7,634

# The Biopharmaceutical Sector is the Most R&D-Intensive in the U.S.



Biopharmaceutical companies invested more than 10 times the amount of R&D per employee than manufacturing industries overall

R&D Expenditures per Employee, by Manufacturing Sub-sector and Industry, 2000-2008



\*Asterisks indicate manufacturing subsectors

**Source 8:** PhRMA analysis based on National Science Board. "Science and Engineering Indicators 2012." Arlington, VA: National Science Foundation (NSB12-01), 2012. R.A. Woods. "Employment Outlook: 2008-2018. Industry Output and Employment Projections to 2018." Monthly Labor Review 2009; Washington, DC: Bureau of Labor Statistics.

Celgene's R&D expenditure per employee is ~265,000



4 BETTER HEALTHCARE,  
BETTER OUTCOMES

# Celgene: Delivering on Its Promise of Bringing New Treatments to Patients in Need

Celgene's already developed innovative therapies are bringing tremendous benefits to providers, patients and healthcare systems worldwide:

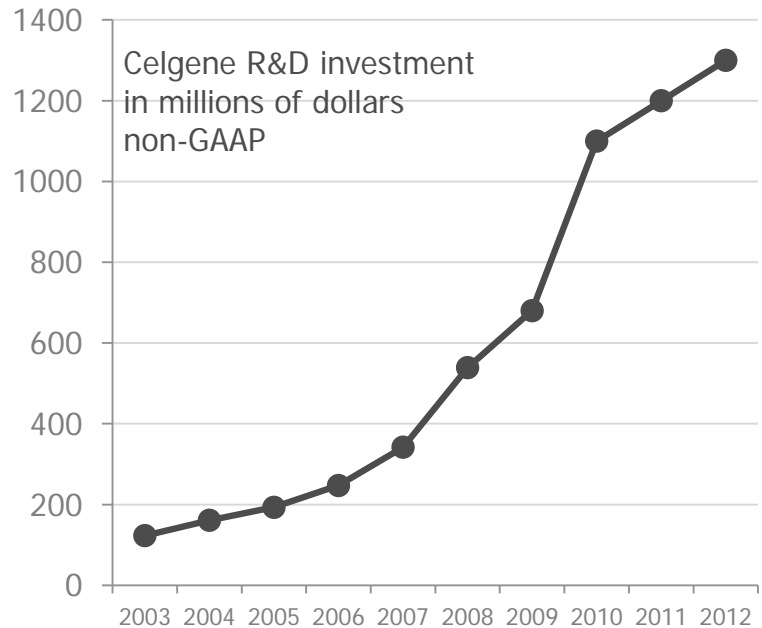
Reducing hospitalizations

Preventing disability

Eliminating surgeries

Improving quality of life

Extending survival





# Sustaining a Deep and Diverse Pipeline of Paradigm-Changing Innovation

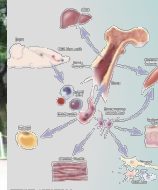


**Drug Discovery  
Alliance Development**



**Epigenetics/Signaling**  
San Diego, CA

**Cellular Therapeutics**  
Warren, NJ



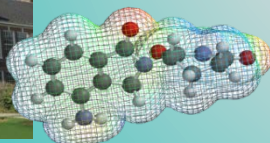
**Translational Development**  
San Francisco, CA



**Nonclinical & Early  
Clinical Development**



**IMiDs®**  
Summit, NJ



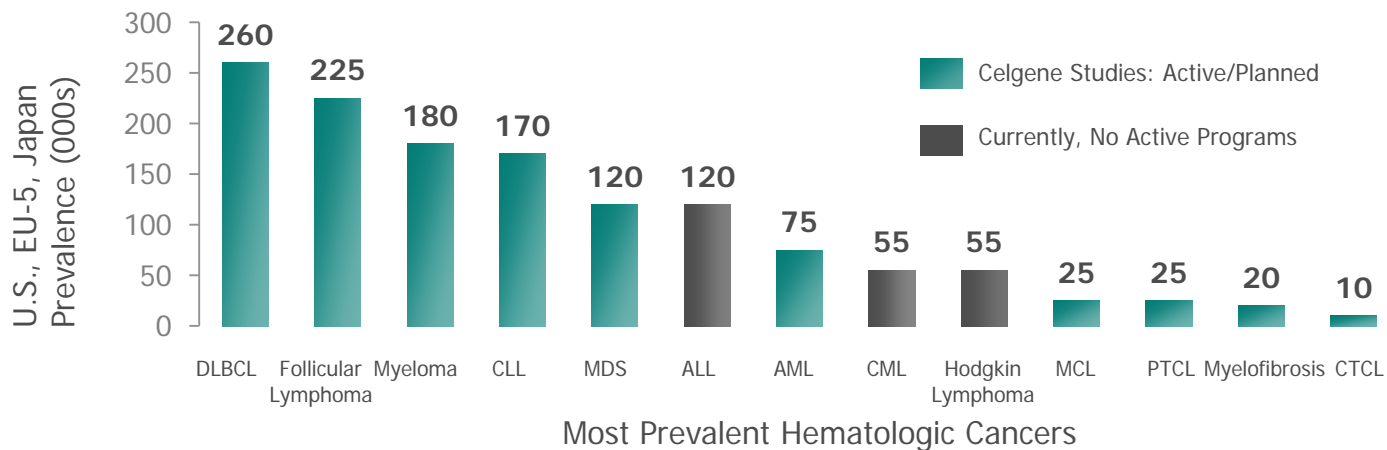
**Avilomics™  
Research**  
Bedford, MA



**CITRE**  
Seville, Spain

**4** BETTER HEALTHCARE,  
BETTER OUTCOMES

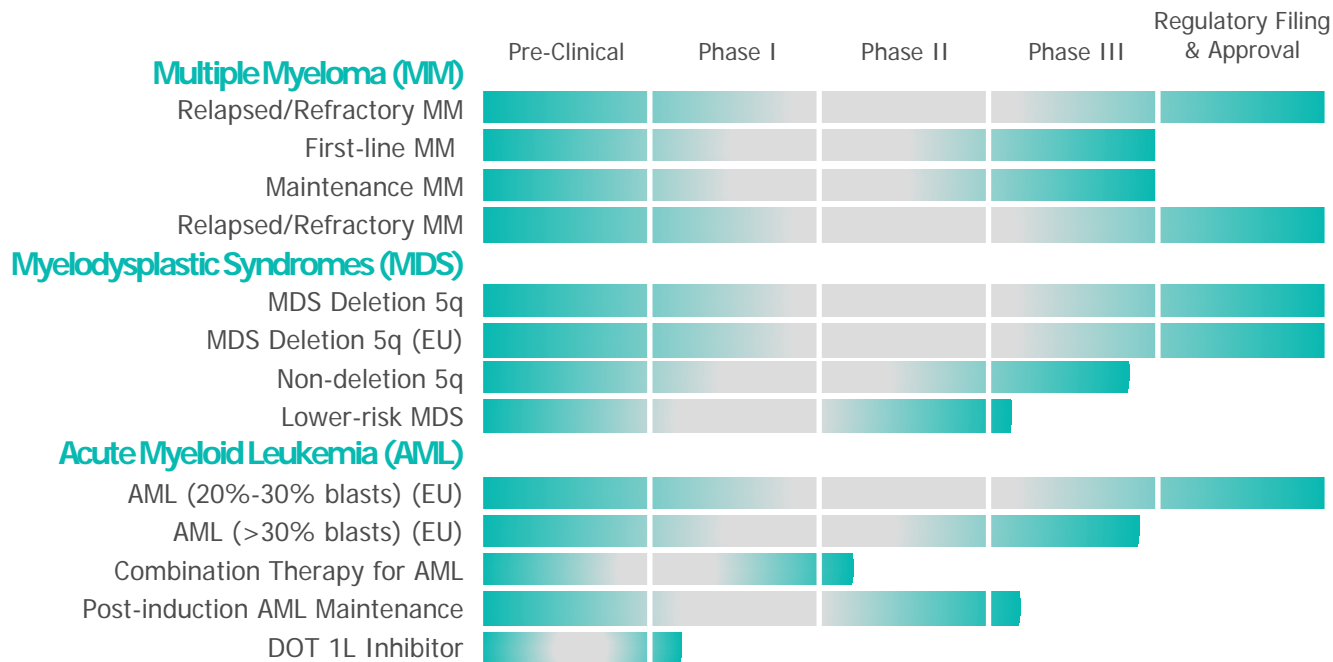
## Celgene Research Spans Broad Range of Hematological Malignancies



**250,000**

People will die this year from hematologic cancers that are considered orphan diseases

# Celgene's Leading Hematology Pipeline

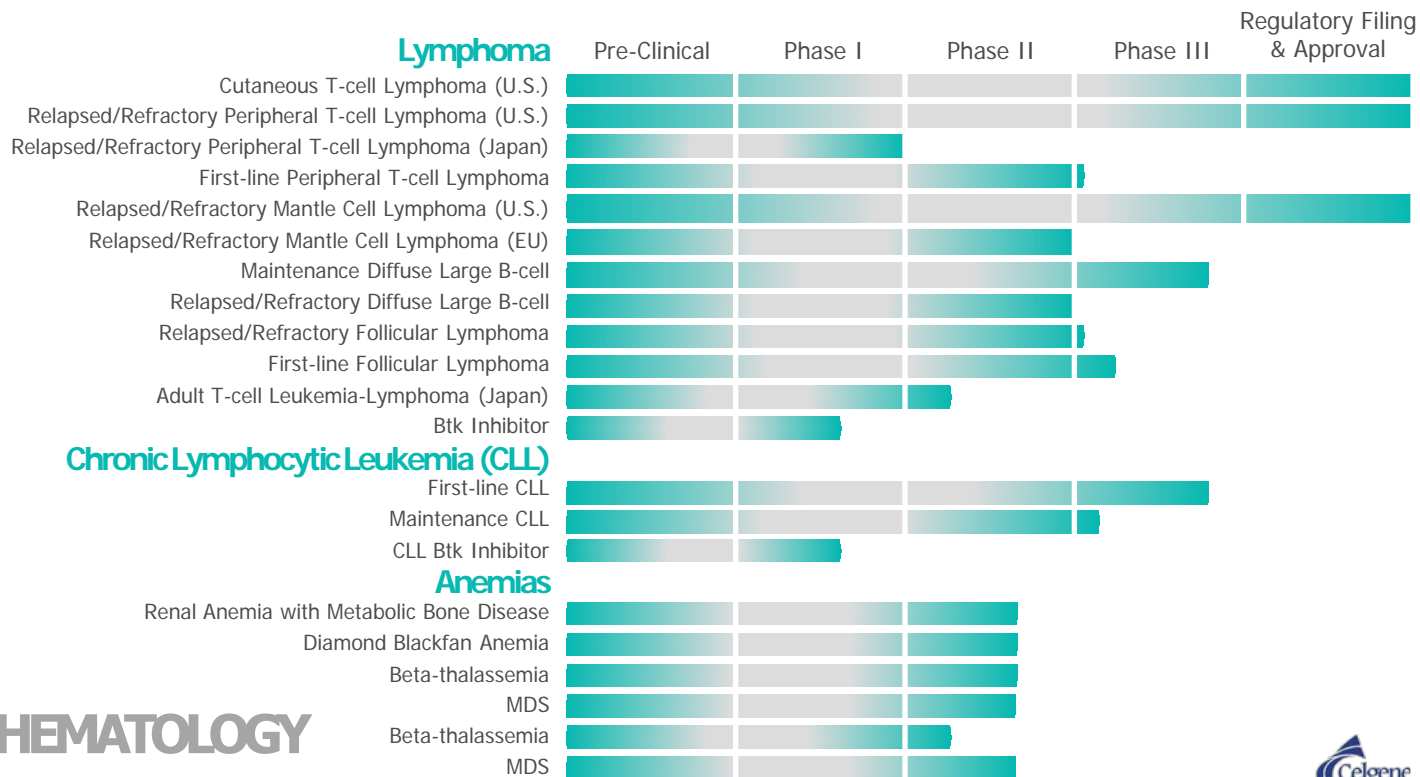


## HEMATOLOGY

See Notes and Sources, #9.

4 BETTER HEALTHCARE,  
BETTER OUTCOMES

# Celgene's Leading Hematology Pipeline (cont'd)



# Changing the Course of Disease by Focusing on The Cause and Not the Symptoms



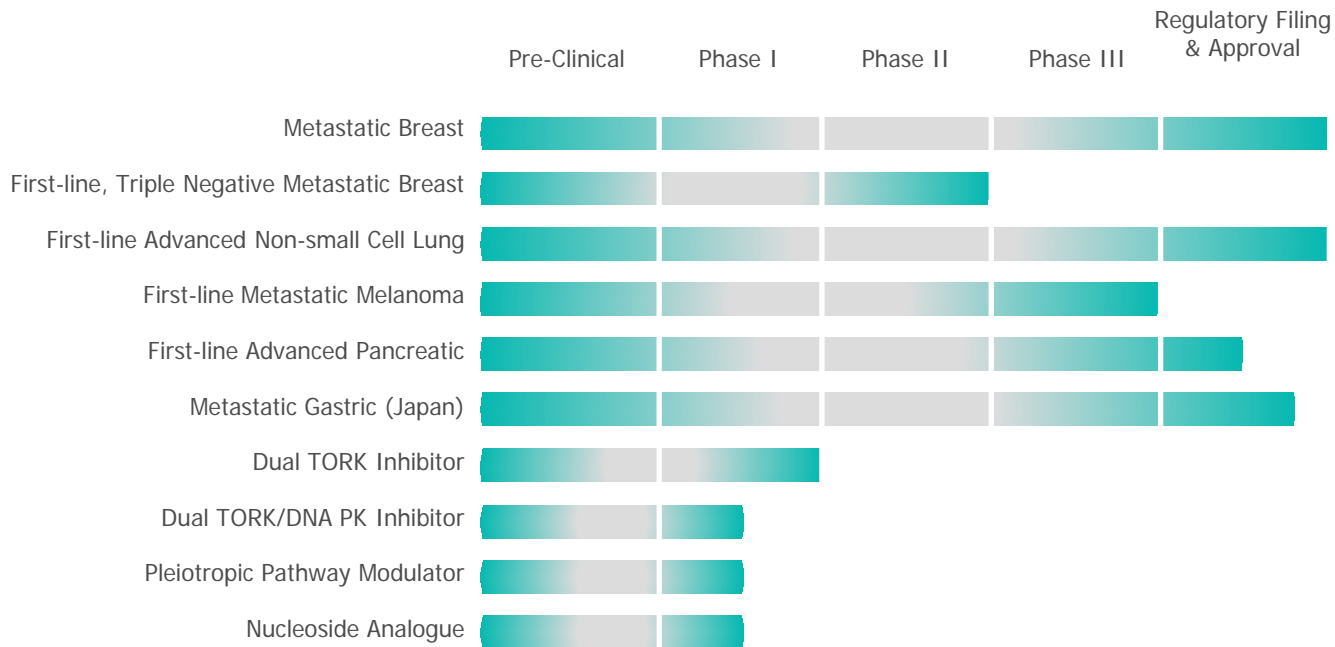
Celgene Hematology is the foundation on which our company was built. From our earliest efforts with thalidomide in myeloma to ongoing clinical studies in multiple diseases, our therapies are transforming the landscape of the treatment of blood cancers.

Focused on treating the underlying disease, therapies such as our IMiDs® franchise do more than simply address the symptoms of disease. Using a combination of actions, these therapies both attack cancer cells and bolster the body's own defense system to fight diseases such as myeloma.

In addition to IMiDs, our hematology portfolio includes powerful epigenetic therapies that dampen tumor cells' ability to survive and reproduce. This mechanism of action has allowed us to make great strides in diseases such as myelodysplastic syndromes and t-cell lymphomas.

Research continues around next-generation therapies in our current indications and in other diseases with significant unmet needs, including acute myeloid leukemia, myelofibrosis, non-Hodgkin lymphoma, diffuse large B-cell lymphoma, mantle cell lymphoma, chronic lymphoid leukemia and more.

# Celgene's Expanding Oncology Pipeline



# Changing the Course of Human Health Through Bold Pursuits in Oncologic Research

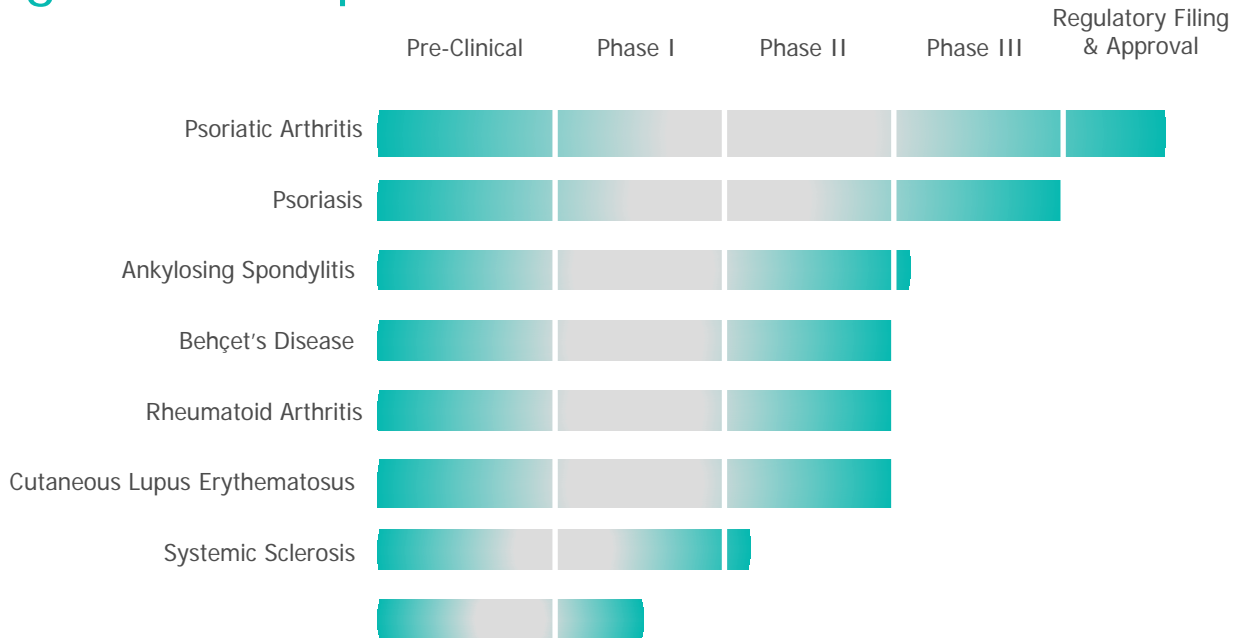
Along with the global growth of our company has come an expanded view of indications in which we believe we can make a significant contribution. In particular, solid tumor cancers are a natural extension of the success we have achieved in hematology.

Our compounds are showing promise in a range of tumor types and have entered phase III trials in pancreatic cancer – a historically difficult disease to treat at late stages.

Within the oncology franchise, our principal therapy combines a traditional taxane with human albumin through a unique nanotechnology-based formulation process. The resulting product delivers more of the treatment to its intended destination, while simultaneously minimizing solvent-related safety concerns.

With the emergence of our Celgene Oncology group, we are moving into new disease areas with the potential to help exponentially more patients.

# Celgene's Inflammation and Immunology Emerging Product Pipeline



## INFLAMMATION & IMMUNOLOGY



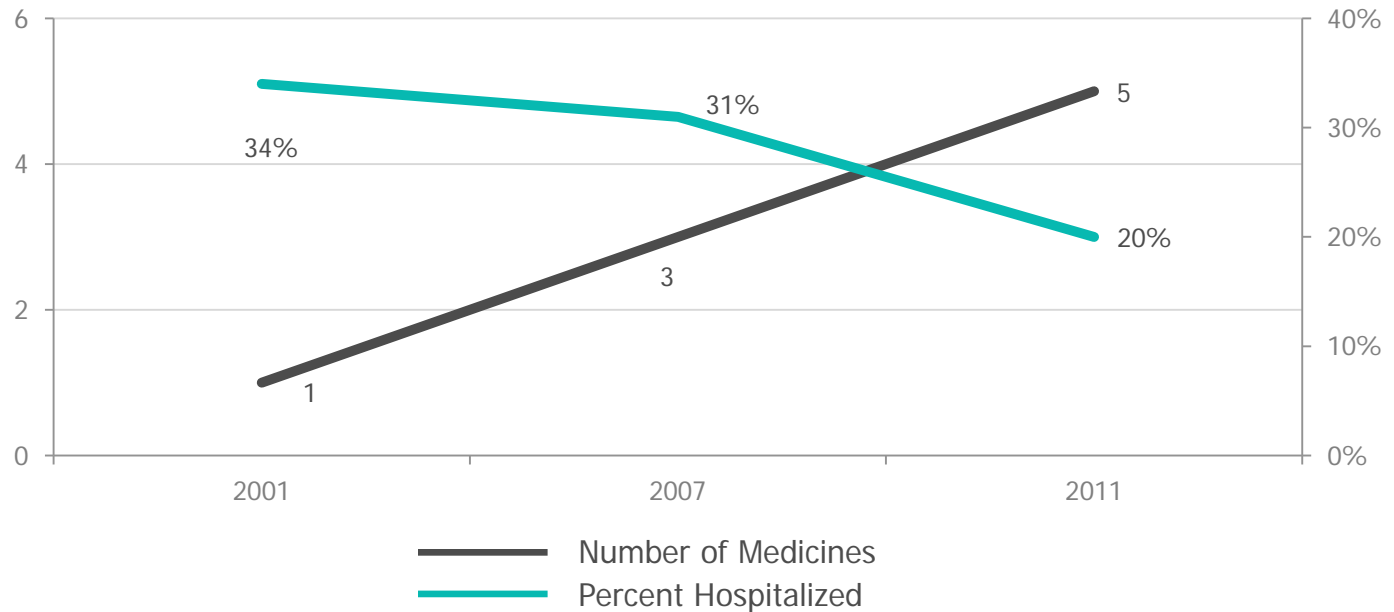
# Next Generation Therapies Delivering Quality Outcomes for Better Healthcare

Celgene I&I holds the potential to help a large group of patients outside of our core cancer discipline, those living with debilitating immune-related and inflammatory diseases like psoriasis, arthritic conditions, Crohn's disease and more.

Long a part of the company's pipeline, these therapies have advanced into late-stage clinical trials where they are addressing significant unmet needs in serious disease areas.

Built upon the ongoing research of scientists in both Celgene and Celgene Cellular Therapeutic laboratories, I&I now features an emerging pipeline of innovative oral immunomodulatory compounds, kinase inhibitors and placenta-derived cellular therapies.

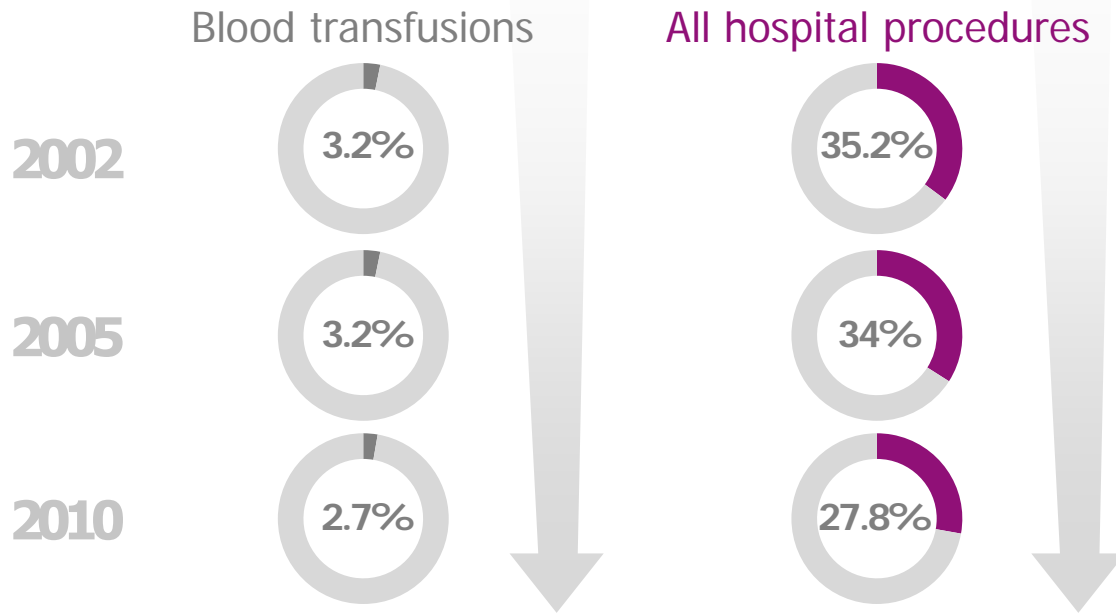
# Innovative Therapies Reduce Hospitalization Rates Associated with Blood Cancers



**Source 11:** Table 1.22 – continued U.S. Prevalence Counts, Invasive Cancers Only, January 1, 2010a Using Different Tumor Inclusion Criteria [http://seer.cancer.gov/csr/1975\\_2010/results\\_merged/topic\\_prevcounts.pdf](http://seer.cancer.gov/csr/1975_2010/results_merged/topic_prevcounts.pdf). Data on procedures and hospitalizations from <http://hcupnet.ahrq.gov/>. Weighted national estimates from HCUP Nationwide Inpatient Sample (NIS), 2001-2011. Agency for Healthcare Research and Quality (AHRQ), based on data collected by individual States and provided to AHRQ by the States.



# Declining Blood Transfusions and Hospital Procedures per Myeloma Patient



Source 12: Agency for Healthcare Research and Quality's HCUPnet: Healthcare Cost and Utilization Project. Nationwide Inpatient Sample. Available at <http://hcupnet.ahrq.gov/HCUPnet.jsp>. Accessed 11/14/13.

## Fewer Myeloma Patients Being Hospitalized

3.3

2000



2.3

2009

Ratio of number of hospital discharges for principal diagnosis of myeloma to 5-year limited-duration myeloma prevalence

**Source 12:** Agency for Healthcare Research and Quality's HCUPnet: Healthcare Cost and Utilization Project. Nationwide Inpatient Sample. Available at <http://hcupnet.ahrq.gov/HCUPnet.jsp>. Accessed 11/14/13. National Cancer Institute, Surveillance Epidemiology and End Results (SEER). Available at <http://seer.cancer.gov/faststats/selections.php?>. Accessed 11/14/13.

# Notes and Sources



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7. Archstone Consulting Analysis, PhRMA Industry Profile 2008. Burrill & Company Analysis), Minnesota IMPLAN Group, Inc., R. J. Shapiro and N. D. Pham (2007).
8. PhRMA analysis based on National Science Board. "Science and Engineering Indicators 2012." Arlington, VA: National Science Foundation (NSB12-01), 2012. R.A. Woods. "Employment Outlook: 2008–2018. Industry Output and Employment Projections to 2018." Monthly Labor Review 2009; Washington, DC: Bureau of Labor Statistics.
9. The products represented as in development and found in the product pipeline are intended for investors and members of the media to provide general information on Celgene. This information is not represented to be a complete description and is subject to change without notice. Celgene Corporation may from time to time update this information but does not warrant that will take place at any particular time nor assume any obligation to update this information.
10. Based on Kantar Health's CancerMPact® epidemiology database for the U.S., EU5 and Japan. 2011.
11. Table 1.22 – continued U.S. Prevalence Counts, Invasive Cancers Only, January 1, 2010a. Using Different Tumor Inclusion Criteria [http://seer.cancer.gov/csr/1975\\_2010/results\\_merged/topic\\_prevcounts.pdf](http://seer.cancer.gov/csr/1975_2010/results_merged/topic_prevcounts.pdf). Data on procedures and hospitalizations from <http://hcupnet.ahrq.gov/>. Weighted national estimates from HCUP Nationwide Inpatient Sample (NIS), 2001-2011. Agency for Healthcare Research and Quality (AHRQ), based on data collected by individual States and provided to AHRQ by the States.
12. Agency for Healthcare Research and Quality's HCUPnet: Healthcare Cost and Utilization Project. Nationwide Inpatient Sample. Available at <http://hcupnet.ahrq.gov/HCUPnet.jsp>. Accessed 11/14/13. National Cancer Institute, Surveillance Epidemiology and End Results (SEER). Available at <http://seer.cancer.gov/faststats/selections.php>. Accessed 11/14/13.





# 5

## A WORLD FREE FROM CANCER



### WHAT IS REQUIRED TO ACCELERATE INNOVATION IN CANCER?


This section will highlight the importance of sustaining and spreading innovation throughout the world. The alternative would lead to the negative impact of halting medical progress and innovation in cancer and its effect on survival, death, prosperity and progress.

Yet, we will also show that the cost and commitment necessary to keep our promise to our children of a world free from cancer is increasing. The (mostly private sector) investment in a cancer free world is not keeping up with the potential and is even declining.

Impeding innovation and interrupting the virtuous cycle of medical progress will cost both lives and economic growth. Fortunately, we know what is needed to sustain the incentives for investment in innovation to continue the medical progress that the virtuous cycle of innovation will bring in the 21<sup>st</sup> century.



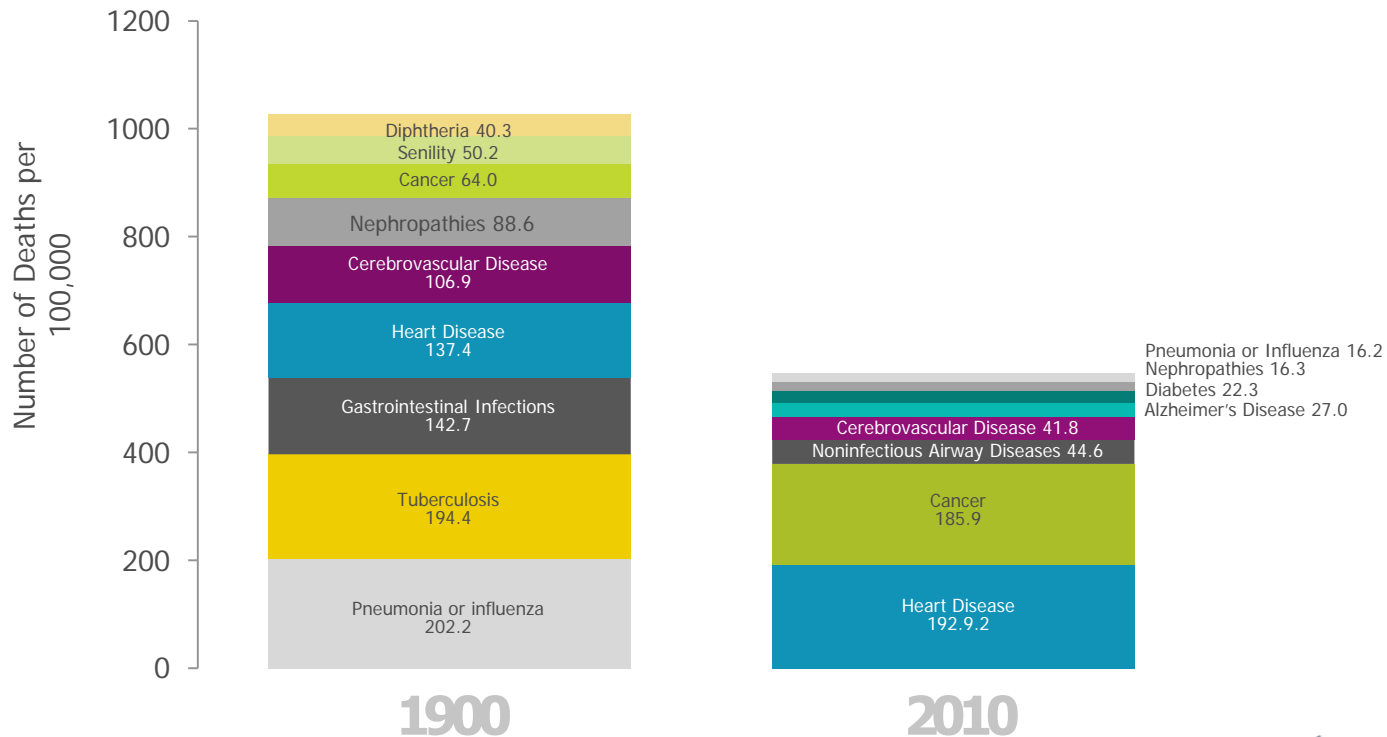




“ Cancer research **today**, especially on the frontiers America’s cancer researchers are renowned for spearheading, requires **investment** at a **scale unimaginable** 40 years ago. ”

National Cancer Institute

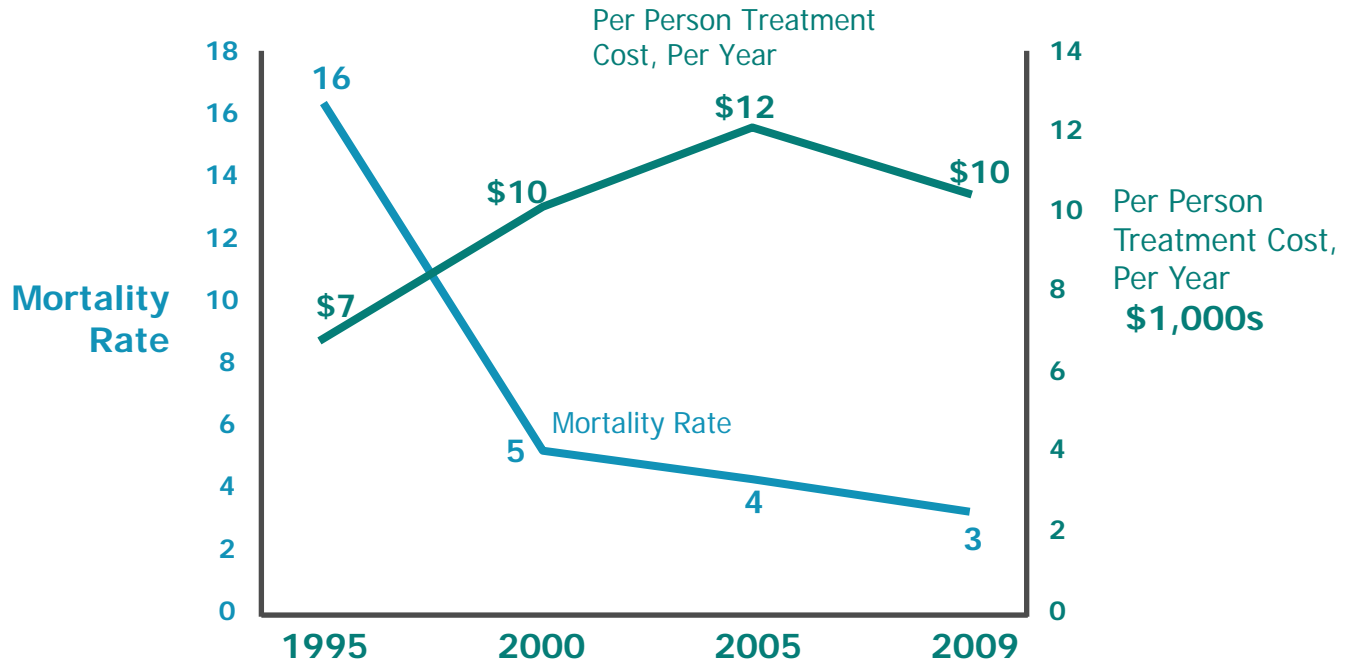
# Medical Innovation Freeing the World from Disease



Source 1: Centers for Disease Control and Prevention and National Center for Health Statistics. National Vital Statistics System. 2011. Available at <http://www.cdc.gov/nchs/nvss.htm>. Accessed 11/12/12.



# Use of New HIV Therapies Associated with Decline in HIV Deaths



Source 2: Agency for Healthcare Research and Quality's HCUPnet: Healthcare Cost and Utilization Project. Available at <http://hcupnet.ahrq.gov/>. Kaiser Family Foundation AIDS Drug Assistance Program Fact Sheets. Available at <http://www.kff.org/hivaids/>. Accessed 11/16/12.

## The Value of Medical Innovation: HIV



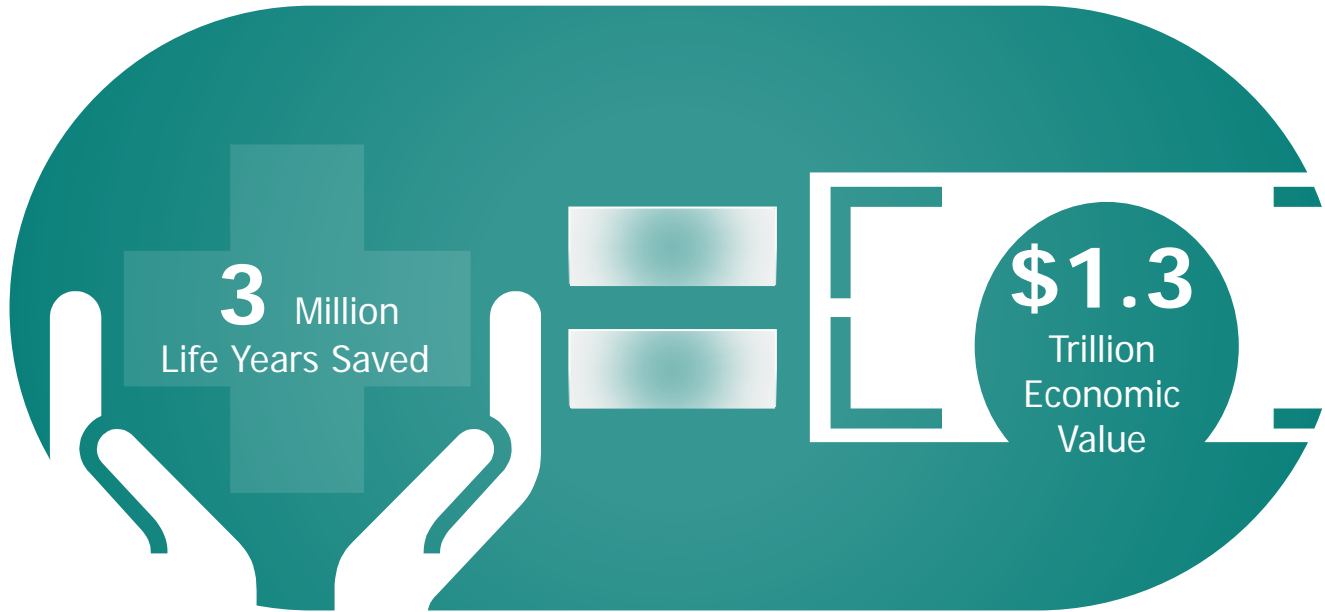
4 HIV Therapies



90% decline in  
people dying from HIV

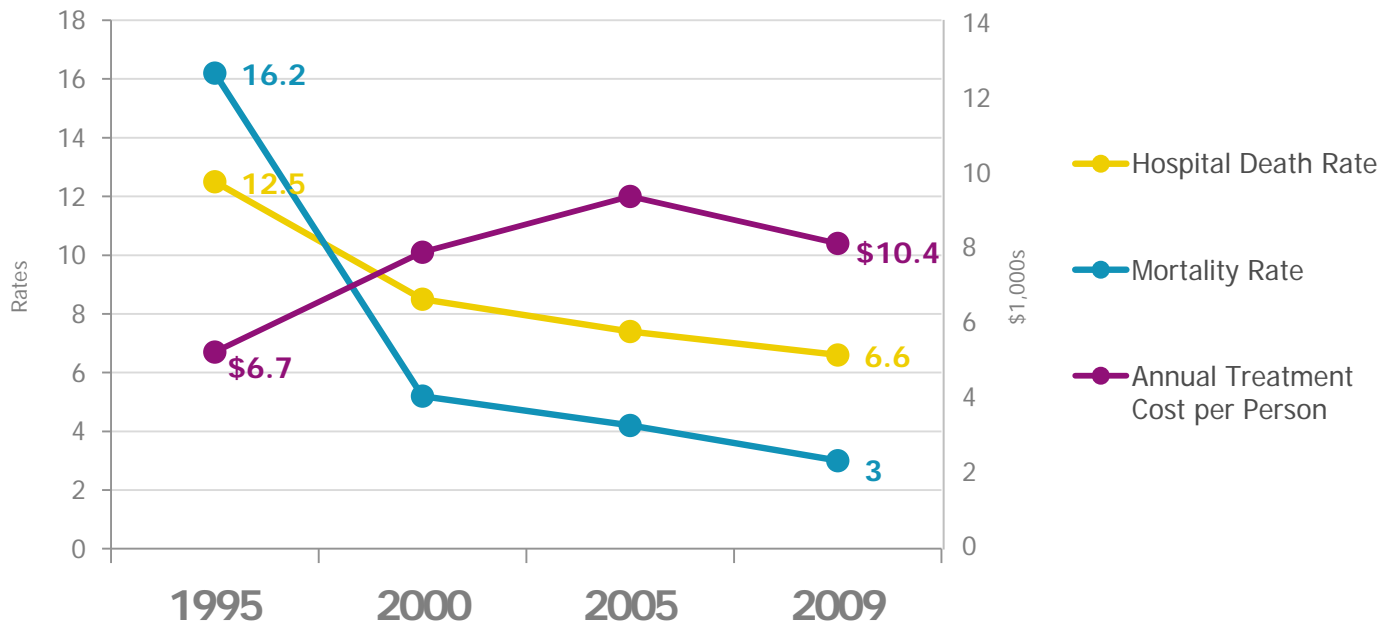


# Medical Innovation Delivered on Its Promise for Patients with HIV



**Source 4:** Walensky RP, Freedberg KA, Weinstein MC. Cost-Effectiveness of HIV Testing and Treatment in the United States. *Clin Infect Dis.* 2007; 45, Supplement 4: S248-S254. Available at [http://cid.oxfordjournals.org/content/45/Supplement\\_4/S248.full](http://cid.oxfordjournals.org/content/45/Supplement_4/S248.full). Accessed 11/16/12.

# Introduction of HIV Therapies Associated with Decline in Hospitalization and Death

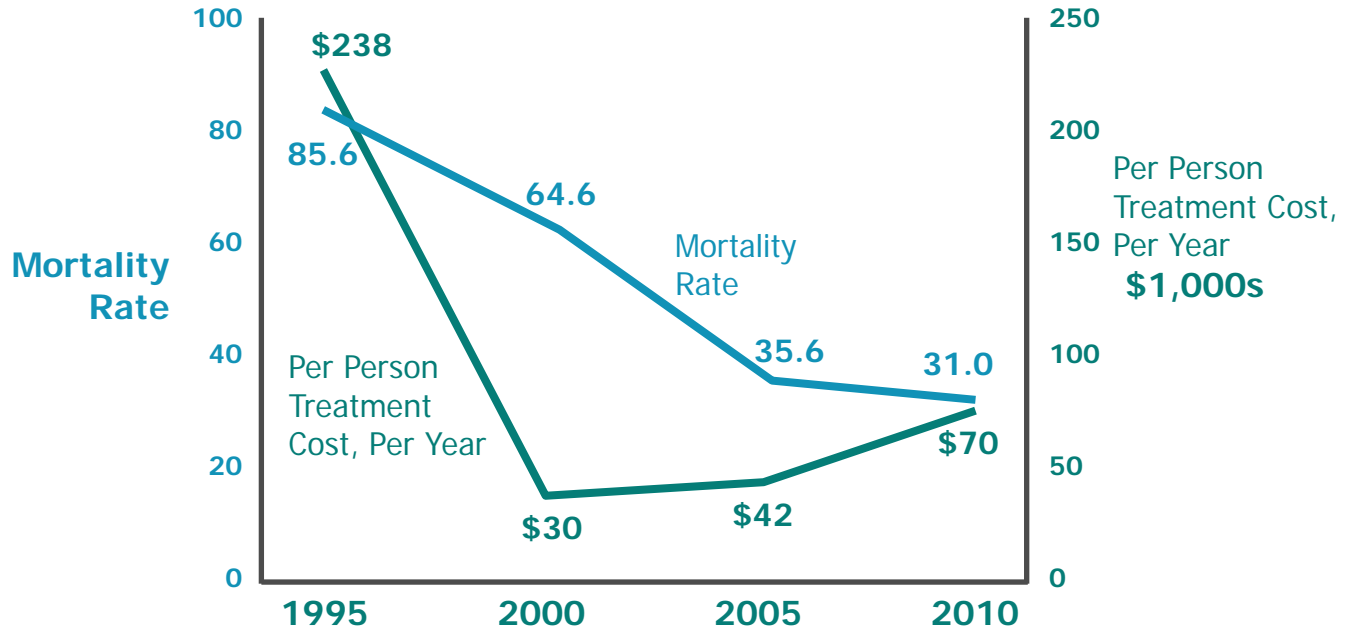


Source 5: Centers for Disease Control and Prevention and National Center for Health Statistics. AIDS and HIV Statistics. Available at

94 <http://www.cdc.gov/nchs/fastats/aids-hiv.htm>. Kaiser Family Foundation ADAP Fact Sheets. Available at <http://www.kff.org/hiv/aids/>. Accessed 11/13/12.



# Medical Innovation Increased Longevity and Reduced Healthcare Costs for CML Patients



Source 6: National Cancer Institute, Surveillance Epidemiology and End Results (SEER). Available at <http://seer.cancer.gov/faststats/>. Accessed 7/31/13.

Source 7: Drug Bank. Interferon Alfa-2b, Recombinant. Available at <http://www.drugbank.ca/drugs/DB00105>. Accessed 7/31/13.

Source 8: Glivec Patient Assistance Program Patient Guide. Available at <http://ramoslink.info/pubs/GlivecPAP.pdf>. Accessed 7/31/13.

# Novel Targeted CML Therapies Help Patients Live Longer and Healthier

Since 2001



## 3 New CML Therapies



**68%**  
decline in  
people dying  
from CML





# Decrease in CML Deaths Resulted in Economic Growth

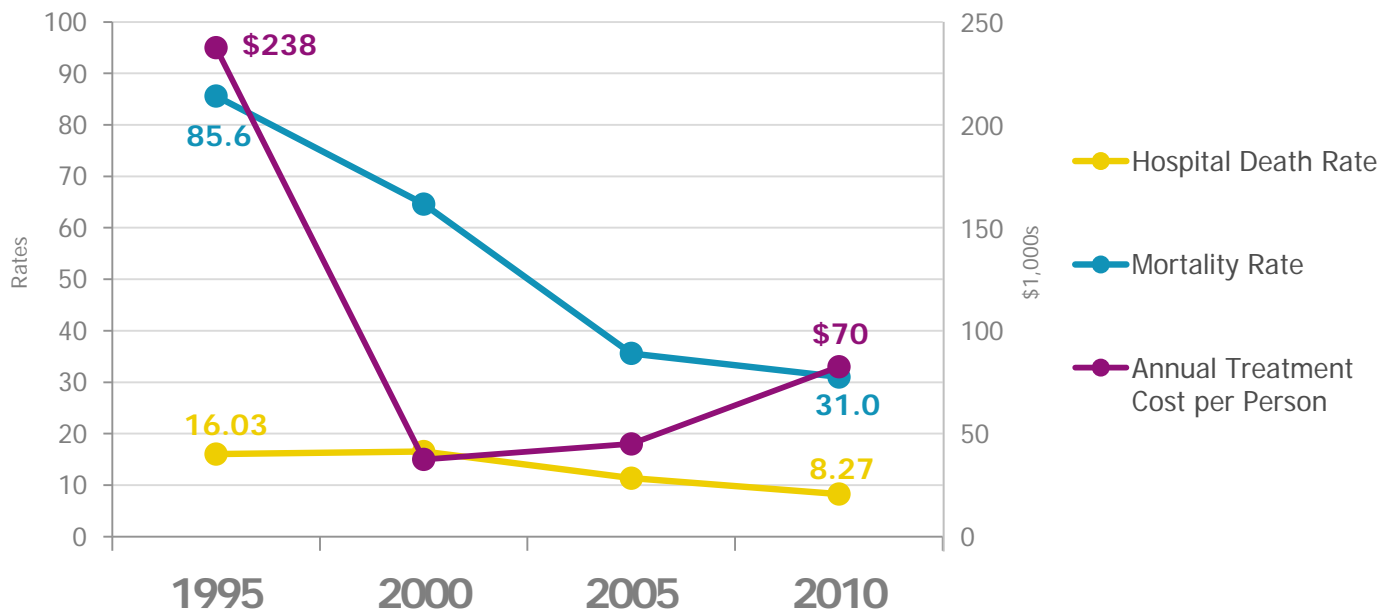


*\*Assuming an average incidence of 5,000 new CML diagnosis and 70% 20-year survival rate*

*\*\*Based on first- and second-line tyrosine kinase inhibitors*

Source 10: SCB Analysis, National Cancer Institute, Surveillance Epidemiology and End Results (SEER). Available at <http://seer.cancer.gov/faststats/>. Accessed 7/31/13.

# Introduction of Targeted CML Therapies Associated with Decline in Hospitalization and Death



Source 6: National Cancer Institute, Surveillance Epidemiology and End Results (SEER). Available at <http://seer.cancer.gov/faststats/>. Accessed 7/31/13.

Source 7: Drug Bank. Interferon Alfa-2b, Recombinant. Available at <http://www.drugbank.ca/drugs/DB00105>. Accessed 7/31/13.

Source 8: Glivec Patient Assistance Program Patient Guide. Available at <http://ramoslink.info/pubs/GlivecPAP.pdf>. Accessed 7/31/13.

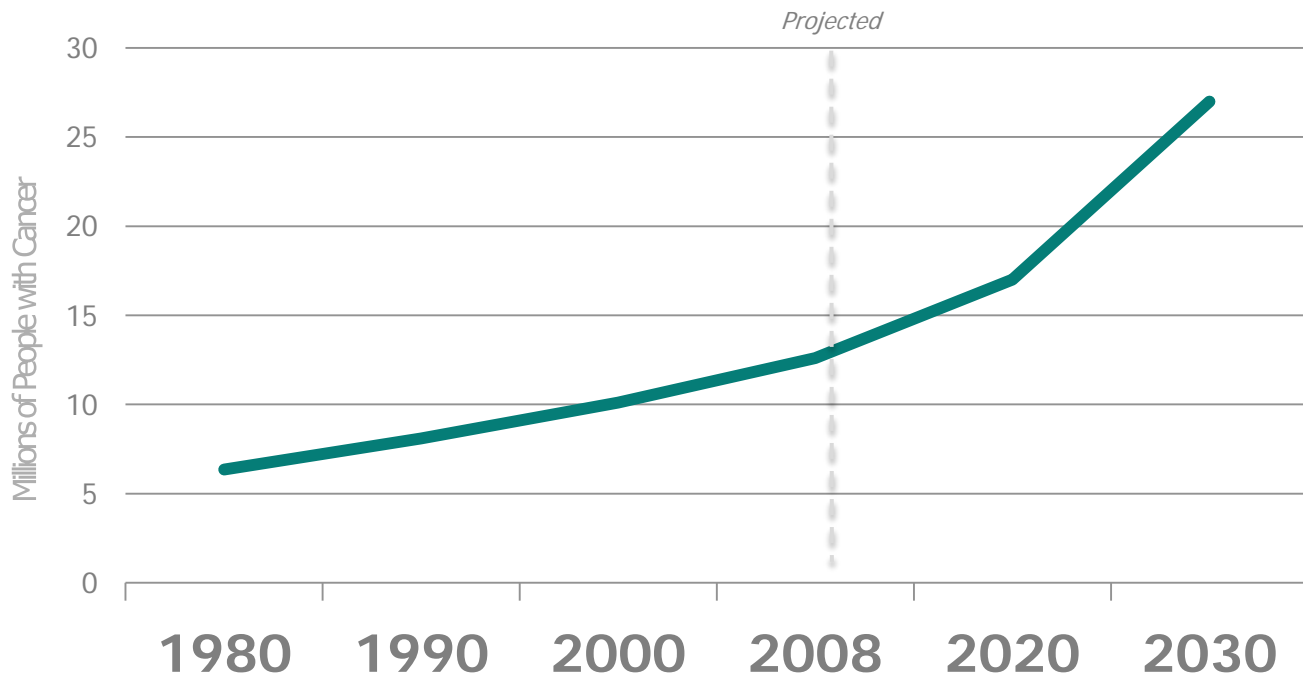
Source 11: Agency for Healthcare Research and Quality. Healthcare Cost and Utilization Project (HCUP). Available at <http://hcupnet.ahrq.gov/>.

Accessed 7/31/13.





# Cancer Incidence is Increasing Worldwide



# The Promise of Medical Innovation = Fewer Lives Lost



## Life Years Lost to Cancer

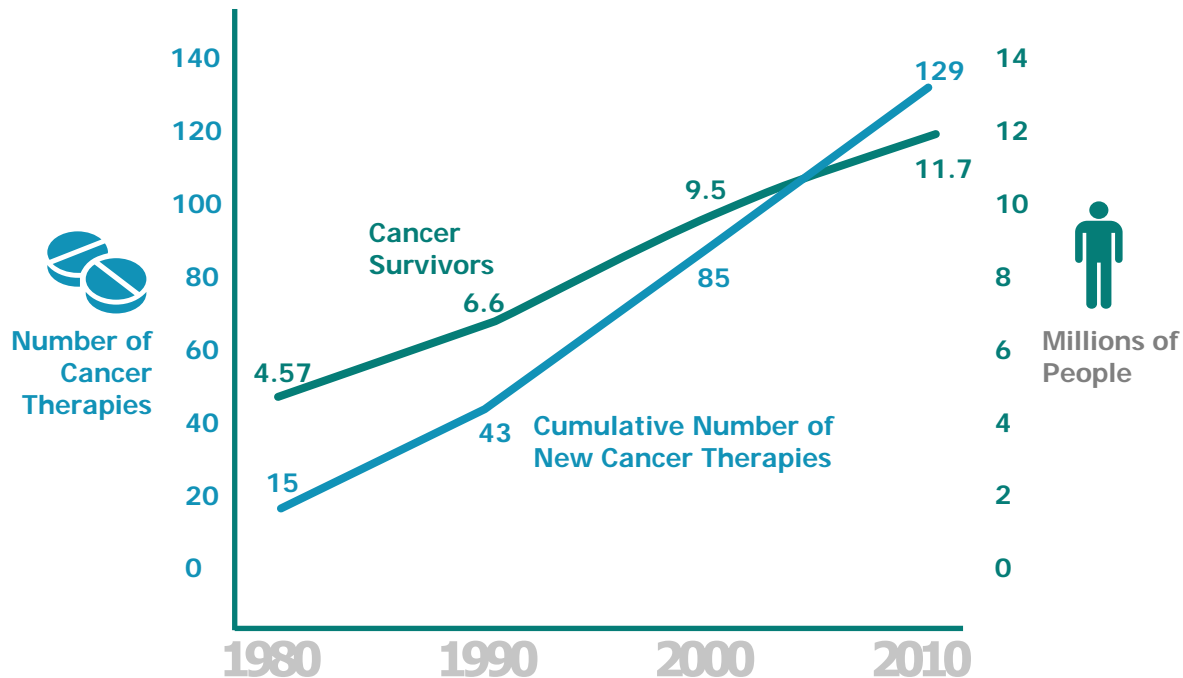
2010 **169** Million

**43%**  
REDUCTION

Projected in  
2040 **96** Million

**Source 13:** Estimate and projection of reduction of 96 million life years lost to cancer projects linear decline based on previous decade long change. Soerjomataram I, et al Global burden of cancer in 2008: a systematic analysis of disability-adjusted life-years in 12 world regions. Lancet 2012; 10.1016/S0140-6736(12)60919-2.

# More People are Surviving as More New Therapies Are Developed



Source 14: National Cancer Institute, Surveillance Epidemiology and End Results (SEER). Available at [http://seer.cancer.gov/csr/1975\\_2009\\_pops09/index.html](http://seer.cancer.gov/csr/1975_2009_pops09/index.html). Accessed 10/31/12. U.S. Food and Drug Administration's Drug Approval Database. Available at <http://www.accessdata.fda.gov/scripts/cder/drugsatfda/index.cfm>. Accessed 11/16/12.



Only **1%** of healthcare dollars are dedicated to new cancer therapies

Since 1990, this small investment in innovative treatments has resulted in ...



**New Economic Activity**

**Source 15:** Lichtenberg, FR. Current estimates based on "Has Medical Innovation Reduced Cancer Mortality?" National Bureau of Economic Research. Revised April 12, 2013. Calculation based on average per year increase in life years between 1990-2010 times average value of life year of \$96,000 Statista. Statistics and facts about cancer in the U.S. Available at <http://www.statista.com/topics/1192/cancer-in-the-us/>. Accessed 10/31/13.

## Cancer Treatment Results in Tenfold Increase in Productivity





# Investment in Medical Innovation is the Most Powerful Force for Human Progress

Save  
**\$158**  
Billion  
a year

If cancer were prevented in 2020, we could save:

- \$158 Billion** a year in medical costs
- 1.9 Million** lives
- \$2.2 Trillion** in value

**Source 17:** National Cancer Institute. Cancer costs projected to reach at least \$158 billion in 2020. Available at <http://www.cancer.gov/newscenter/newsfromnci/2011/CostCancer2020>. Accessed 11/15/12. Estimates based on preventing the estimated 1.9 million new cancer cases expected in 2020. Mariotto AB, Yabroff KR, Shao Y, Feuer EJ, and Brown ML. Projections of the Cost of Cancer Care in the United States: 2010-2020. 2011. JNCI, Vol. 103, No. 2.

# Science Driving Medical Innovation is Accelerating Exponentially

## Increases in Medical Innovation



1KB



2MB

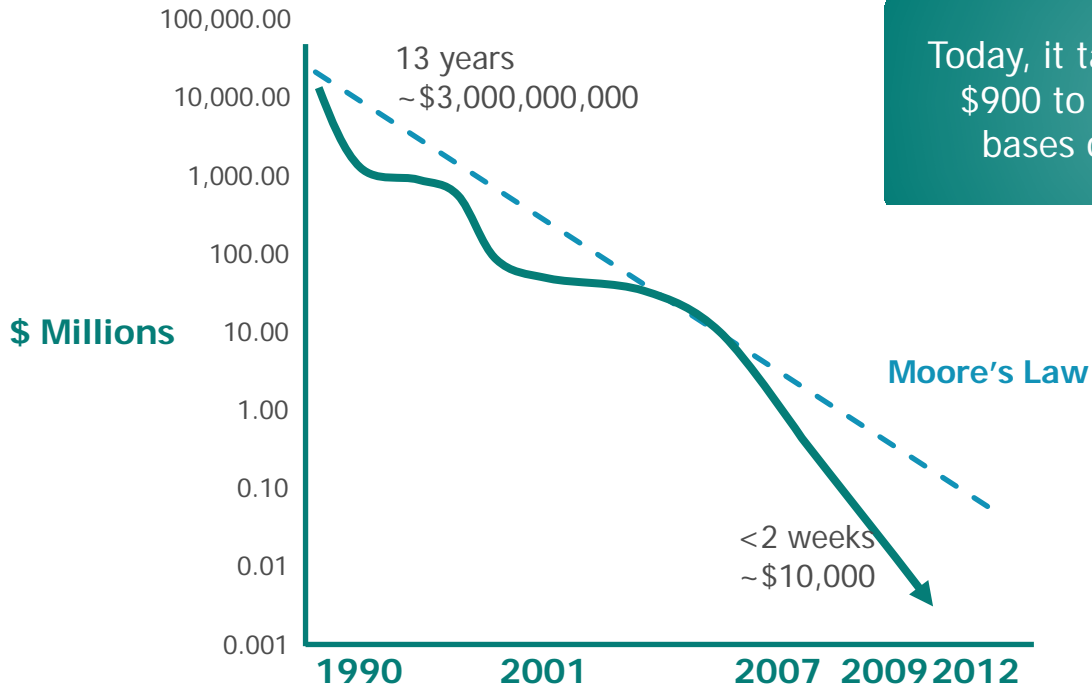


2GB

### Moore's Law:

Coined around 1970, this law states that processor speeds, or overall processing power, for computers will double every two years

# Investment in Medical Innovation Outpacing Moore's Law

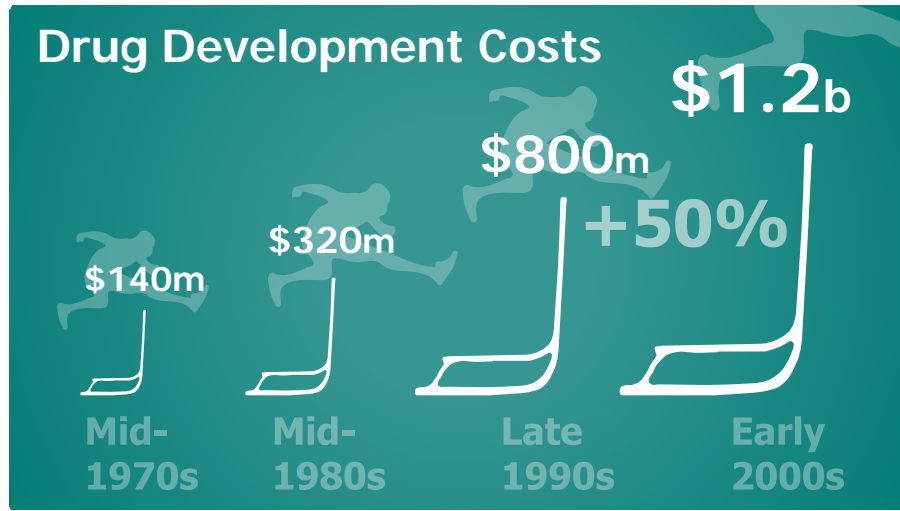


Today, it takes six hours and \$900 to read 150 million bases of genetic code

Source 19: Life Technologies. Available at <http://www.lifetechnologies.com/us/en/home.html> . Accessed 11/16/12. National Center for Human Genome Research Institute. Available at <http://www.genome.gov/>. Accessed 11/16/12.

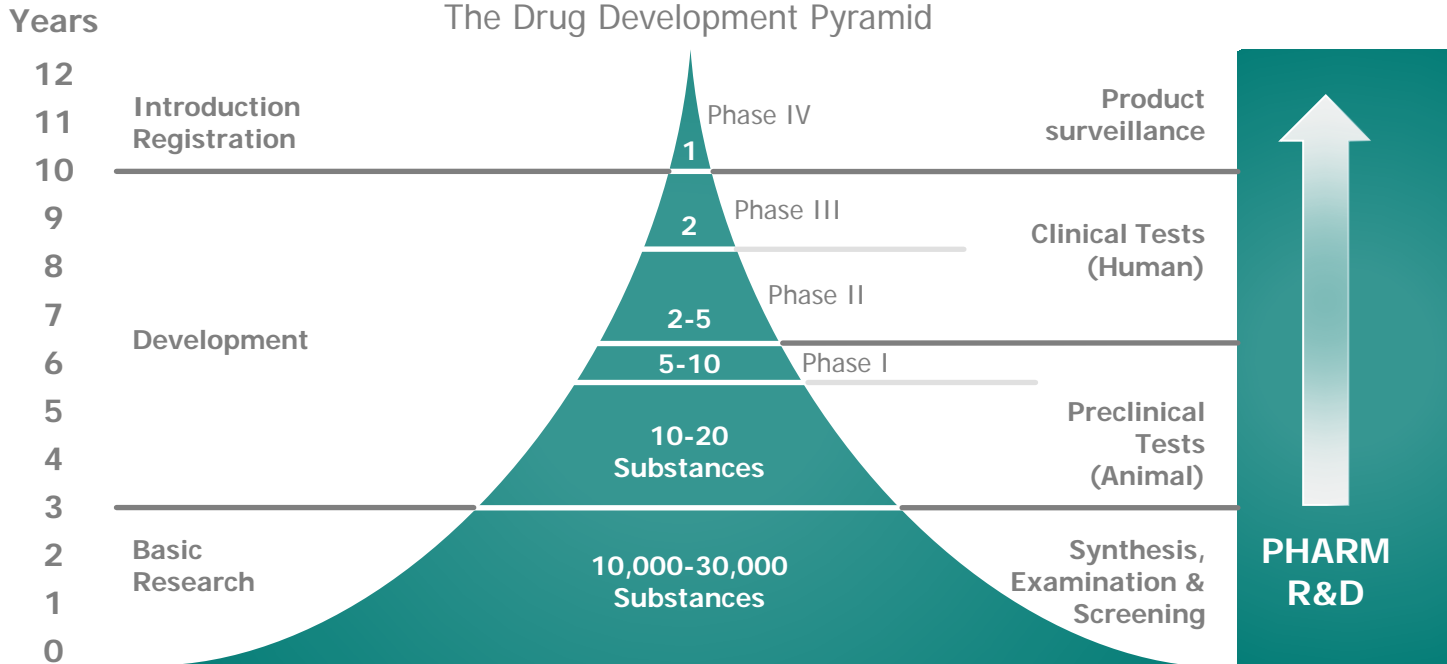
# Drug Development Costs Reach New Highs

The average cost to develop one new approved therapy – including the cost of failures – **increased approximately 50%** between the late 1990s and early 2000s



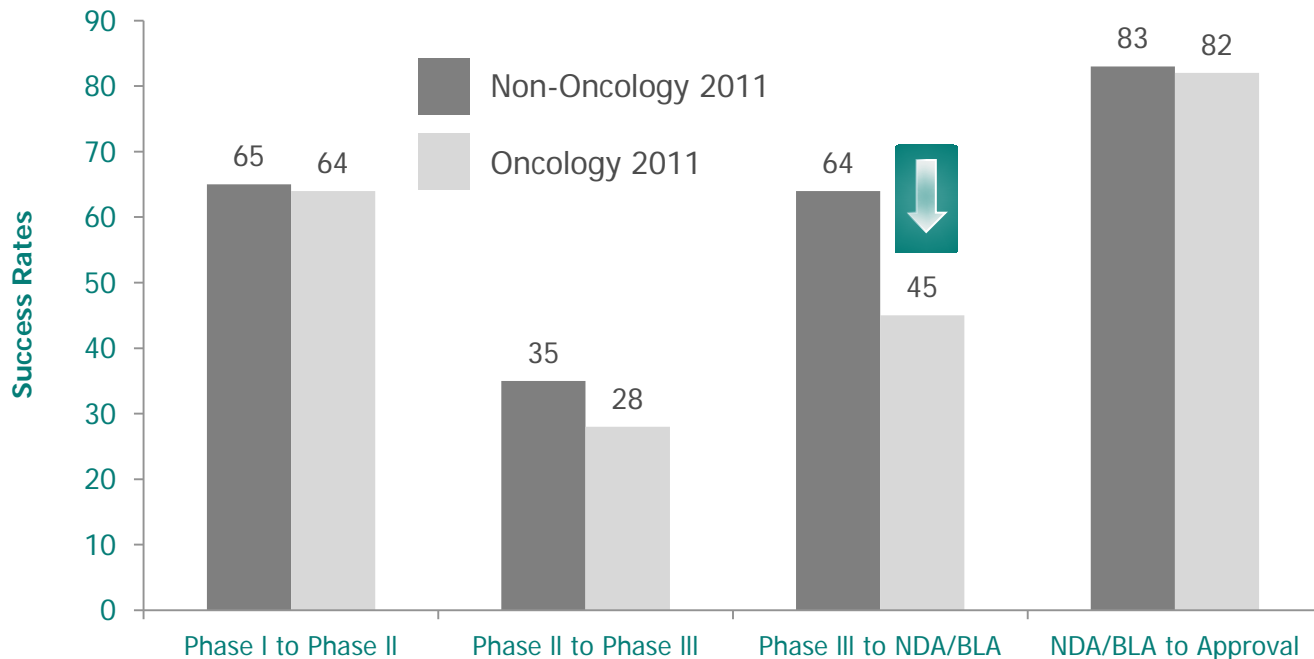
**Source 20:** DiMasi JA and Grabowski H. The Cost of Biopharmaceutical R&D: Is Biotech Different? Managerial and Decision Economics. 2007 (28): 469–79. Pharmaceutical Research and Manufacturers of America 2013 Profile Biopharmaceutical Research Industry. Available at <http://phrma.org/sites/default/files/pdf/PhRMA%20Profile%202013.pdf>. Accessed 11/15/13. Tufts Center for the Study of Drug Development, "Average Cost to Develop a New Biotechnology Product Is \$1.2 Billion, According to the Tufts Center for the Study of Drug Development." November 9, 2006. Available at <http://csdd.tufts.edu/NewsEvents/NewsArticle.asp?newsid=69>. Accessed 11/14/13.

# Takes 12 Years or Longer to Bring a New Treatment to Patients



Source 21: Briggs A. Effective Use of Health Technology Assessment to Maximize Market Access: Start Early and Update Often. Oxford Outcomes (an ICON plc Company). Available at <http://www.iconplc.com/icon-files/insight-newsletter/June11/effective.html>. Accessed 11/1/13.

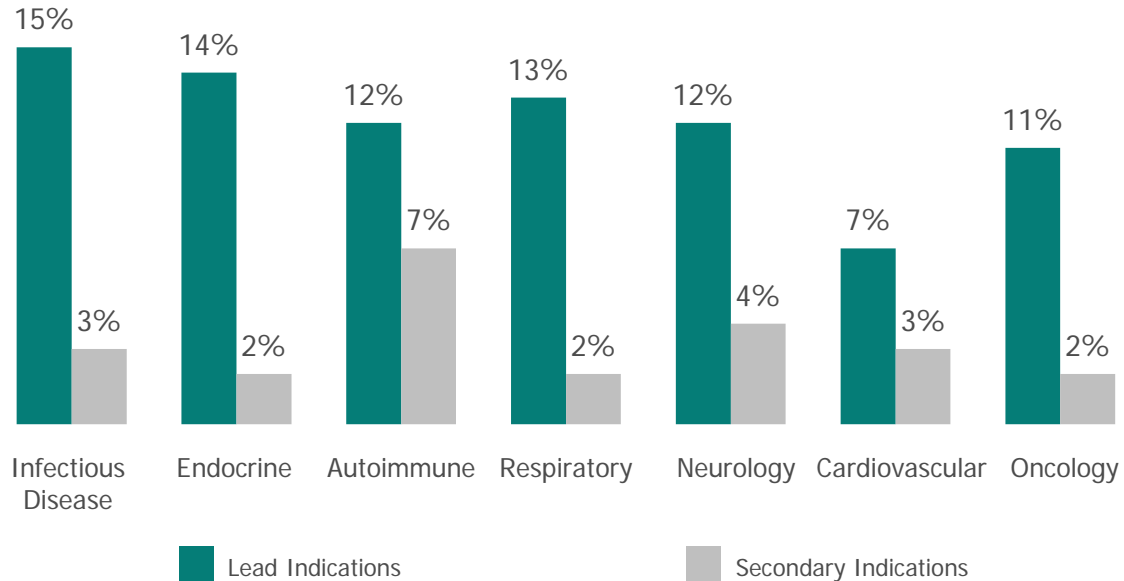
## Developing New Cancer Therapies Takes Even Longer, With Lower Success Rates



Source 22: BIOTech Now. Oncology Clinical Trials – Secrets of Success. Available at <http://www.biotech-now.org/business-and-investments/2012/02/oncology-clinical-trials-secrets-of-success>. Accessed 11/15/13.

# New Cancer Therapies Face Higher Hurdles

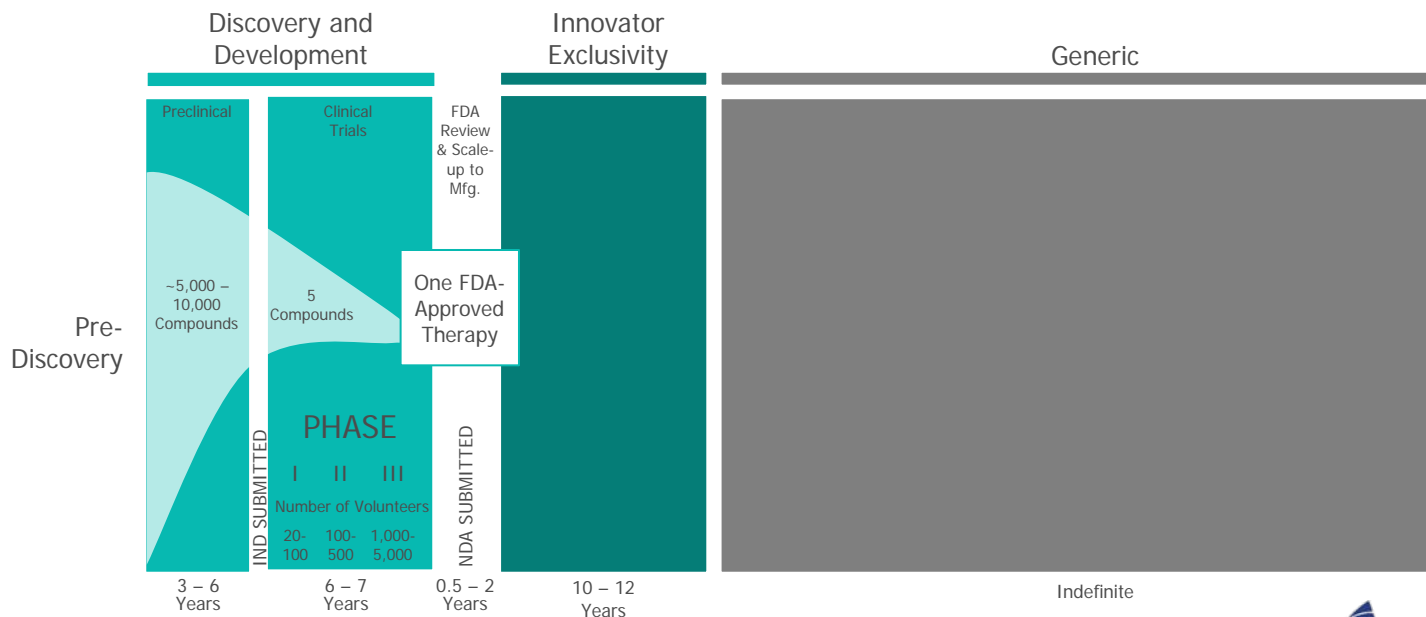
## Higher Overall Success by Disease



Source 23: BIOTech Now. Clinical Success Rate by Disease Area. Available at <http://www.biotech-now.org/events/2011/02/release-of-biobiomedtracker-drug-approval-rates-study/attachment/clinical-success-rate-blog-pic-2>. Accessed 11/15/13.

# A Novel Therapy Spends the Least Amount of Time in Its Lifecycle as a Branded Treatment

Developing a new medicine takes an average of 10–15 years; the Congressional Budget Office reports that “relatively few drugs survive the clinical trial process.” Innovative therapies have a limited time in their lifecycle to recapture investment and fund future innovation.



Source 24: Drug Discovery and Development: Understanding the R&D Process. Available at [www.innovation.org](http://www.innovation.org). Accessed 11/15/12. Congressional Budget Office, Research and Development in the Pharmaceutical Industry, 2006.

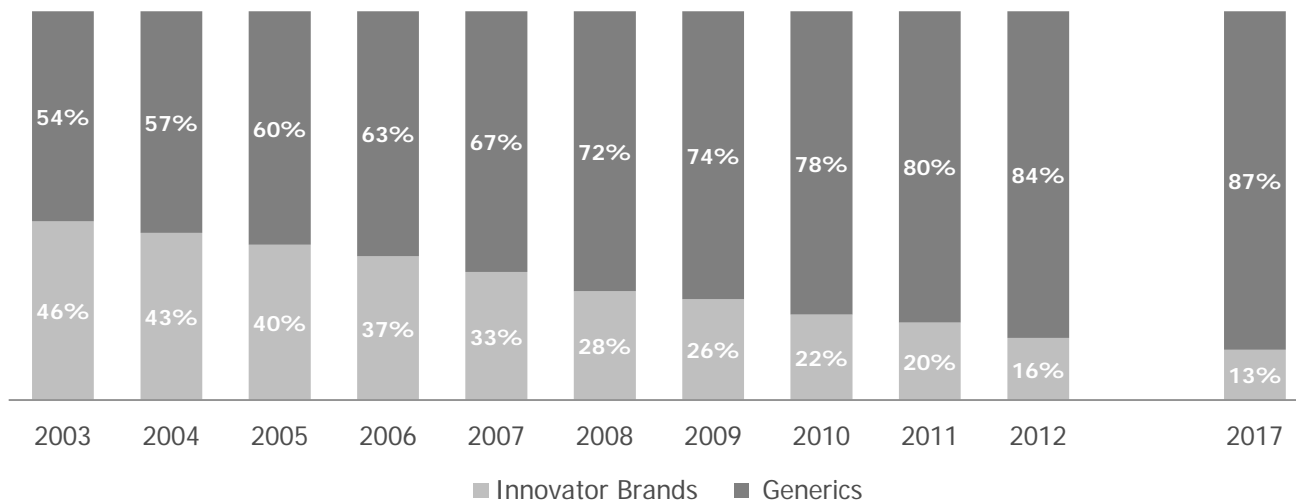




# A Dangerous Trend that Could Jeopardize the Virtuous Cycle of Medical Innovation



## Percent Share of Prescriptions

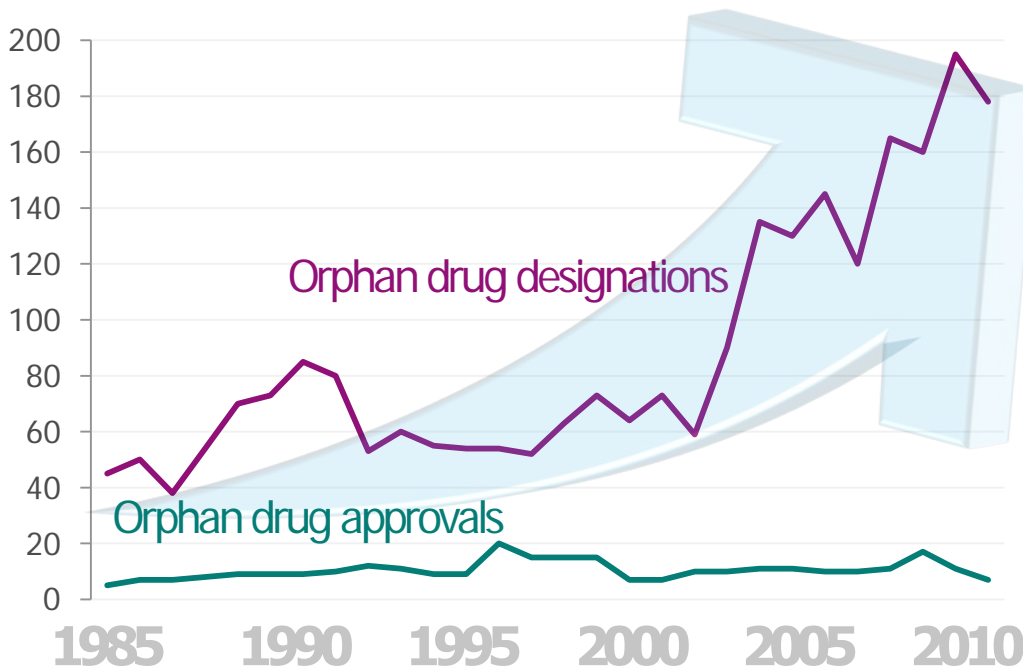


**Chart Notes:** Includes all prescriptions dispensed through retail pharmacies, including independent and chain drug stores, food store pharmacies and mail order as well as long-term care facilities. Generics include branded and unbranded generic medicines. Prescription counts are not adjusted for length of therapy. 90-day and 30-day prescriptions are both counted as one prescription.

**Source 25:** IMS Institute for Health Informatics Declining Medicine Use and Costs: For Better or Worse? A Review of the Use of Medicines in the United States in 2012. Available at <http://static.correofarmaceutico.com/docs/2013/05/20/usareport.pdf>. Accessed 11/12/13.

## Number of New Orphan Drug Designations Climb Yet Approvals Not Keeping Pace

Number of orphan drug designations and approvals

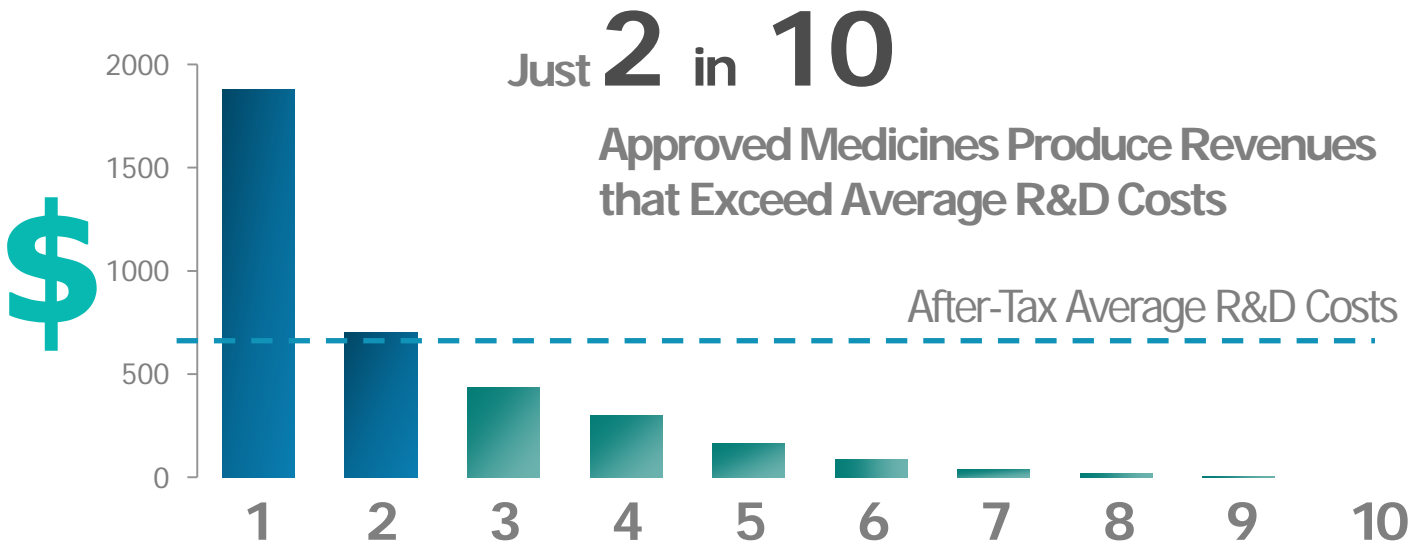


Source 26: Karst KR and Sasinowski FJ. FDA Law Blog. Chart accessed at The Orphan Drug Act: 30 Years and Still Going Strong!

114 [http://www.fdalawblog.net/fda\\_law\\_blog\\_hyman\\_phelps/2013/02/the-orphan-drug-act-30-years-and-still-going-strong.html](http://www.fdalawblog.net/fda_law_blog_hyman_phelps/2013/02/the-orphan-drug-act-30-years-and-still-going-strong.html). Accessed 11/12/13.



# Even After Approval, Few Medicines Recover Their R&D Costs



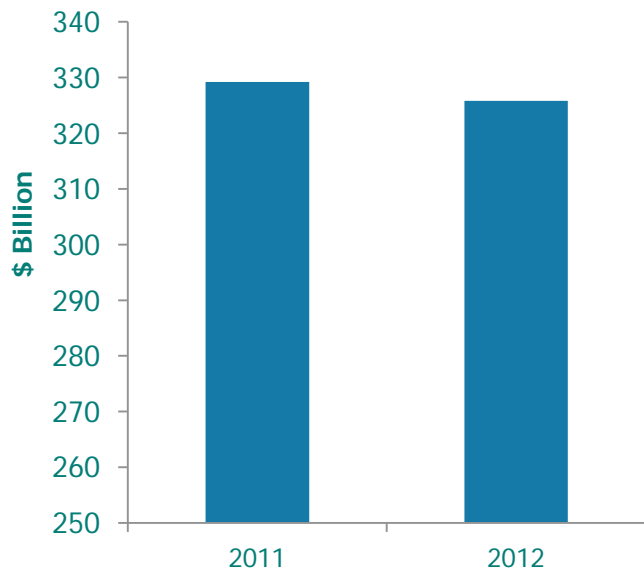
New Medicines Introduced Between 1990 and 1994, Grouped by Tenths, by Lifetime Sales

Note: Prescription drug development costs represent after-tax out-of-pocket costs in 2000 dollars for drugs introduced from 1990–94. The same analysis found that the total cost of developing a new drug was \$1.3 billion in 2006. Average R&D Costs include the cost of the approved medicines as well as those that fail to reach approval.

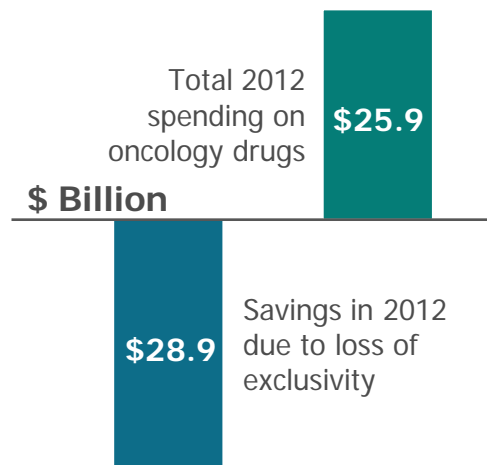
**Source 27:** Vernon JA, Golec JH and DiMasi JA. Drug development costs when financial risk is measured using the Fama-French three-factor model. *Health Economics*. 2009; 002-5.

# Spending on Biopharmaceuticals in Perspective: Savings from Patent Expirations in 2012 Exceeded Entire U.S. Market for Oncology Products

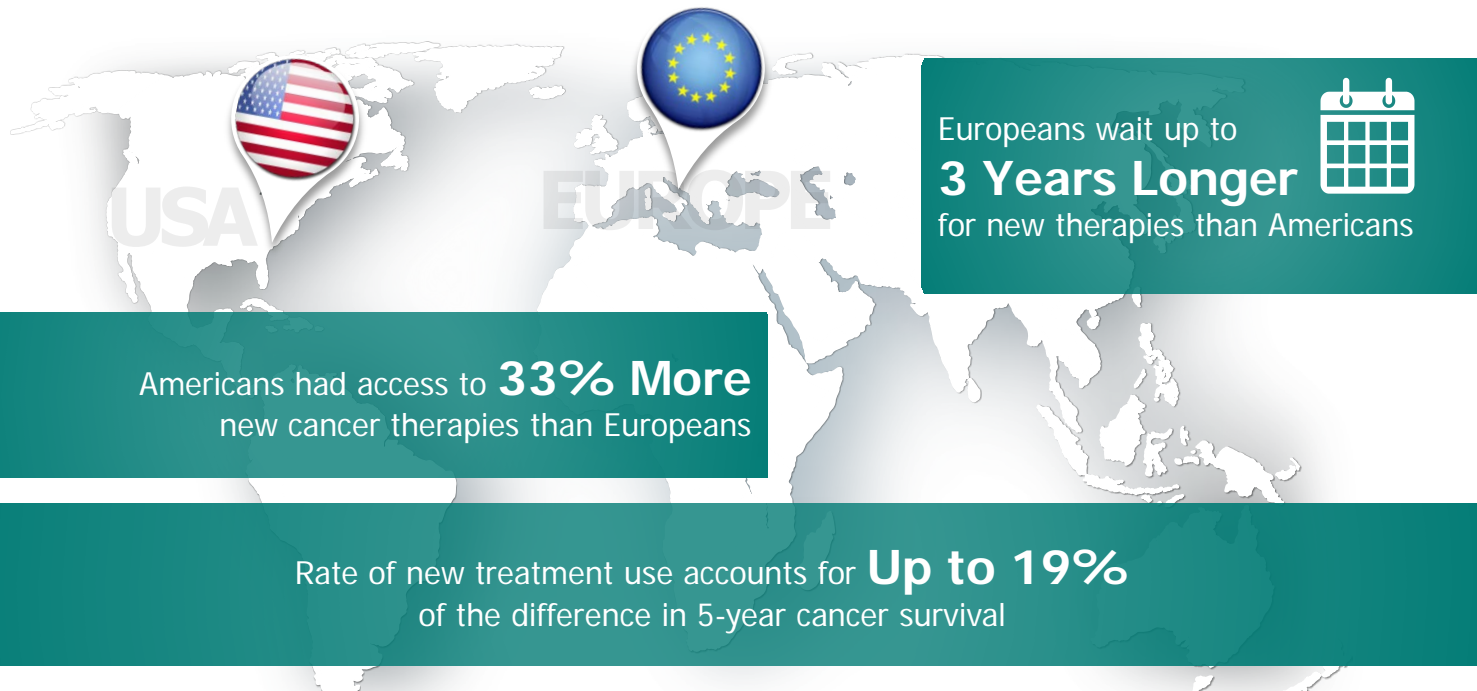
U.S. Prescription Drug Spending  
in 2011 and 2012



2012 U.S. Prescription  
Drug Spending



# Unintended Consequence of the Fourth Hurdle (Reimbursement) on Survival for Cancer Patients



**Source 29:** Tufts Center for the Study of Drug Development. Impact Report. July/Aug 2012. Vol 14, No. 4. Available at [http://csdd.tufts.edu/files/uploads/jul-aug\\_2012\\_ir\\_summary.pdf](http://csdd.tufts.edu/files/uploads/jul-aug_2012_ir_summary.pdf). Accessed 11/14/13. Philipson T, Eber M, Lakdawalla DN, et al. An Analysis of Whether Higher Health Care Spending in the United States Versus Europe is 'Worth It' in the Case of Cancer. Health Affairs. April 2012.

## Investment in Innovation/Treatment Equals Better Patient Outcomes

**U.S.**

Average survival  
**11.1 years**

**EUROPE**

Average survival  
**9.3 years**

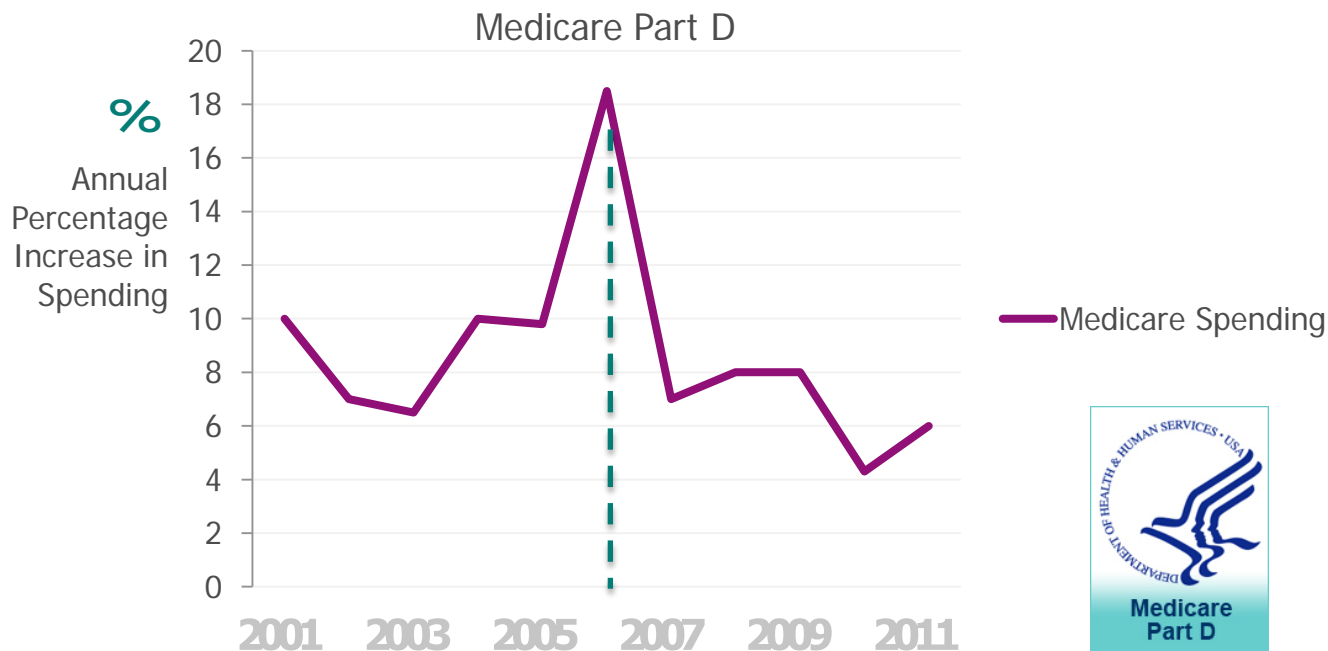
Overall, the U.S.  
generated more than

**\$500 Billion**

additional value for cancer patients,  
net of its higher costs of treatment

“Differences in U.S. costs reflect more rapid uptake of new technologies that may lead to difference in survival.”

# Introduction of Medicare Part D Associated With a Decline in Medicare Spending



**Source 31:** U.S. Centers for Medicare & Medicaid Services, Office of the Actuary, National Health Statistics Group; U.S. Department of Commerce, Bureau of Economic Analysis; and U.S. Bureau of the Census. Available at <http://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/downloads/tables.pdf>. Accessed 11/14/13.

## Congressional Budget Office (CBO) Concludes Increased Use of Novel Therapies Lowers Medicare Spending



Every **1% increase in prescriptions filled** among Medicare beneficiaries ...



... **Lowers Medicare spending** for other health services, such as hospital admissions, physician visits, lab tests or medical equipment, by **0.2%**



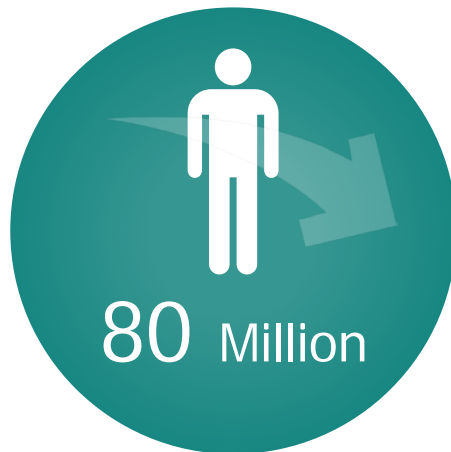
# Potential Cost of Increased Reimbursement Regulation on Innovation



Between 2010-2040:



Decline in R&D for cancer and rare diseases



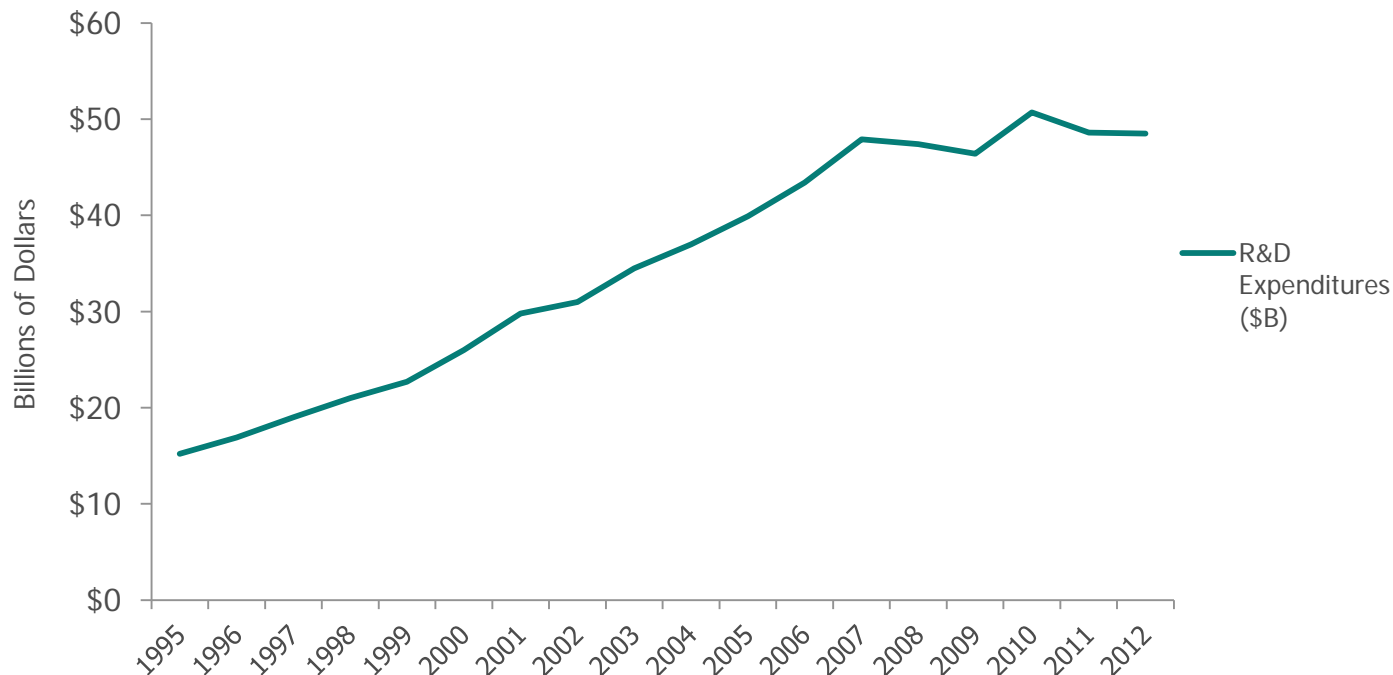
Fewer life years



Lost economic growth potential

**Source 33:** Vernon J and Goldberg R. Comparative Effectiveness Research: Effect on Pharmaceutical Innovation, Value of Health and Longevity. Center for Medicine in the Public Interest. 2011. Available at <http://www.cmpi.org/uploads/File/Comparative-2.pdf>. Accessed 11/15/13.

## Rate of Private Sector R&D Spend Slowing



**Source 34:** Pharmaceutical Research and Manufacturers of America, PhRMA Annual Membership Survey, 1996–2012. National Institute of Health Office of Budget. History of Congressional Appropriations. Available at <http://officeofbudget.od.nih.gov/pdfs/FY08/FY08%20COMPLETED/appic3806%20-%20transposed%20%2090%20-%2099.pdf> (for 1995-1999), [http://officeofbudget.od.nih.gov/pdfs/FY12/Approp.%20History%20by%20IC\)2012.pdf](http://officeofbudget.od.nih.gov/pdfs/FY12/Approp.%20History%20by%20IC)2012.pdf) 122 for 2000-2012). Accessed 7/23/13.



# We Are at a Critical Crossroads for Medical Innovation



## A MAJOR PARADOX

The **potential** of **science** is **greater** than ever ...



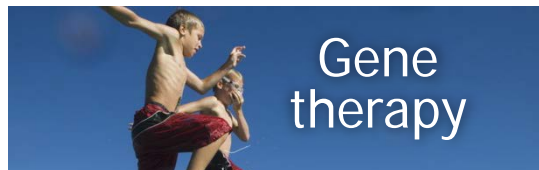
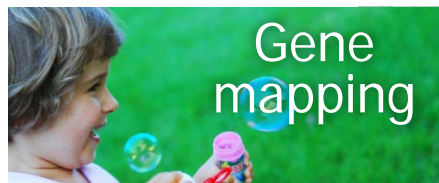
But the outlook for **investment** has never been more **uncertain**



R&D Investment  Longer, Better, Healthier Lives

# What's at Stake ...

Today's investments in healthcare and R&D can **create a world free from cancer** for our children and our children's children.



# Notes and Sources



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17. National Cancer Institute. Cancer costs projected to reach at least \$158 billion in 2020. Available at <http://www.cancer.gov/newscenter/newsfromnci/2011/CostCancer2020>. Accessed 11/15/12. Estimates based on preventing the estimated 1.9 million new cancer cases expected in 2020. Mariotto AB, Yabroff KR, Shao Y, Feuer EJ, and Brown ML. Projections of the Cost of Cancer Care in the United States: 2010-2020. 2011. JNCI, Vol. 103, No. 2.

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A hand is shown holding a glowing lightbulb against a dark background. The lightbulb is illuminated from within, casting a warm glow. The hand is positioned in the upper right quadrant of the frame. The overall image has a dark, moody aesthetic with a teal and yellow color palette.

““ The best way to **predict**  
the **future** is to **invent it.**””

American Computer Scientist  
**Alan Kay**



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