

— 2016 —
MORE THAN
25%

Of New Medicines
Approved by FDA were
Precision Medicines

— 2016 —
MORE THAN
7,000

New Medicines
in Development
Globally



THE VALUE OF MEDICAL INNOVATION IN CANCER 2017 EDITION

— 2015 —
Biopharmaceutical
Industry Sponsored
9,059
CLINICAL TRIALS
AROUND THE WORLD







“ 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.”

Theodore Levitt
American Economist



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INTRODUCTION

Among the technological innovations of the 20th and 21st centuries, medical innovation has been one of the most significant contributions to our ability to live longer, healthier lives.

It is turning knowledge about disease mechanisms at the genetic and cellular level into breakthrough therapies that cure or prevent illness. It brings about a virtuous cycle of better health and greater prosperity which, in turn, stimulates additional investment in even more advanced innovations for preventing and treating disease.







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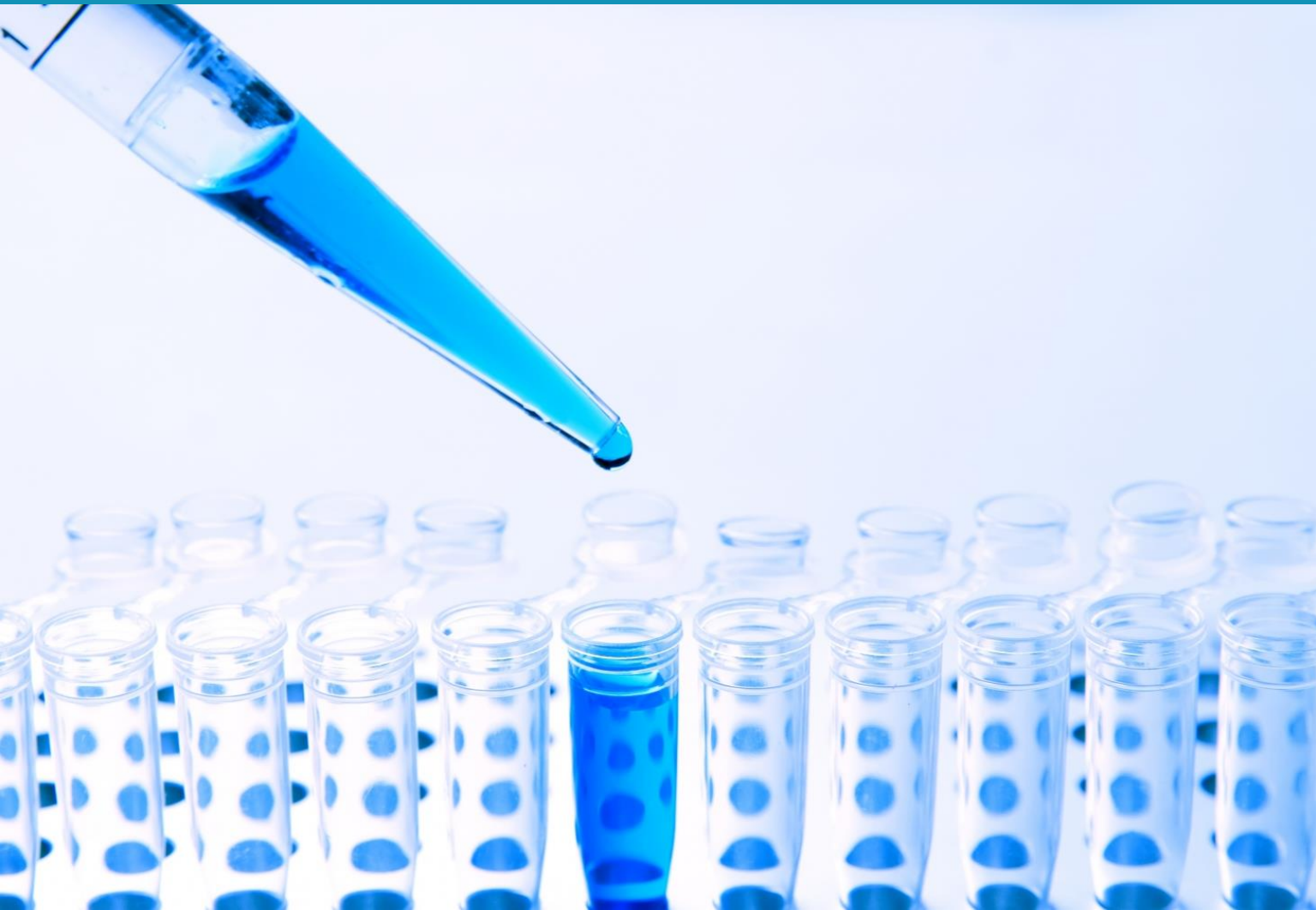
VIRTUOUS CYCLE OF INNOVATION



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

Medical innovation is an important contributor to longer life and economic prosperity. In the 21st century, medical innovation is dramatically improving health outcomes, reducing the overall cost of healthcare and stimulating the growth of the global economy – producing a world that can be free from cancers.

This virtuous cycle of innovation, in turn, stimulates investment in biomedical research to further improve health and create economic value throughout the world.



“ 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.”

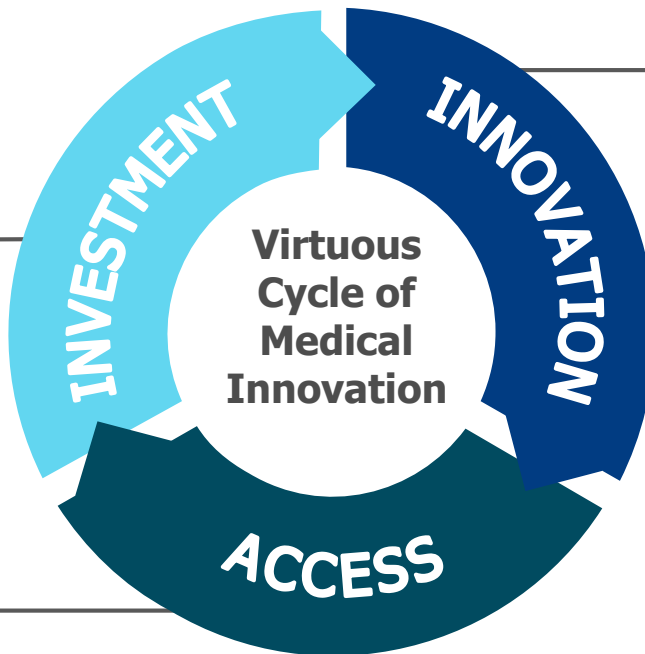
Eric Topol, MD

Author, *The Creative Destruction of Medicine*

Medical Innovation is a Virtuous Cycle

Access and reimbursement for innovation today make possible the investment in research and development that leads to future medical advances

Celgene has invested on average 36 percent of revenue in research and development during the past five years



Continuous investment of time and resources by biopharmaceutical companies such as Celgene leads to new medical breakthroughs

Access and reimbursement for innovative therapies fund investment in future medical advances

The Impact of Medical Innovation



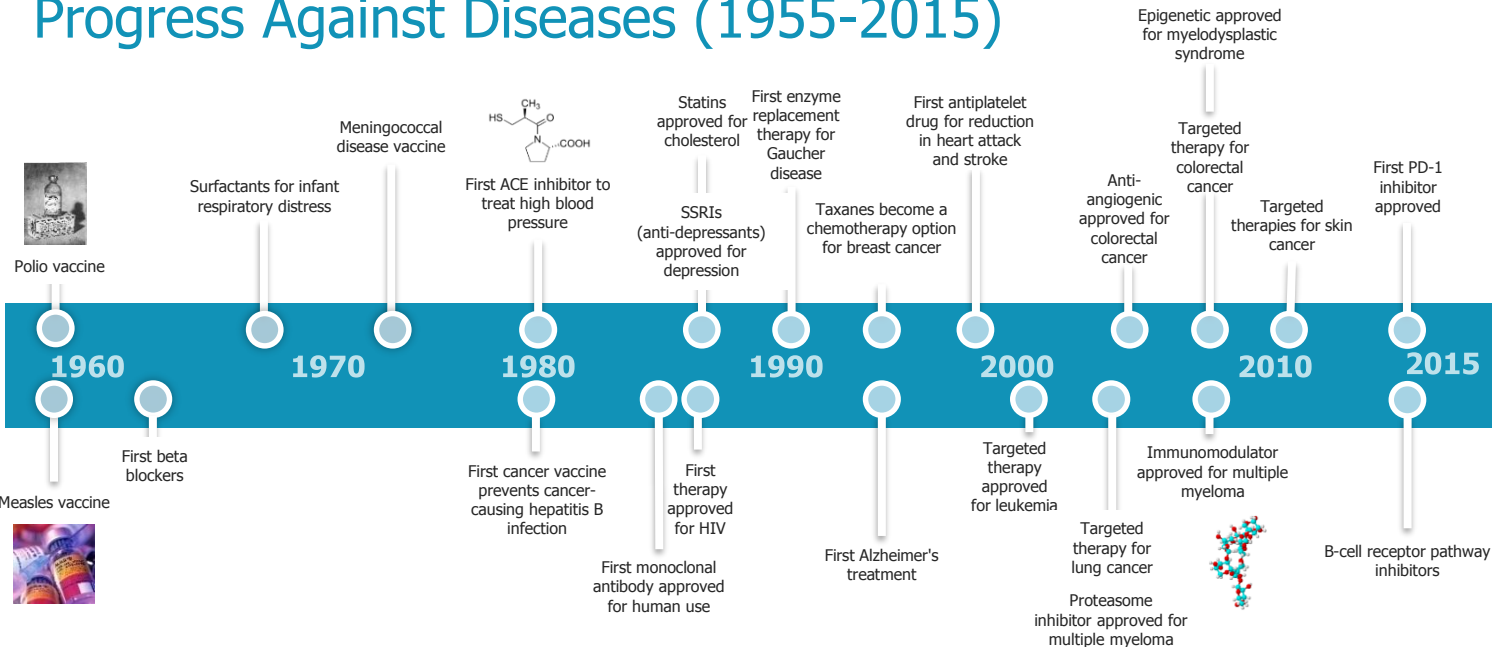
In July 1999, the U.S. Centers for Disease Control and Prevention's *Morbidity and Mortality Weekly Report* noted the death of a 90-year-old woman. She had been deathly ill in 1942, but as a last resort, doctors treated her with what was then called "an obscure, experimental drug."

This 33-year-old woman went from death's door to getting married, raising a family and living nearly 60 more years.

In fact, she was the first U.S. civilian whose life was saved by ... *penicillin*.

Source 1: Centers for Disease Control and Prevention (CDC). Achievements in Public Health, 1900-1999: Control of Infectious Diseases. *MMWR Morb Mortal Wkly Rep* 1999;48(29):621-9. Available at <http://www.cdc.gov/mmwr/preview/mmwrhtml/mm4829a1.htm>. Accessed July 2017.

Progress Against Diseases (1955-2015)



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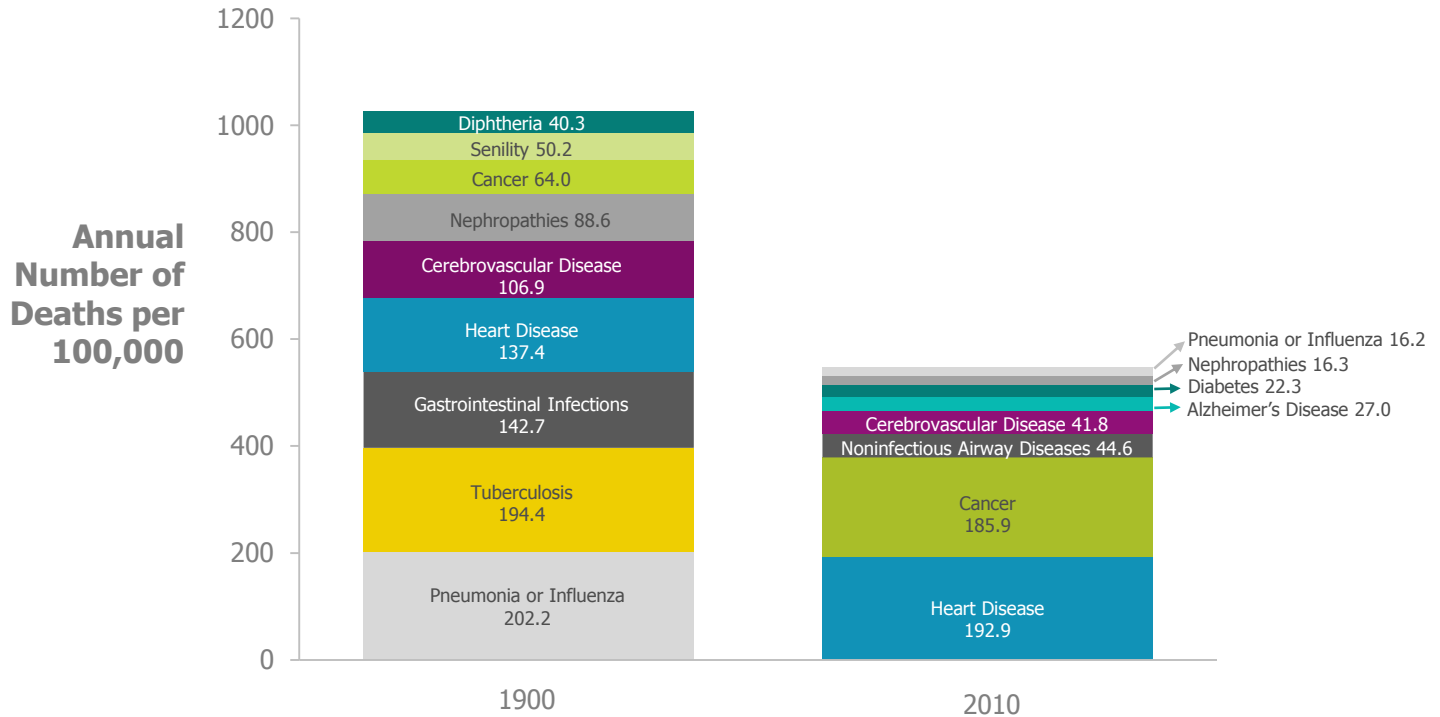
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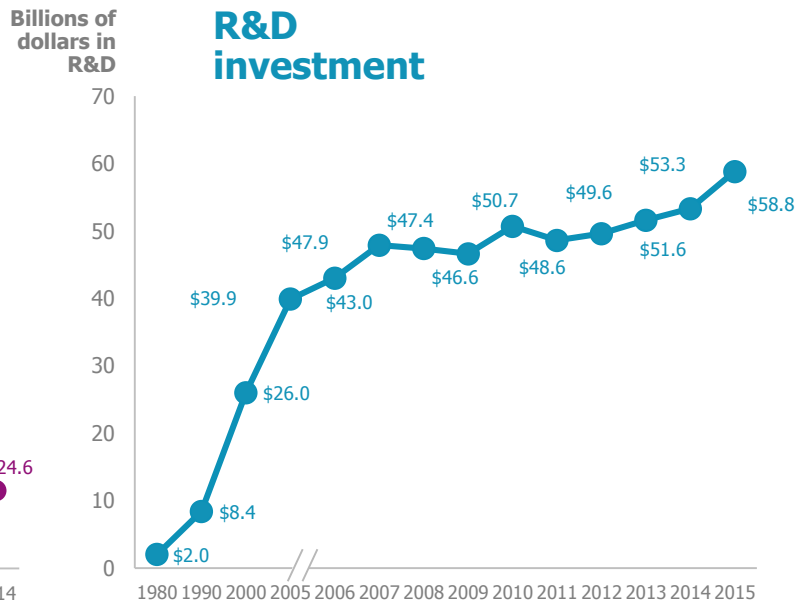
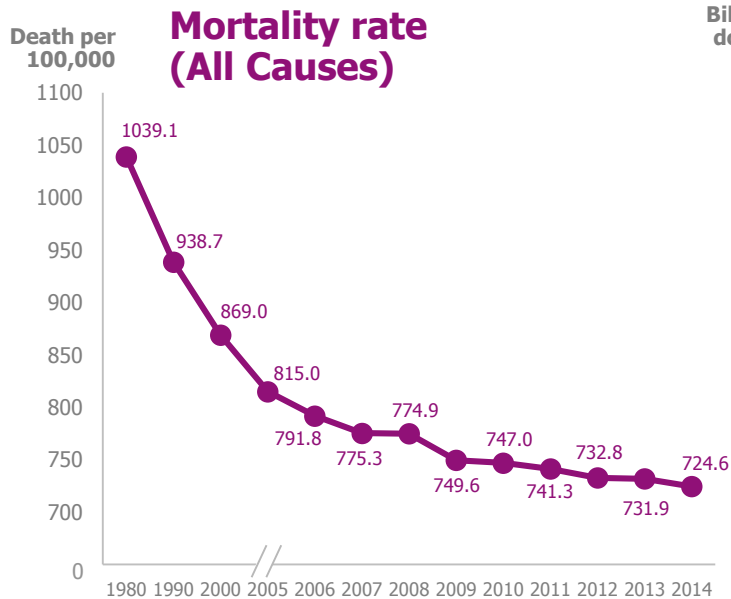


Medical Innovation: Contributing to the Reduction in the Burden of Diseases Around the World



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Mortality Rates Have Fallen; Investment in Healthcare R&D Has Risen*



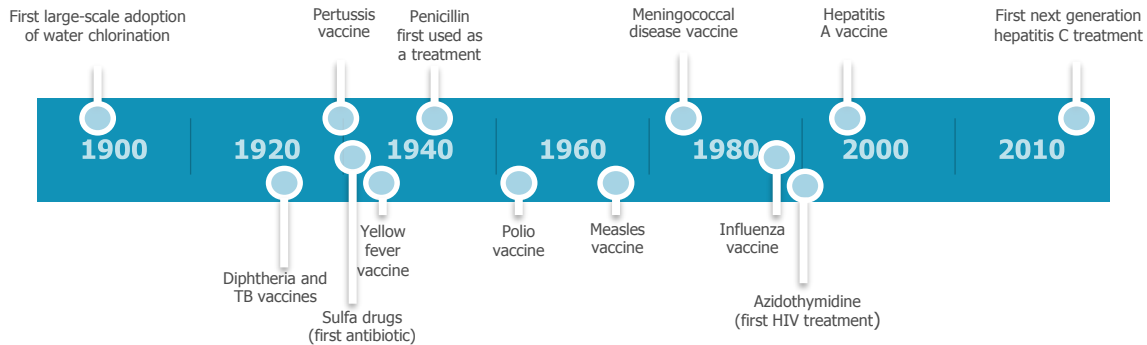
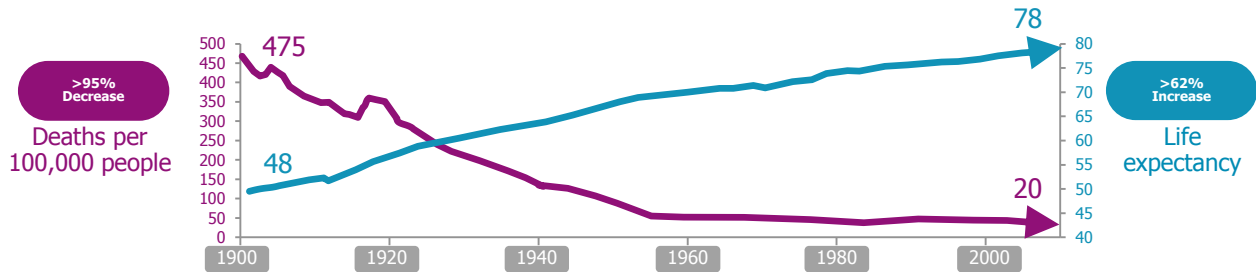
*R&D spend for PhRMA members only

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Advances in Treatment of Infectious Diseases Have Yielded Large Gains in Life Expectancy

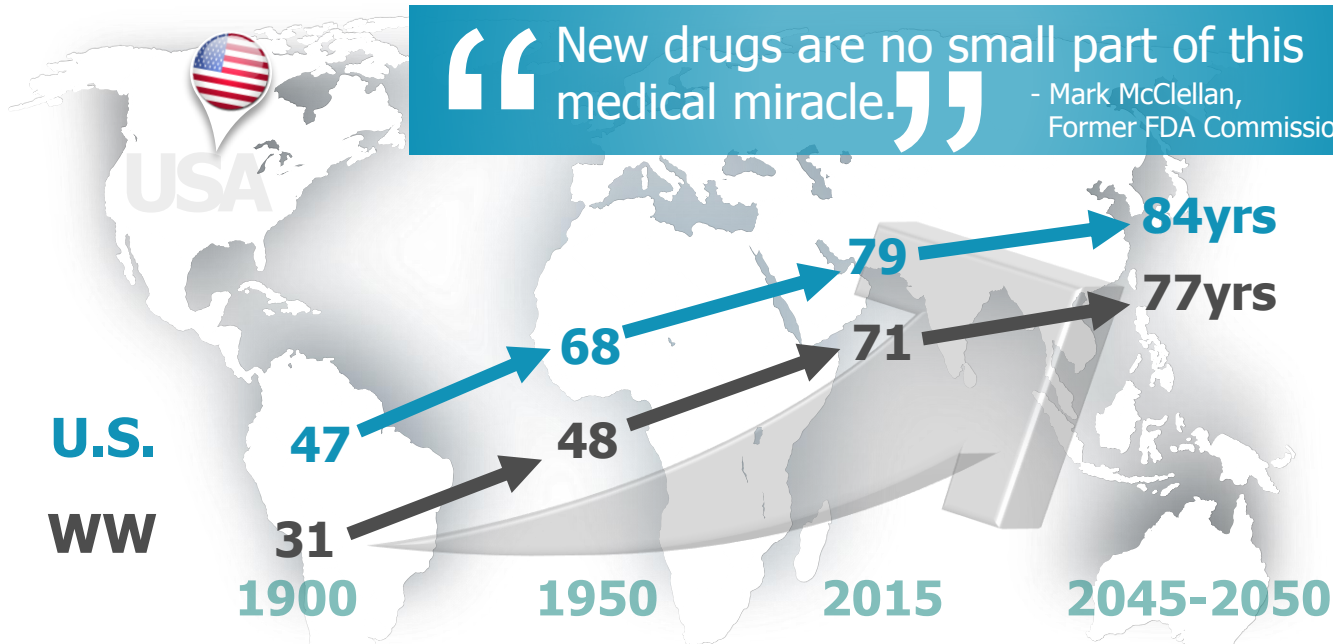


Source 5: The Hamilton Project at the Brookings Institution. Deaths from Major Infectious Diseases. Available at http://www.hamiltonproject.org/multimedia/charts/deaths_from_major_infectious_disease/. Accessed July 2017.

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The Miracle and Gift of Longer Life

“ New drugs are no small part of this medical miracle.” - Mark McClellan, Former FDA Commissioner



Innovative Medicines = Advances in Life Expectancy

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Medical Innovation in Vaccinations: Saving Lives and Cost While Fueling Productivity





Since 1924, vaccinations have **prevented 103 million cases of childhood infection**, representing ~95% of infections that would have occurred, **including 26 million in the last decade alone.**



According to the World Health Organization (WHO)

immunizations **save**
AN ESTIMATED 2.5 MILLION CHILD LIVES EVERY YEAR

	In the U.S., vaccine use saves \$13.5 billion in direct costs.		Globally, vaccinations help avoid long-term costs of lost productivity due to disability and death , which may add savings of around \$61 billion.
<p>“ I strongly believe in the virtues of vaccination ... to allow my country to have a better economic future. Healthy children are more likely to attend schools and become economically productive adults. ”</p> <p>- President Ibrahim Boubacar Keita, Republic of Mali 2015</p>			

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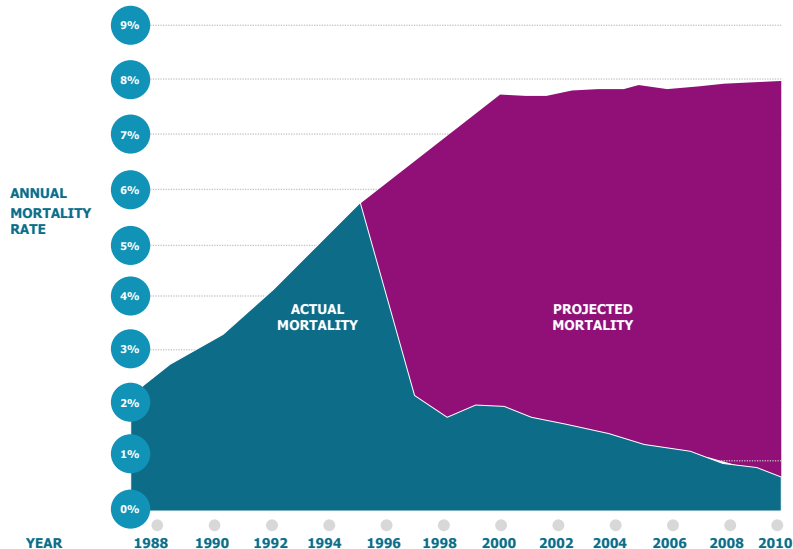
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Value of Medical Innovation in HIV

ACTUAL VS PROJECTED DEATH RATES FOR HIV/AIDS IN THE UNITED STATES



"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

~81%
decline in people
dying from HIV*

*Between 1995-2015



4 HIV Therapies

Approved by the FDA between late 1995 and early 1996

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Innovative HIV Treatment Lowers Prevalence and Increases Life Expectancy



A 2017 study found that **20-year-olds** who started with **antiretroviral therapy** in 2013 are predicted to **live up to 10 years longer** than those who first underwent similar treatment in 1996 – when it first became widely available.



The treatment also prevents spread of the disease, by **as much as 96%**, in couples where one partner has HIV.

“ Our research illustrates a success story of how **improved HIV treatments** coupled with screening, prevention and treatment of health problems associated with HIV infection can **extend the life span** of people diagnosed with HIV. ”

- Adam Trickey,
Medical Statistician at the University of Bristol
2017

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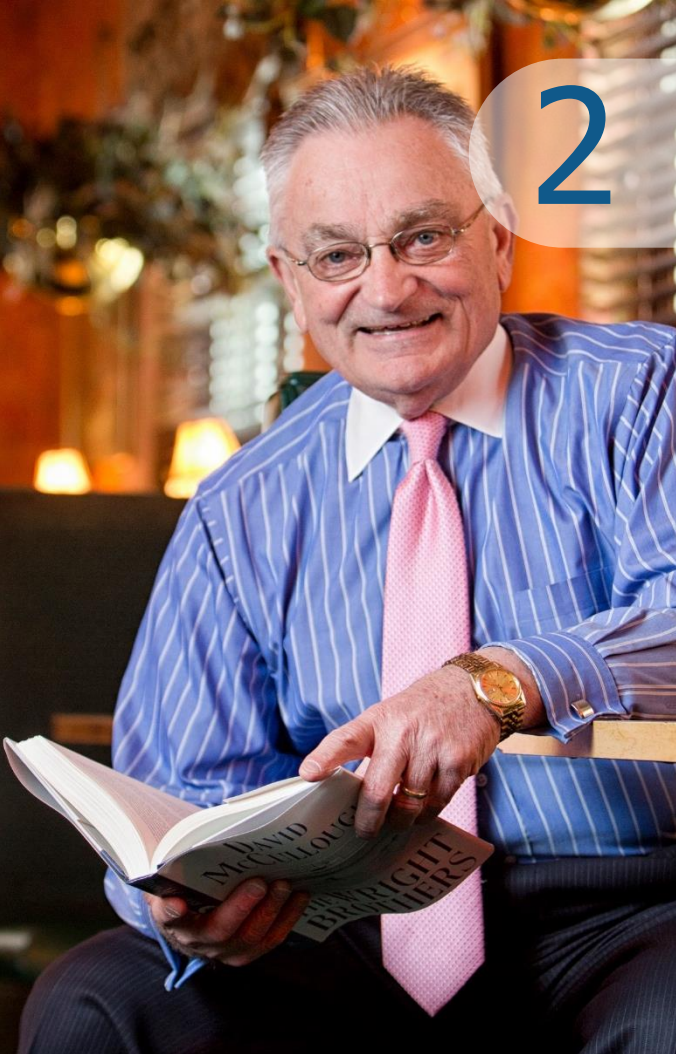
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2

LIVING LONGER, BETTER AND HEALTHIER



RECENT MEDICAL INNOVATIONS IN CANCER ARE LEADING TO LONGER LIFE, BETTER HEALTH AND IMPROVED QUALITY OF LIFE.

This section will show what has been accomplished over the last few decades. There has been substantial progress in helping some people become free from cancers – allowing them to live longer, healthier lives.

Innovative cancer treatments have been shown to contribute significantly to survival gains in cancer. In addition, as with other diseases, some people treated for cancer can live and prosper as well as someone without the illness.



“

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.

”

Dr. Siddhartha Mukherjee

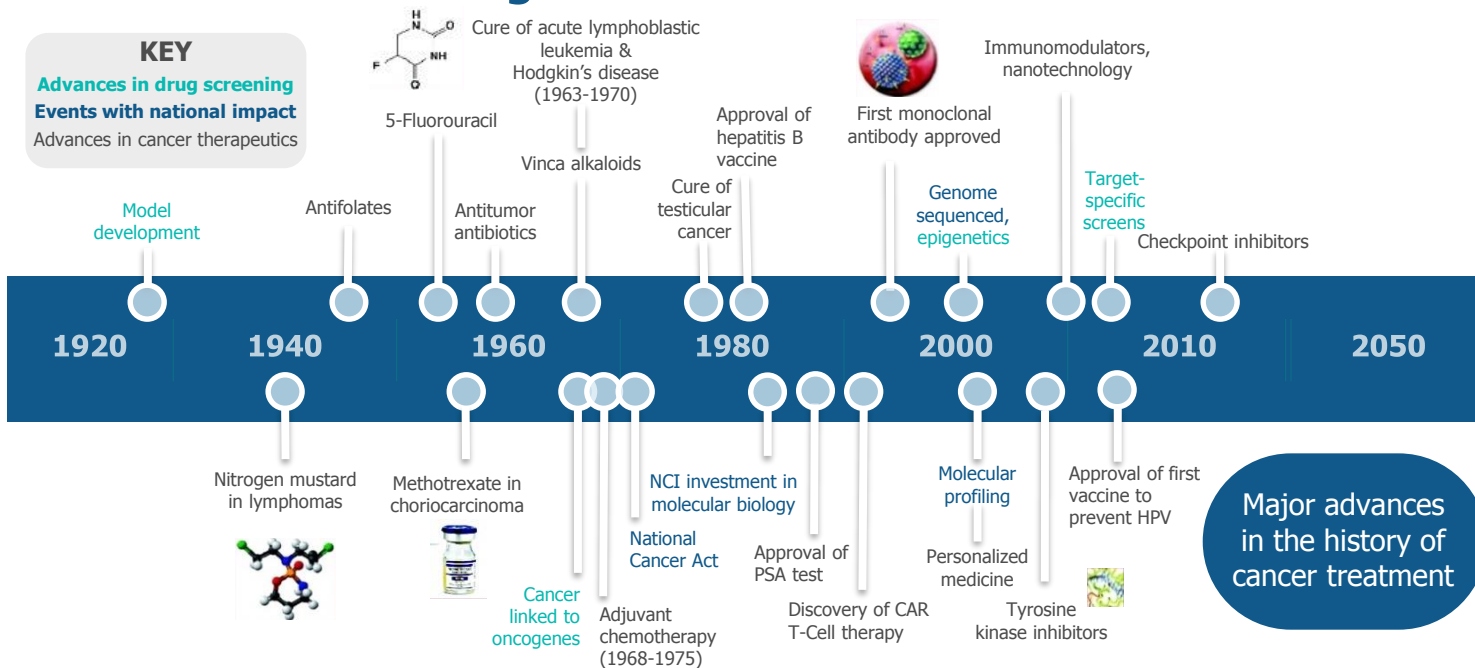
Author, *The Emperor of All Maladies*
2011

Milestones in War Against Cancer in the U.S.

KEY

Advances in drug screening
Events with national impact

Advances in cancer therapeutics



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Source 1 e: U.S. Food and Drug Administration. June 12, 2008 Approval Letter Human Papillomavirus Quadrivalent (Types 6, 11, 16, 18) Vaccine, Recombinant. Available at <https://www.fda.gov/BiologicsBloodVaccines/Vaccines/ApprovedProducts/ucm111282.htm>. Accessed November 2017.

Source 1 f: National Cancer Institute. The Abraxane® Story: A Nanoparticle Platform Delivers Improved Anticancer Activity. Available at http://nano.cancer.gov/action/news/nanotech_news_2006-01-30e.asp. Accessed August 2017.

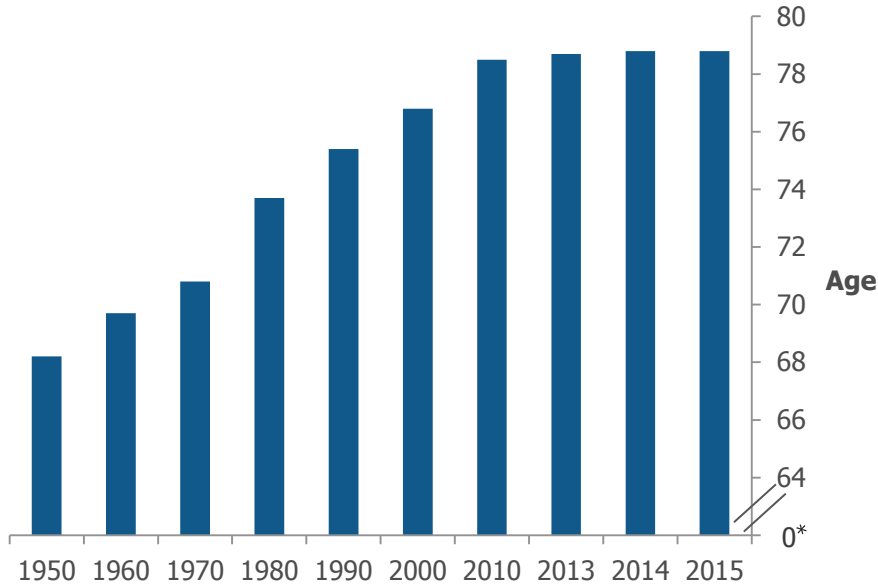
Source 1 g: Alexander W. The Checkpoint Immunotherapy Revolution. *P&T Journal*. Available at <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4771089/>. Accessed November 2017.

Source 1 h: Chaudhary P M. Dr. Chaudhary on History of CAR T-Cell Therapy. *OncoLive*. Available at <http://www.onclive.com/printer?url=onclive-tv/dr-chaudhary-on-history-of-car-tcell-therapy>. Accessed October 2017.

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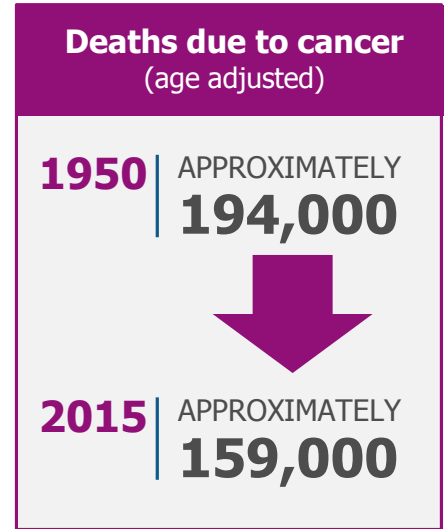


Medical Progress: A Driver in Increased Life Expectancy



* y-axis skips from 0 to 64, and then continues in intervals of 2 from there on

■ Life Expectancy at Birth



Source 2: National Vital Statistics Report. Centers for Disease Control and Prevention and National Center for Health Statistics. Table 22: Life expectancy at birth, at age 65, and at age 75, by sex, race, and Hispanic origin: United States, selected years 1900-2010. Available at https://www.cdc.gov/nchs/data/nvsr/nvsr65/nvsr65_04.pdf. Accessed August 2017.

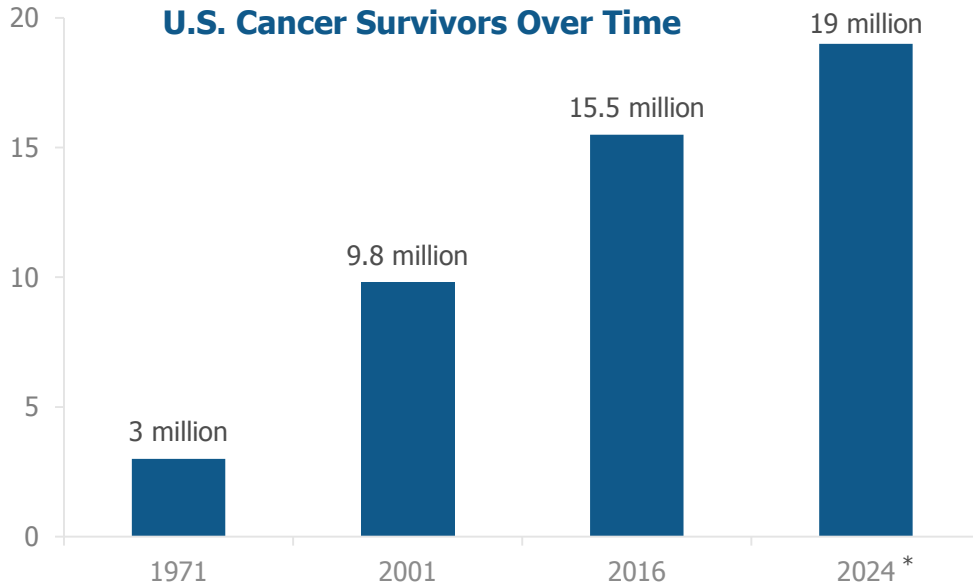
Source 2 a: Centers for Disease Control. Table 17. Age Adjusted Death Rates 1950 – 2014. Available at <https://www.cdc.gov/nchs/data/aus/2015/017.pdf>. Accessed August 2017.

Source 2 b: Mortality in the United States, 2015. Available at <https://www.cdc.gov/nchs/data/databriefs/db267.pdf>. Accessed August 2017.

Source 2 c: Health United States, 2016. Available at <https://www.cdc.gov/nchs/data/aus/aus16.pdf#019>. Accessed August 2017.

The Number of Cancer Survivors is Steadily Rising

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



*2024 data is estimated

Source 3: American Cancer Society. Cancer Treatment and Survivorship Facts & Figures, 2017. Available at <http://www.cancer.org/acs/groups/content@research/documents/document/acspc-042801.pdf>. Accessed November 2017.

Source 3 a: Centers for Disease Control and Prevention, "Cancer Survivors-United States, 2007," 10 March 2011,

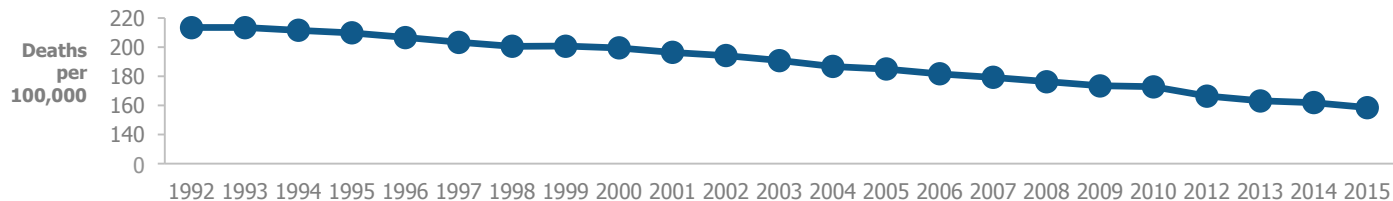
http://www.cdc.gov/cancer/survivorship/what_cdc_is_doing/research/survivors_article.htm. R Siegel, et al. Cancer Treatment and Survivorship Statistics, 2012.

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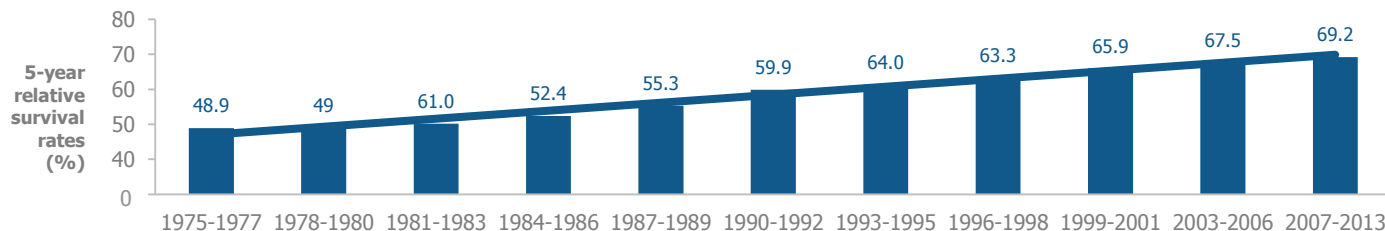
Source 3 b: DeSantis CE. Cancer Journal for Clinicians. Cancer treatment and survivorship statistics, 2014. Available at <http://onlinelibrary.wiley.com/doi/10.3322/caac.21235/full>. Accessed August 2017.



Cancer-Related Death Rates are Declining



5-Year Relative Survival Rates of Cancer Patients are Increasing



Source 4: Centers for Disease Control and Prevention (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 July 2017.

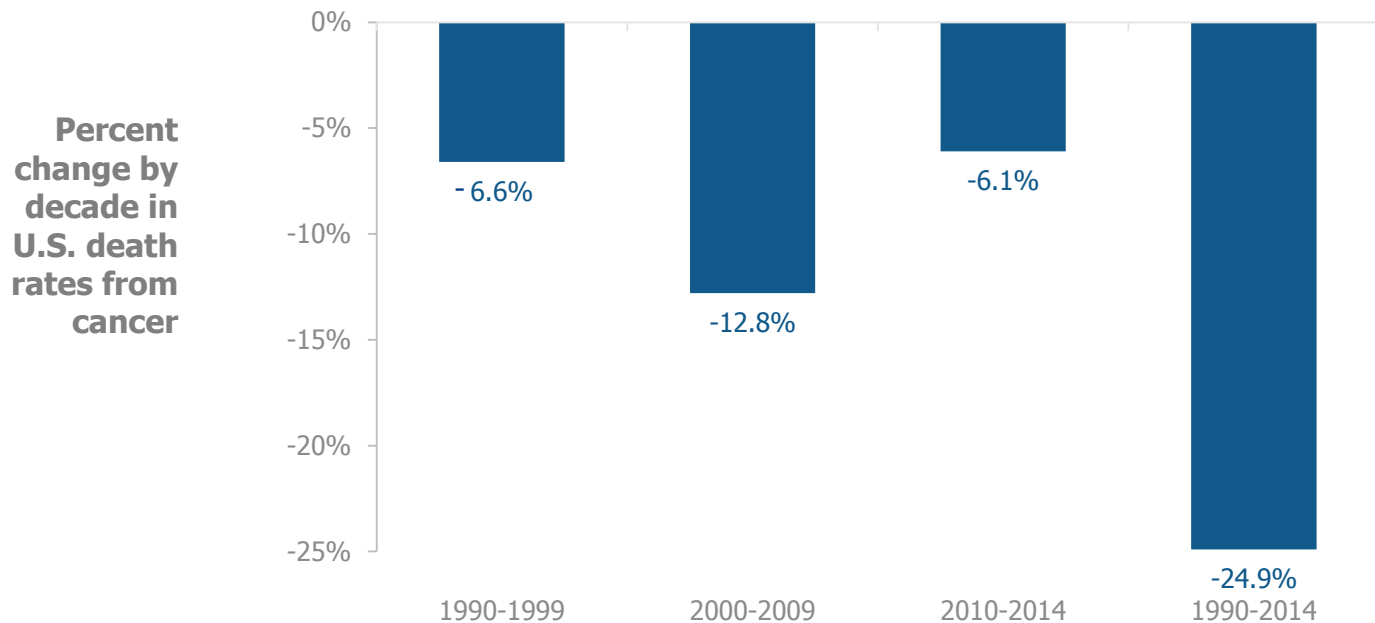
Source 4 a: National Cancer Institute, Surveillance, Epidemiology, and End Results (SEER) Program. SEER Cancer Statistics Review, 1975–2014. 5-Year Relative and Period Survival (Percent) by Race, Diagnosis Year, Stage and Age. Available at https://seer.cancer.gov/csr/1975_2014/results_merged/topic_survival.pdf. Accessed August 2017.

Source 4 b: Centers for Disease Control and Prevention (CDC). Health, United States, 2014. Available at <https://www.cdc.gov/nchs/data/hs/2014/026.pdf>. Accessed July 2017.

Source 4 c: Centers for Disease Control and Prevention (CDC). Health, United States, 2016. Available at <http://www.cdc.gov/nchs/hs/contents2016.htm#024>. Accessed July 2017.

Steady Decline in Cancer Death Rates

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

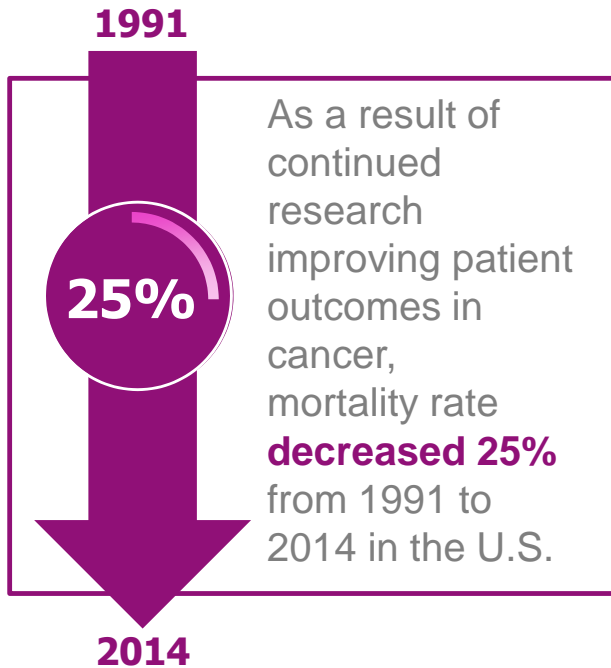


Source 5: Pharmaceutical Research and Manufacturers of American. Biopharmaceuticals in Perspective chart pack. Available at <http://phrma-docs.phrma.org/files/dmfile/chart-pack-biopharmaceuticals-in-perspective4.pdf>. Accessed August 2017. Calculated through National Cancer Institute, Surveillance, Epidemiology, and End Results Program. Number of new cases and deaths per 100,000 people (all races, males and females), age-adjusted. <http://seer.cancer.gov/statfacts/html/ld/all.html>. Accessed April 2016. Accessed on August 2017.

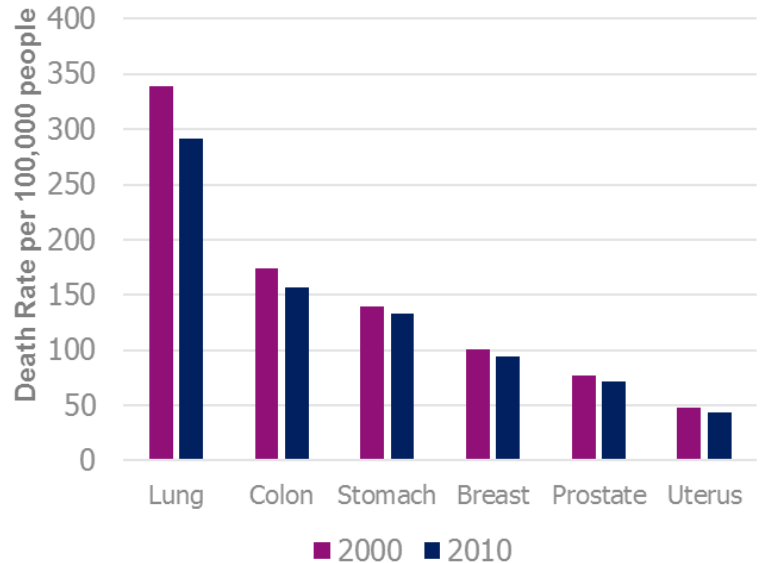
Source 5 a: American Cancer Society. Cancer Treatment and Survivorship Facts & Figures, 2017. Available at <http://www.cancer.org/acs/groups/content@research/documents/document/acspc-042801.pdf>. Accessed November 2017.



Globally, Death Rates Across Common Cancer Sites Have Declined



Mortality is Decreasing Across Common Cancer Sites: 2000 - 2010

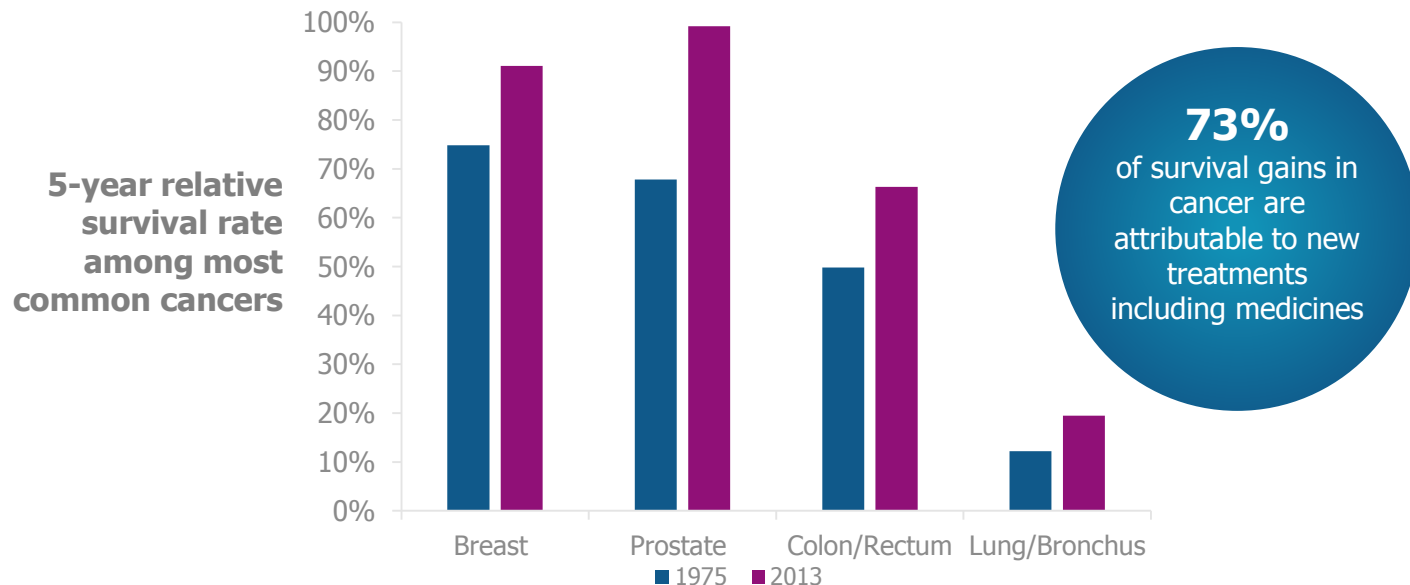


Source 6: American Cancer Society. Cancer Facts and Figures: Death Rate Down 25% Since 1991. Available at <https://www.cancer.org/content/dam/cancer-org/research/cancer-facts-and-statistics/annual-cancer-facts-and-figures/2017/cancer-facts-and-figures-2017.pdf>. Accessed August 2017.

Source 6 a: Hashim D et al. The global decrease in cancer mortality: trends and disparities. Annals of Oncology. Available at <https://academic.oup.com/annonc/article/27/5/926/2769784/The-global-decrease-in-cancer-mortality-trends-and>. Accessed August 2017.

Five-Year Survival is Increasing for Many Types of Cancer Since 1975

The chances that a cancer patient will live 5 years or more has increased on average by 41% across all cancers.

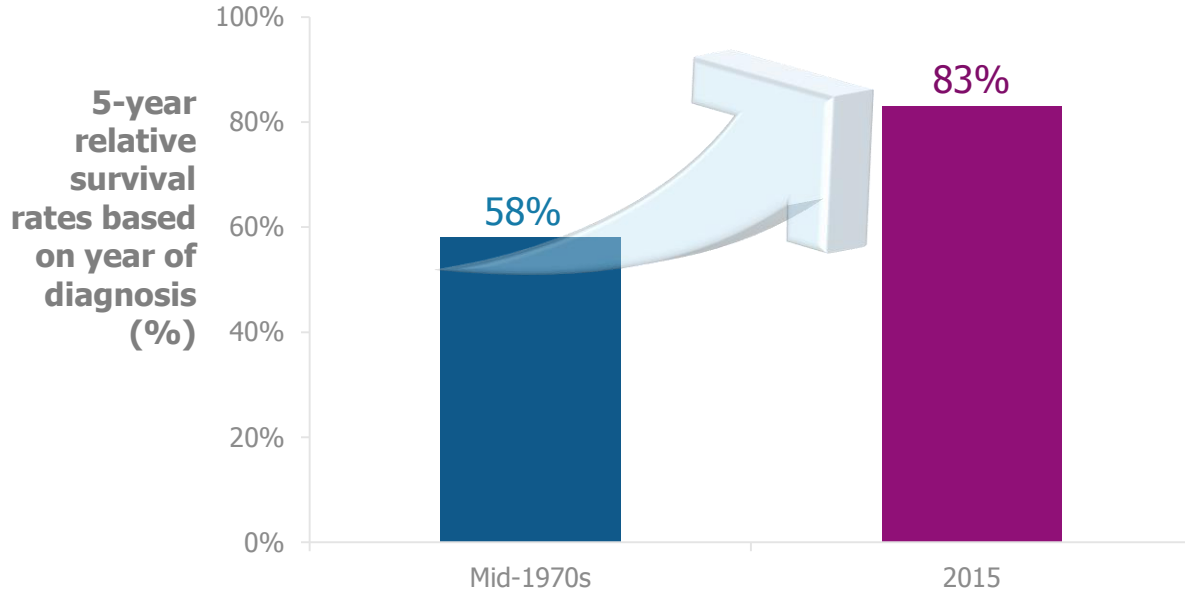


Source 7: National Cancer Institute, Surveillance, Epidemiology, and End Results (SEER) Program. SEER Cancer Statistics Review, 1975-2014. Table 1.5 Age-Adjusted SEER Incidence and U.S. Death Rates and 5-Year Relative Survival (Percent) By Primary Cancer Site, Sex and Time Period. Available at https://seer.cancer.gov/csr/1975_2014/results_merged/topic_survival.pdf. Accessed October 2017.

Source 7 a: American Cancer Society Cancer Facts and Figures 2016. Available at <https://www.cancer.org/content/dam/cancer-org/research/cancer-facts-and-statistics/annual-cancer-facts-and-figures/2016/cancer-facts-and-figures-2016.pdf>. Accessed October 2017.

Source 7 b: Seabury, "Quantifying Gains in the War on Cancer Due to Improved Treatment and Earlier Detection," Forum for Health Economics and Policy 2016; 19(1): 141-156 that states. Accessed August 2017.

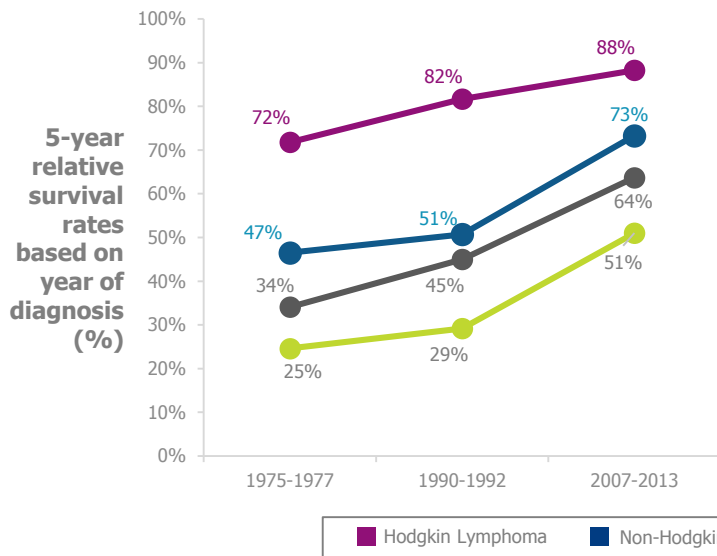
Survival Rates for Childhood Cancers Have Increased 43% Over the Last Several Decades



Source 8: American Cancer Society, "Cancer facts & Figures 2016," Available at <https://www.cancer.org/content/dam/cancer-org/research/cancer-facts-and-statistics/annual-cancer-facts-and-figures/2016/cancer-facts-and-figures-2016.pdf>. Accessed August 2017.

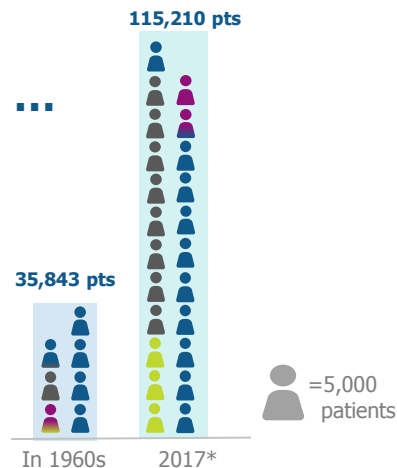
Dramatic Survival Gains Have Been Achieved in Hematology Over the Past Half Century

Survival Trends in Hematology



The Value ...

Patients surviving at 5 years (today's incident population)



* 2017 Data is Estimated

Source 9: National Cancer Institute, Surveillance, Epidemiology, and End Results (SEER) Program. SEER Cancer Statistics Review, 1975-2014. 5-Year Relative and Period Survival (Percent) by Race, Diagnosis Year, Stage and Age. Available at https://seer.cancer.gov/csr/1975_2014/results_merged/topic_survival.pdf. Accessed August 2017.

Source 9 a: American Cancer Society. Cancer Statistics and Figures, 2017. Available at <https://www.cancer.org/content/dam/cancer-org/research/cancer-facts-and-statistics/annual-cancer-facts-and-figures/2017/cancer-facts-and-figures-2017.pdf>. Accessed August 2017.

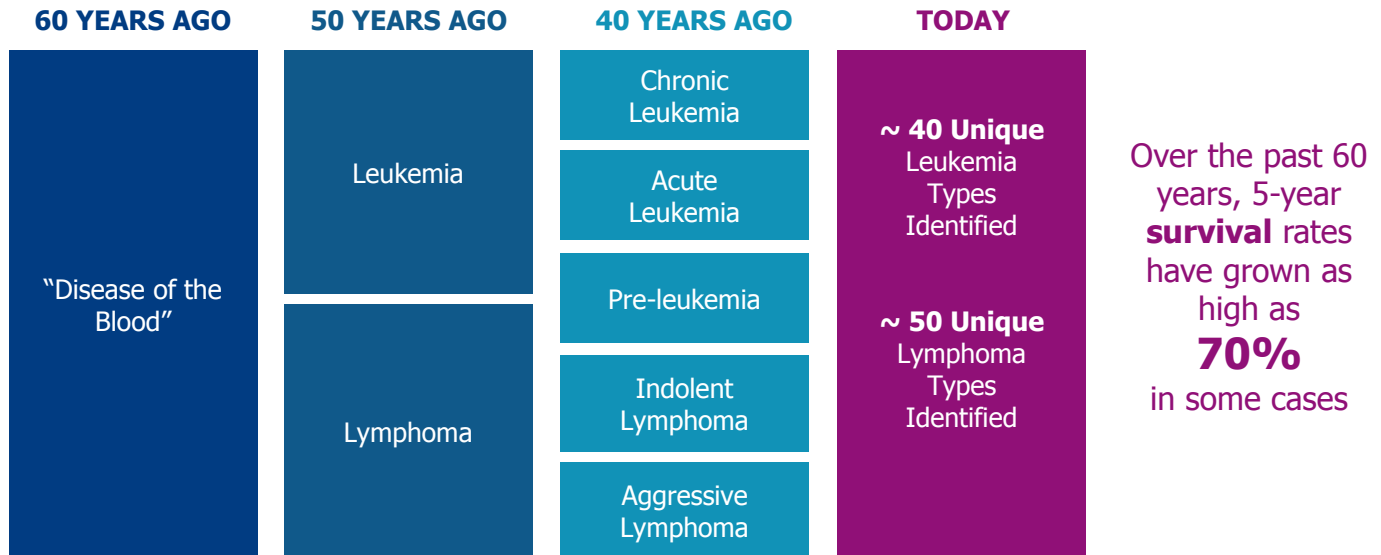
Source 9 b: Bastian, A. The Rising Cost of Medical Care: Understanding the Problem and Exploring Solutions. GFK Market Access. Available at <https://ash.confex.com/ash/2014/webprogram/Session5387.html>. Accessed November 2017.



Medical Advances and More Precise Treatment Have Transformed Cancer Diagnosis



A greater understanding of the molecular basis of disease has transformed what was once known collectively as “disease of the blood,” into multiple subtypes of leukemias and lymphomas, opening up new treatment approaches.



Source 10: M Aspinal, former President Genzyme Genetics (http://www.comtecmed.com/biomarker/2014/Uploads/Editor/PDF/ppt/Edward%20Abrahams_Key%20Note%20Lecture.pdf); National Cancer Institute,; SEER Cancer Statistics Review, 1975-2011. Available at http://seer.cancer.gov/csr/1975_2011/, based on November 2013 SEER data submission, posted to the SEER web site, April 2014; PhRMA, “Medicines in Development for Leukemia & Lymphoma,” April 2015 (all sites accessed May 2015). Accessed August 2017.



“ 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

The Future is Bright for Patients with Myeloma



Susie Novis

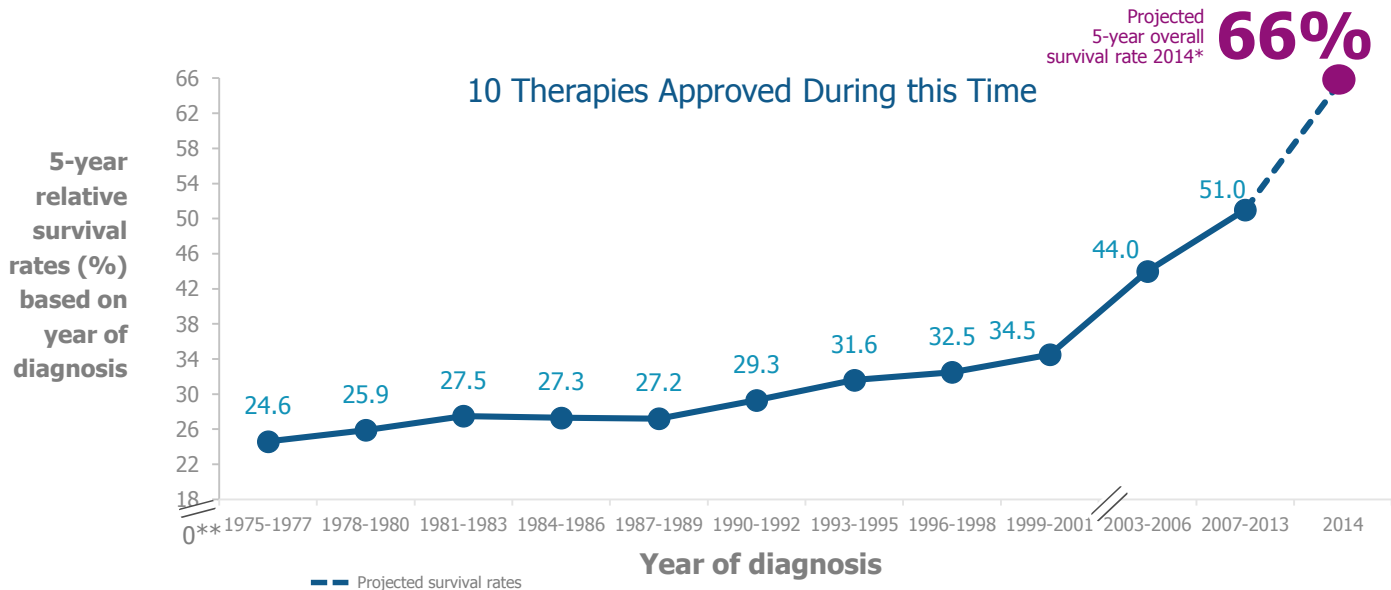
President and Co-Founder
International Myeloma Foundation

“...We are very close to making myeloma a chronic and manageable disease. Myeloma is a small disease with a big impact on the way we treat cancer.”



Source 11: International Myeloma Foundation. The International Myeloma Foundation Says New Studies Show Longer Survival for Myeloma Patients and Improved Response to Treatments. Available at <http://www.businesswire.com/news/home/20080531005029/en/International-Myeloma-Foundation-New-Studies-Show-Longer>. Accessed September 2017.

Relative Survival Rate for Multiple Myeloma Patients Soars Between 2001 and 2014



* Based on recent trends in the 5-year relative survival rate, for myeloma patients diagnosed in 2014, the relative overall survival rate may have reached as high as 66%

** y-axis skips from 0 to 22 and then continues in intervals of 4 from there on

Source 12: National Cancer Institute, Surveillance, Epidemiology, and End Results (SEER) Program. SEER Cancer Statistics Review, 1975-2014. 5-Year Relative and Period Survival (Percent) by Race, Diagnosis Year, Stage and Age. Available at https://seer.cancer.gov/csr/1975_2014/results_merged/topic_survival.pdf. Accessed August 2017.

Source 12 a: Bergsagel P. Where We Were, Where We Are, Where We Are Going: Progress in Multiple Myeloma. ASCO 2014 Educational Book. Available at <http://meetinglibrary.asco.org/content/114000199-144>. Accessed July 2017.

Source 12 b: National Cancer Institute. Drugs Approved for Multiple Myeloma and Other Plasma Cell Neoplasms. Available at <https://www.cancer.gov/about-cancer/treatment/drugs/multiple-myeloma>. Accessed September 2017.

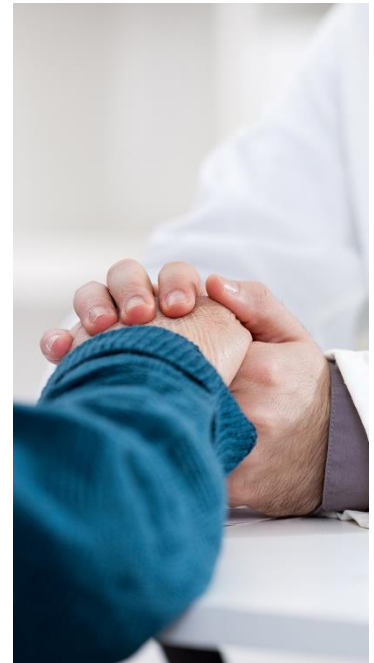


Advancements in Multiple Myeloma Therapies Have the Potential to Increase Quality of Life



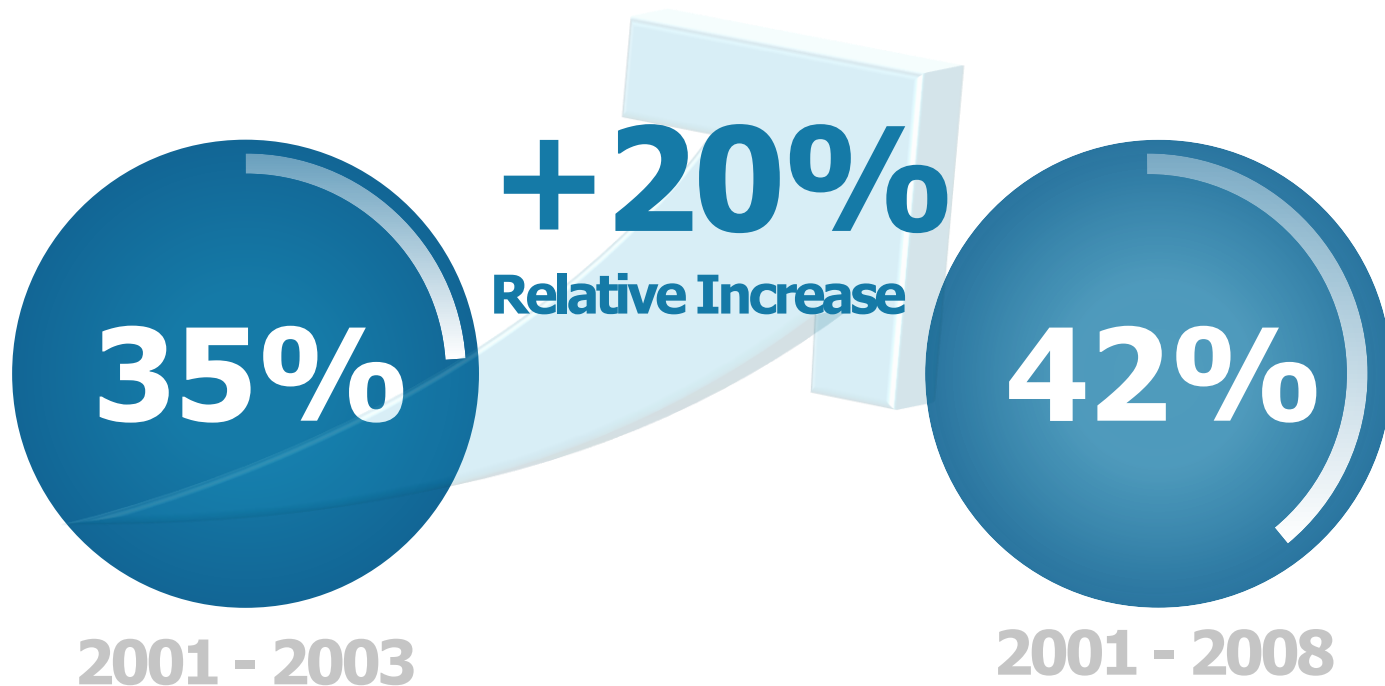
Michel Delforge, MD, PhD
University Hospital Leuven
in Belgium

“ We believe that this improvement in health-related quality of life with [novel therapies] and specifically the reduction in treatment-related side effects, may facilitate patient adherence. Longer treatment duration and better adherence may improve outcomes for the patient. ”



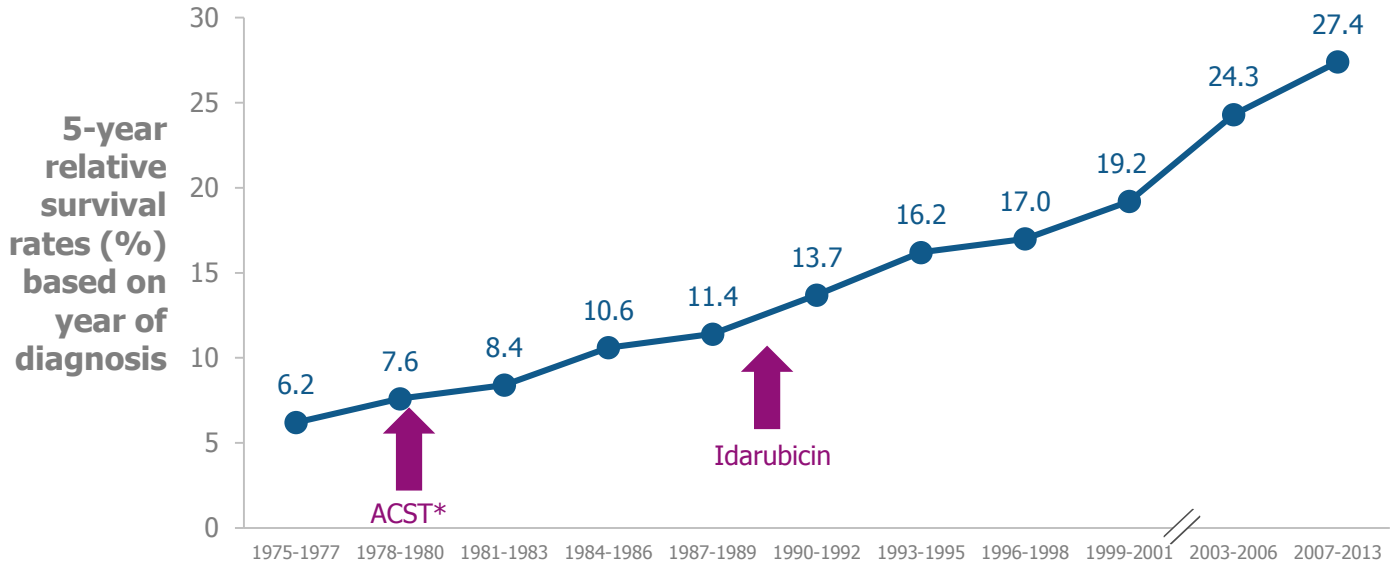
Source 13: Harrison C. Quality of Life: An Increasingly Important Feature of Myeloma Treatment. Cancer Therapy Advisor. Available at <http://www.cancertherapyadvisor.com/side-effect-management/multiple-myeloma-quality-life-more-important-feature-treatment/article/547105/2/>. Accessed August 2017.

Three-year Survival Rates of Patients with Myelodysplastic Syndromes (MDS) Have Seen an Increase



Source 14: Ma, X. Myelodysplastic syndromes: incidence and survival in the United States. *Cancer*. 2007 Apr 15;109(8):1536-42.
Source 14 a: Ma X. Epidemiology of Myelodysplastic Syndromes. *Am J Med*. 2012 Jul; 125(7 Suppl): S2-S5.

Patient Survival in Acute Myeloid Leukemia (AML) Continues to Increase



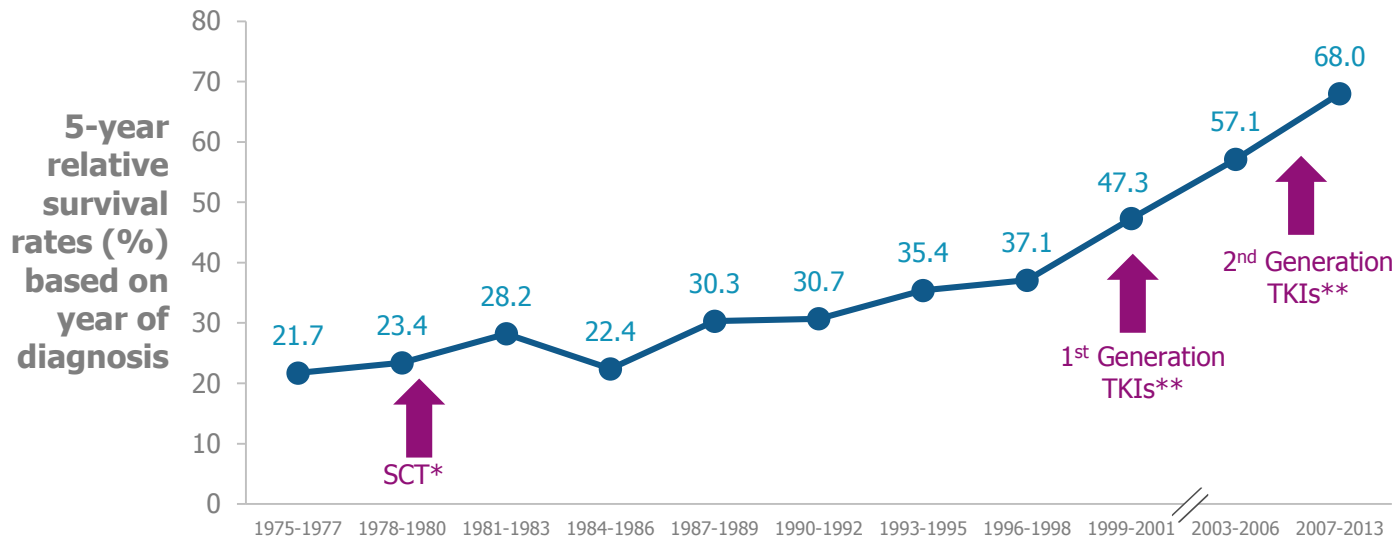
*ACST = allogeneic stem-cell transplantation for patients with AML

Source 15: National Cancer Institute, Surveillance, Epidemiology, and End Results (SEER) Program. SEER Cancer Statistics Review, 1975-2010. Table 13.16: Myeloid Leukemia, 5-Year Relative and Period Survival (Percent) by Race, Sex, Diagnosis Year and Age. Available at http://seer.cancer.gov/archive/csr/1975_2010/results_merged/sect_13_leukemia.pdf. Accessed August 2015.

Source 15 a: National Cancer Institute, Surveillance, Epidemiology, and End Results (SEER) Program. SEER Cancer Statistics Review, 1975-2014. 5-Year Relative and Period Survival (Percent) by Race, Diagnosis Year, Stage and Age. Available at https://seer.cancer.gov/csr/1975_2014/results_merged/topic_survival.pdf. Accessed August 2017.

Source 15 b: Freireich EJ. Et al. The Leukemias: A Half-Century of Discovery. Journal of Clinical Oncology. Available at <http://ascopubs.org/doi/pdf/10.1200/JCO.2014.57.1034>. Accessed October 2017.

Chronic Myeloid Leukemia (CML) Patients Benefiting from Medical Innovation



* SCT = allogeneic stem cell transplant

** TKIs = Tyrosine Kinase Inhibitors

Source 16: National Cancer Institute, Surveillance, Epidemiology, and End Results (SEER) Program. SEER Cancer Statistics Review, 1975-2010. Table 13.16: Myeloid Leukemia, 5-Year Relative and Period Survival (Percent) by Race, Sex, Diagnosis Year and Age. Available at http://seer.cancer.gov/archive/csr/1975_2010/results_merged/sect_13_leukemia.pdf. Accessed July 2017.

Source 16 a: National Cancer Institute, Surveillance, Epidemiology, and End Results (SEER) Program. SEER Cancer Statistics Review, 1975-2014. 5-Year Relative and Period Survival (Percent) by Race, Diagnosis Year, Stage and Age. Available at https://seer.cancer.gov/csr/1975_2014/results_merged/topic_survival.pdf. Accessed August 2017.

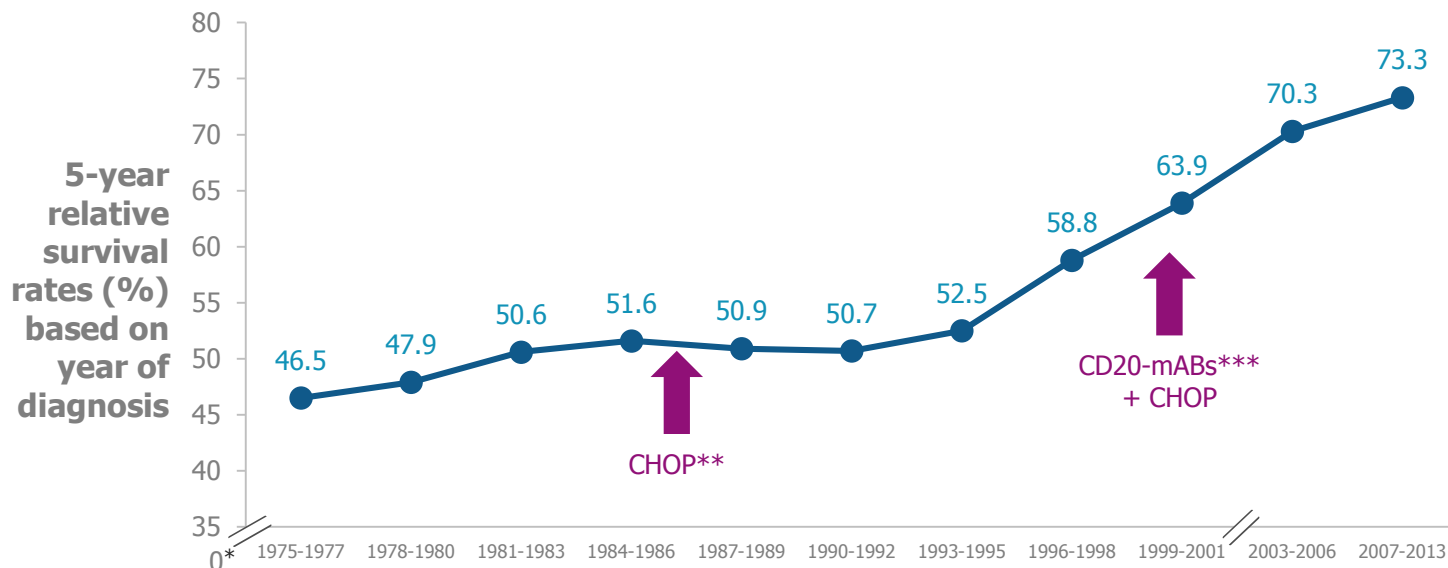
Source 16 b: Weissman I L, et al. The origins of the identification and isolation of hematopoietic stem cells, and their capability to induce donor-specific transplantation tolerance and treat autoimmune diseases. *Blood*. 2008 Nov 1; 112(9): 3543-3553. doi: 10.1182/blood-2008-08-078220. Available at <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC2574516/>. Accessed September 2017.

Source 16 c: Food and Drug Administration. Drugs@FDA: FDA Approved Drug Products. Gleevec (Imatinib). Available at <https://www.accessdata.fda.gov/scripts/cder/daf/index.cfm?event=overview.process&ApplNo=021335>. Accessed September 2017.

Source 16 d: Pophali, P. National Cancer Institute. The Role of New Tyrosine Kinase Inhibitors in Chronic Myeloid Leukemia. *Cancer Journal*. Available at <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4742366/>. Accessed September 2017.



Improving Non-Hodgkin Lymphoma (NHL) Diagnosis and Survival



* y-axis skips from 0 to 40, and then continues in intervals of 5 from there on

** CHOP = cyclophosphamide, doxorubicin, vincristine, prednisone

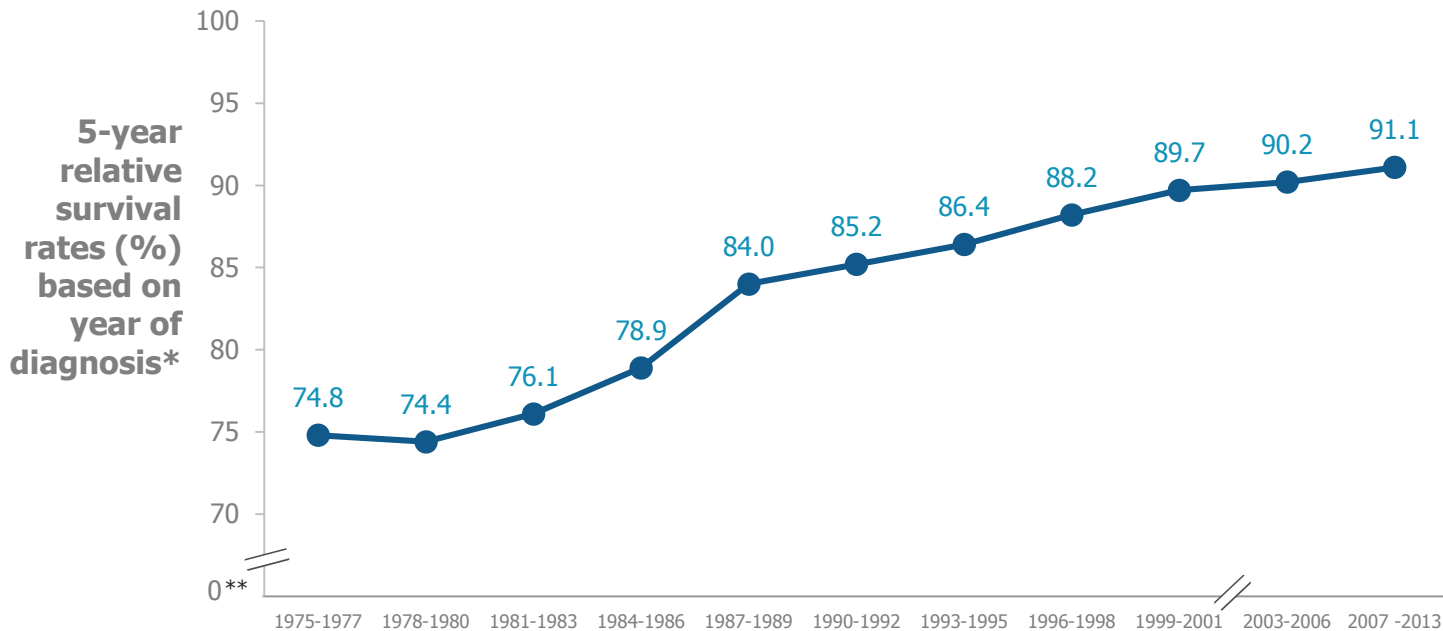
*** mAB = monoclonal antibodies

Source 17: National Cancer Institute, Surveillance, Epidemiology, and End Results (SEER) Program. SEER Cancer Statistics Review, 1975-2014. 5-Year Relative and Period Survival (Percent) by Race, Diagnosis Year, Stage and Age. Available at https://seer.cancer.gov/csr/1975_2014/results_merged/topic_survival.pdf. Accessed August 2017.

Source 17 a: Alduaji, W and Ildige T M. The future of anti-CD20 monoclonal antibodies: are we making progress? *Blood*. 2011 117:2993-3001; doi: <https://doi.org/10.1182/blood-2010-07-298356>. Available at <http://www.bloodjournal.org/content/117/11/2993?variant=fulltext&ssochecked=True>. Accessed September 2017.

Source 17 b: Fisher R I, Gaynor E G, et al. Comparison of a Standard Regimen (CHOP) with Three Intensive Chemotherapy Regimens for Advanced Non-Hodgkin's Lymphoma. *New England Journal of Medicine*. Available at <http://www.nejm.org/doi/full/10.1056/NEJM199304083281404#t=articleBackground>. Accessed September 2017.

Advances Continue in Breast Cancer



*Only data for female cancer patients are included

** y-axis skips from 0 to 70, and then continues in intervals of 5 from there on

Source 18: National Cancer Institute, Surveillance, Epidemiology, and End Results (SEER) Program. SEER Cancer Statistics Review, 1975-2014. 5-Year Relative and Period Survival (Percent) by Race, Diagnosis Year, Stage and Age. Available at https://seer.cancer.gov/csr/1975_2014/results_merged/topic_survival.pdf. Accessed August 2017.



Long-term Breast Cancer Survivors Showed Quality of Life Improvement Similar to that of Their Non-Cancer Counterparts

Studies have found survivors who have been treated showed marked **improvements in social and role functioning** by 1 year post-diagnosis compared to their non-cancer counterparts, with similar quality of life reported on average 12.5 years post-diagnosis.

79% to 82%

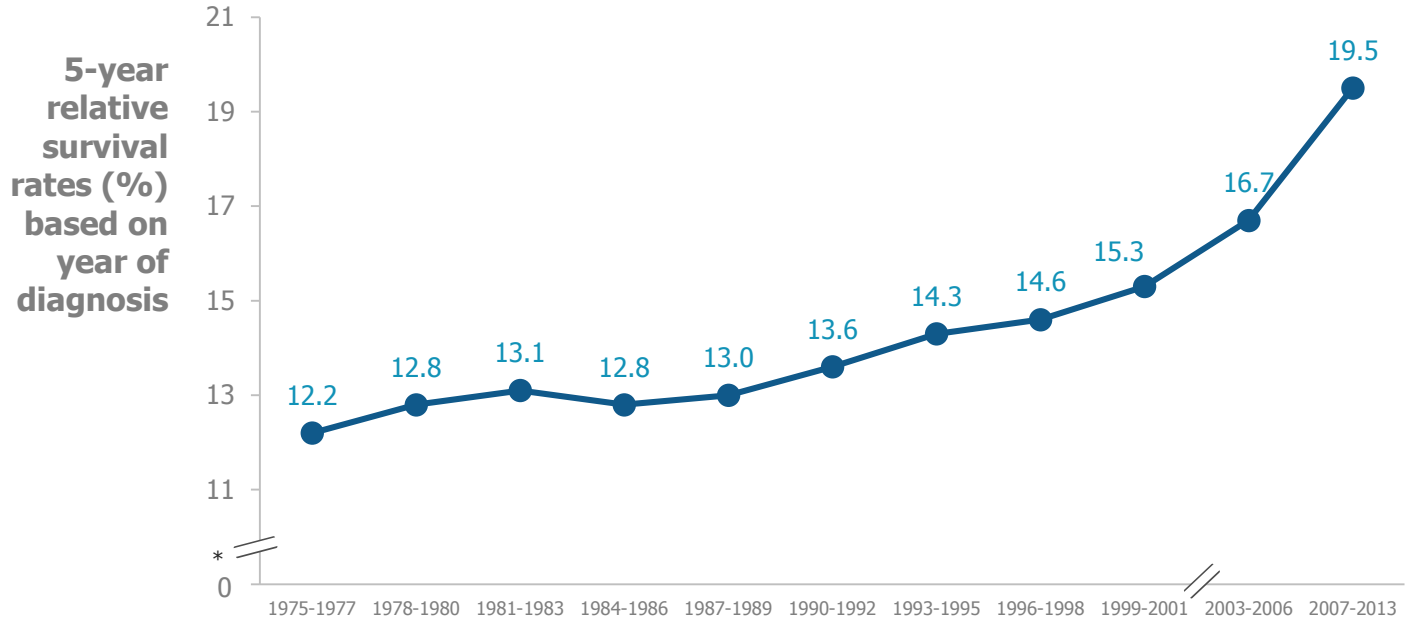
of Breast Cancer survivors
return to work
within the first year
post-diagnosis



According to the European Organization for Research and Treatment of Cancer Quality of Life Questionnaire pain symptom scale, **survivors**, on average, **had less pain** at long-term follow up than controls. Lower pain symptoms in breast cancer survivors compared with the general population were reported in one prior study.

Source 19: DOI: 10.1200/JCO.2012.48.1903 Journal of Clinical Oncology 31, no. 28 (October 2013) 3540-3548. Available at <http://ascopubs.org/doi/abs/10.1200/jco.2012.48.1903>. Accessed on July 2017.

Lung Cancer Five-Year Survival Rate on the Rise

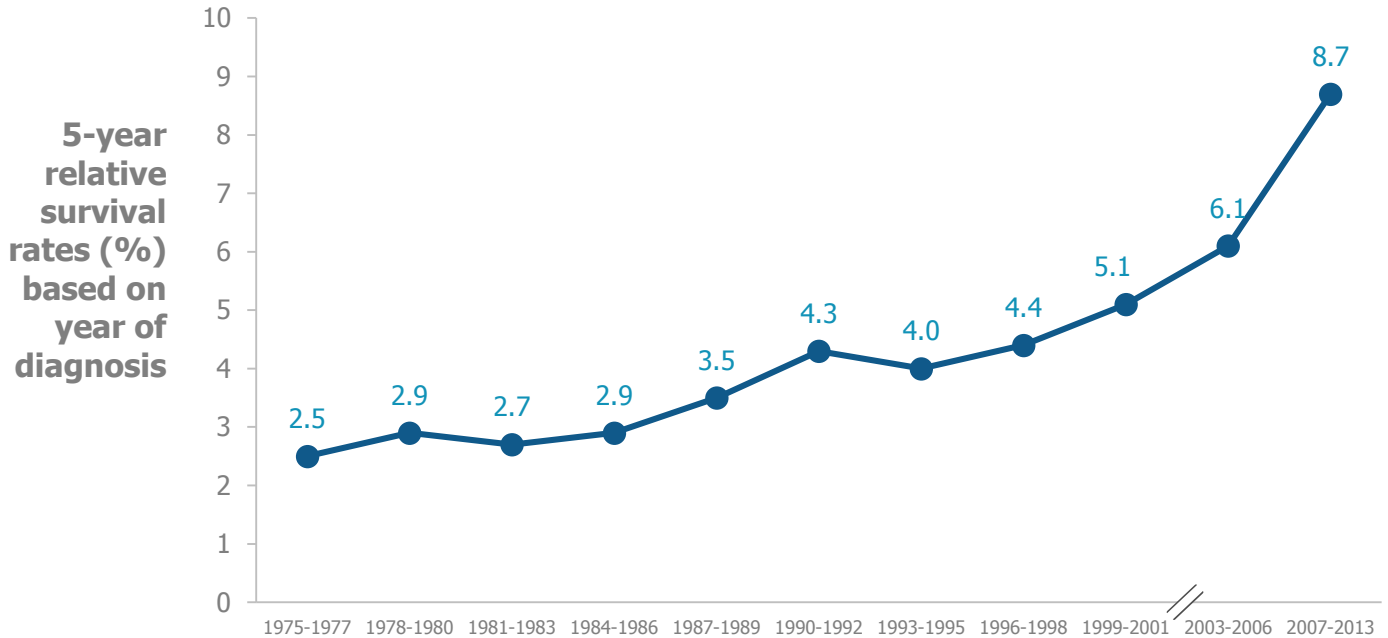


* y-axis skips from 0 to 10, and then continues in intervals of 1 from there on


Source 20: National Cancer Institute, Surveillance, Epidemiology, and End Results (SEER) Program. SEER Cancer Statistics Review, 1975-2014. 5-Year Relative and Period Survival (Percent) by Race, Diagnosis Year, Stage and Age. Available at https://seer.cancer.gov/csr/1975_2014/results_merged/topic_survival.pdf. Accessed August 2017.



Hopeful Signs of Extending Five-Year Survival Rate Among Patients with Pancreatic Cancer



Source 21: National Cancer Institute, Surveillance, Epidemiology, and End Results (SEER) Program. SEER Cancer Statistics Review, 1975-2014. 5-Year Relative and Period Survival (Percent) by Race, Diagnosis Year, Stage and Age. Available at https://seer.cancer.gov/csr/1975_2014/results_merged/topic_survival.pdf. Accessed August 2017.



“ We've made **incredible strides** in cancer treatment, and more cancer **survivors are alive today** than ever before. But oncology isn't just about helping people live longer – we need to **ensure that patients** have the **best quality of life possible** at every stage of their cancer journey, **from active treatment through survivorship.**”

Patricia Ganz, MD, FASCO,
ASCO Expert and Director of Cancer Prevention and Control Research at the Jonsson Comprehensive Cancer Center, University of California, Los Angeles

Notes and Sources



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3

PROGRESS & PROSPERITY



MEDICAL INNOVATION LEADS TO GREATER ECONOMIC GROWTH AND PROSPERITY.

Medical innovation leads to improved health outcomes, lower costs, higher productivity, greater economic value to society and expanded gross domestic product (GDP).

Innovations for polio, infectious disease, diabetes, heart disease and HIV, among others 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 lead to greater productivity and economic growth.



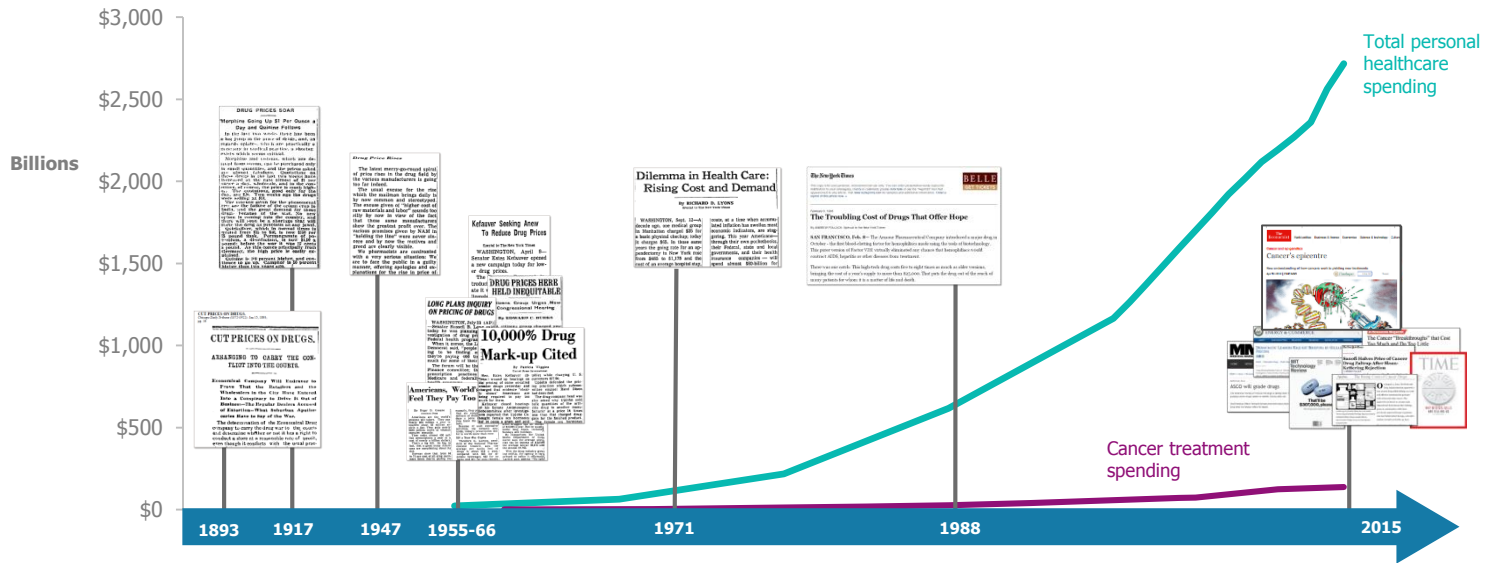


“Over the last half century, improvements in **health** have been as **valuable** as all other sources of **economic growth** and **productivity** combined.”

Kevin Murphy, PhD, and Robert Topel, PhD
University of Chicago Economists

Bringing Healthcare Costs into Focus

Spending on cancer, relative to total healthcare spending, has remained constant over the past half century



Source 1: Centers for Medicare and Medicaid Services. National Health Expenditures; Aggregate and Per Capita Amounts, Annual Percent Change and Percent Distribution: Selected Calendar Years 1960–2012. Available at <http://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/Downloads/tables.pdf>. Accessed July 2017.

Source 1 a: National Cancer Institute. Cancer Trends Progress Report – 2007 Update. Table L1: National Cancer Treatment Expenditures in Billions of Dollars (1963–2004). Available at <http://www.progressreport.cancer.gov/sites/default/files/archive/report2007.pdf>. Accessed July 2017.

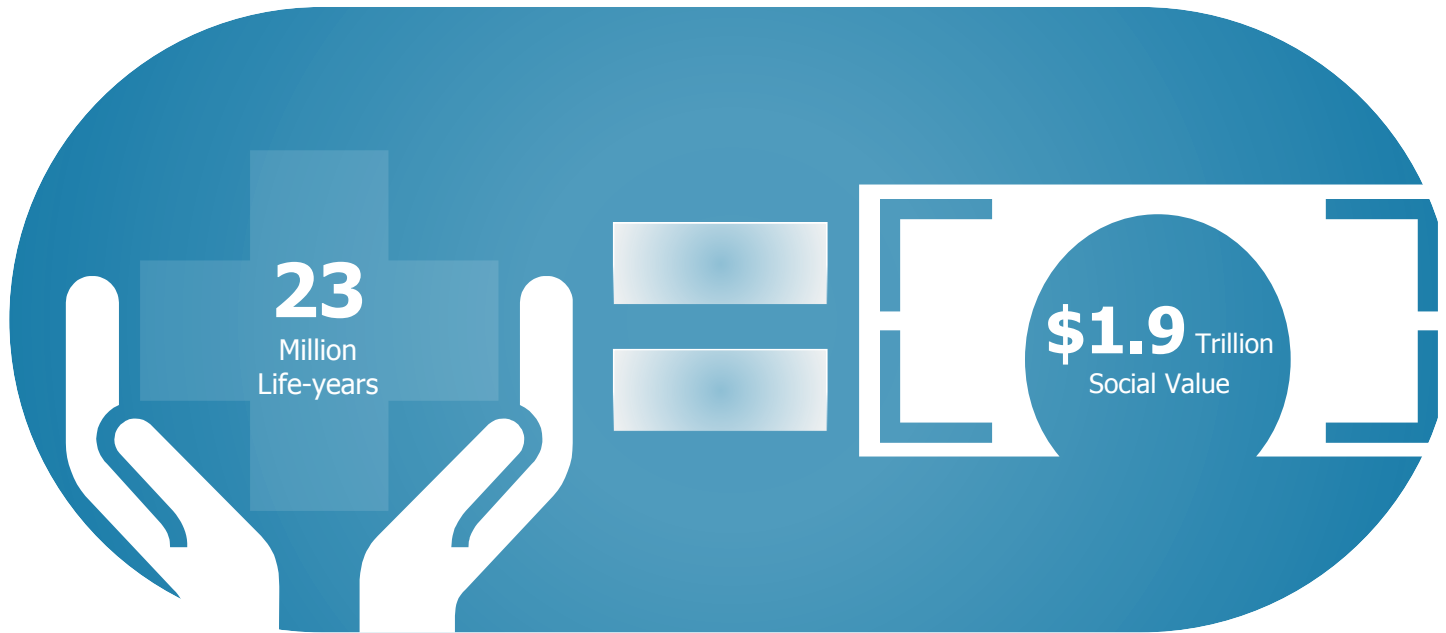
Source 1 b: Center for Medicare and Medicaid Services. Projections of the Cost of Cancer Care in the United States: 2010–2020. Available at <http://www.ncbi.nlm.nih.gov/pmc/articles/PMC3107566/>. Accessed July 2017.

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Source 1 d: Mariotto AB, Yabroff KR, Shao Y, Feuer EJ, Brown ML. Projections of the Cost of Cancer Care in the U.S.: 2010–2020. J Natl Cancer Inst. 2011 Jan. Available at <https://costprojections.cancer.gov/expenditures.html>. Calculated from Full Data Set saved on File. Accessed November 2017.




Additional Life-years Gained and Increased Social Value from Innovative Cancer Treatments, 1988-2000



Source 2: Sun E, Jena A, Lakdawalla D, et al. An Economic Evaluation of the War on Cancer. National Bureau of Economic Research Working Paper No. 15574. Issued in December 2009. Available at <http://www.nber.org/papers/w15574>. Accessed August, 2015.

Living Longer, Better and Healthier Benefits Society

Based on a framework,



1%

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

Worth
\$500 Billion*

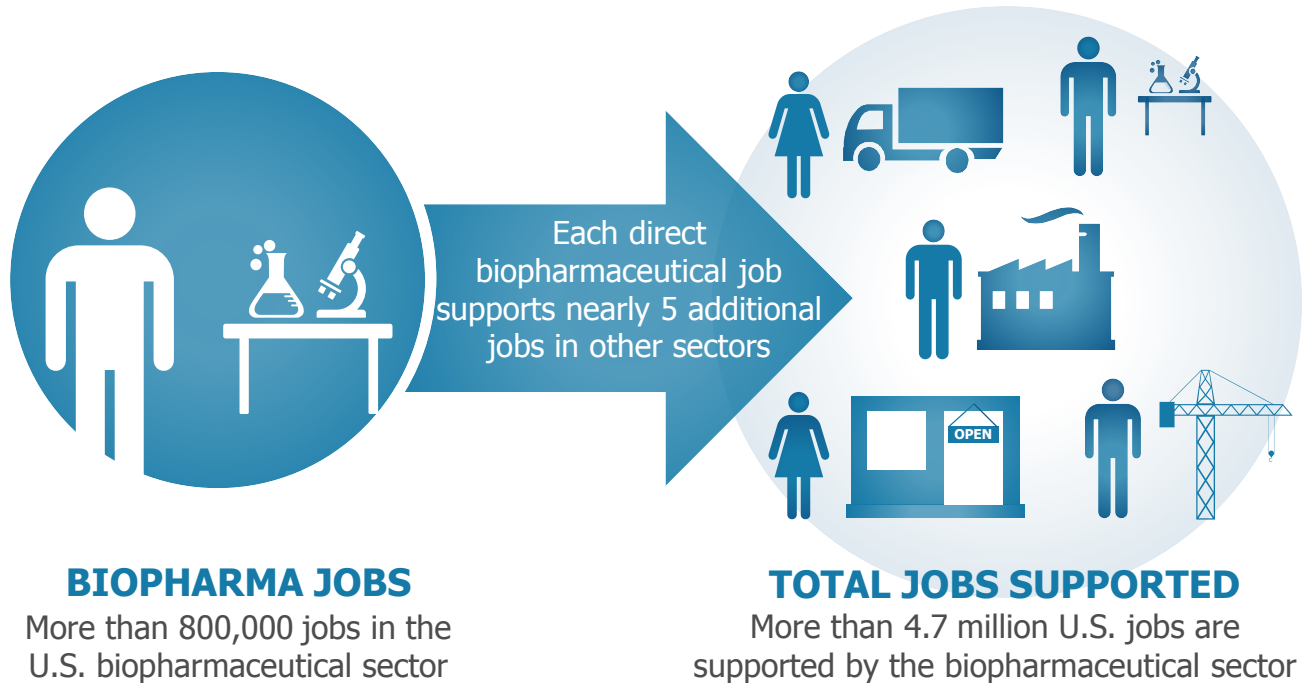
Maximized life expectancy

Stimulated the economy**

* To current and future Americans; study looked at the time period between 1970-2000

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

Biopharmaceutical Sector is Major Job Generator in 21st Century

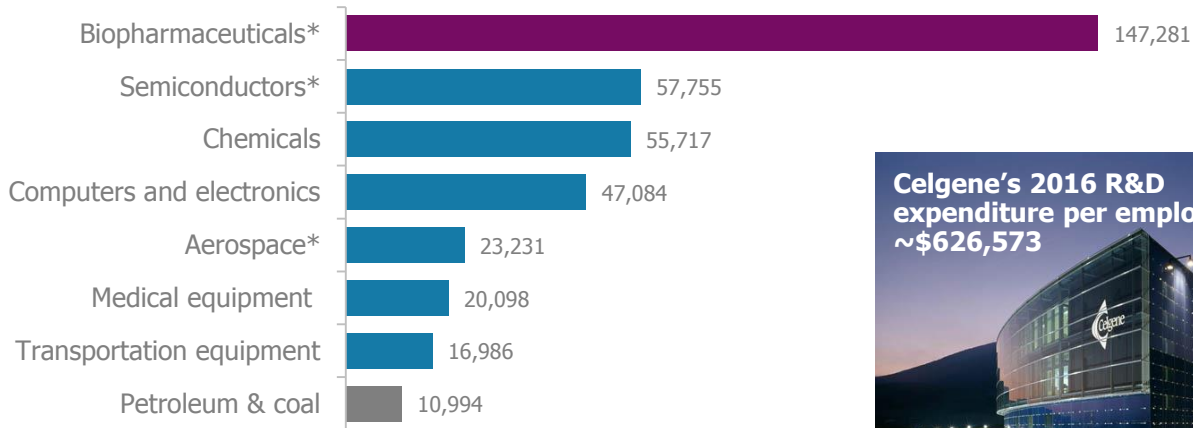


Source 4: TEconomy Partners; for PhRMA. The Economic Impact of the US Biopharmaceutical Industry. Columbus, OH: TEconomy Partners. Available at http://phrma-docs.phrma.org/industryprofile/pdfs/2017IndustryProfile_TheBiopharmaceuticalIndustrysRole.pdf. Accessed July 2017.

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

Biopharmaceutical companies invest approximately more than 12 times the amount of R&D dollars per employee than manufacturing industries overall.

R&D Expenditures per Employee by Manufacturing Industry, 2000-2013



*Manufacturing subsectors

Source 5: Celgene data on file.

Source 5 a: NDP Analytics. IP-Intensive Manufacturing Industries: Driving U.S. Economic Growth from September 2017. Available at <https://static1.squarespace.com/static/52850a5ce4b068394a270176/t/59b8862e9f7456ccb09ede58/1505265200469/IP+Report+2017.pdf>. Accessed November 2017.



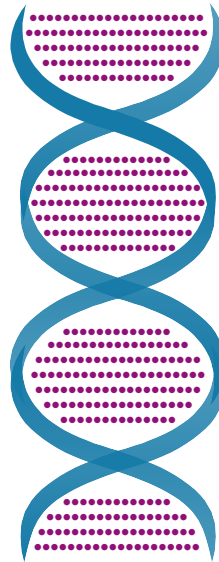
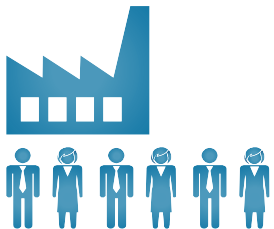
The Human Genome Project: An Example of High Return on Investment in Basic Science

Why Incentivize the Innovators?

1990-2003

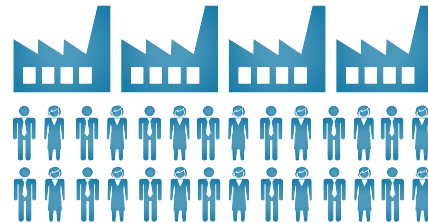
\$3.8 billion

U.S. Investment in the Human Genome Project



2010

The Human Genome Project supported more than 310,000 jobs and added \$67 billion to the U.S. economy



Source 6: Tripp S, Grueber M; Battelle Memorial Institute, Technology Partnership Practice. Economic Impact of the Human Genome Project. May, 2011. Available at http://www.battelle.org/docs/default-document-library/economic_impact_of_the_human_genome_project.pdf?sfvrsn=2. Accessed July 2017.

The Human Genome Leads to More Precise Targeting for Cancer Therapies

The Human Genome Project maximizes the potential of innovative medicines via more precise targeting that can improve lives and meaningfully contribute to society.

Before the
Human Genome Project,
researchers knew
the genetic basis of about

60

DISORDERS

Today, they know
the basis of nearly

5,000

OF CONDITIONS

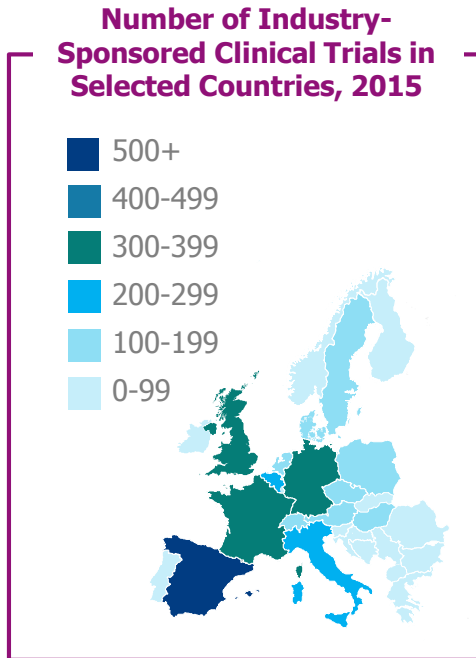
MORE THAN
100

DIFFERENT FDA-APPROVED
MEDICINES



Are now packaged with genomic information that
tells doctors to test their patients for genetic variants
linked to efficacy, dosages or side-effects

Industry-Sponsored Clinical Trials Contribute to Economic Activity in Communities Around the World



— In 2015 —

the biopharmaceutical industry sponsored

9,059

clinical trials

of medicines around the world, supporting tangible economic activity such as jobs in the countries in which the trials are located.

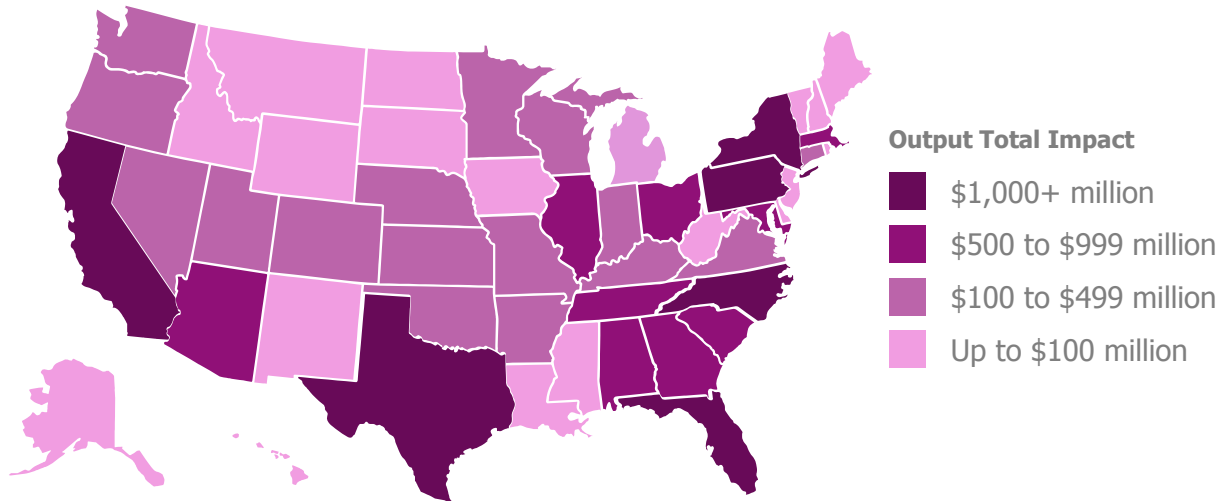
Note: Represents all clinical trials Phase 2 through Phase 4 that registered with clinicaltrials.gov in 2015.

Source 8: Health Advances analysis. clinicaltrials.gov. (accessed February 2016). EFPIA. Available at <https://www.efpia.eu/publications/data-center/value-to-the-economy/clinical-trials/>. Accessed October 2017.

Source 8 a: Susan B. Shurin, MD, Deputy Director, National Heart, Lung, and Blood Institute (NHLBI), National Institutes of Health, Bethesda, Maryland, United States. Clinical Trials for Better Health Policies. OECD Observer. Available at http://oecdobserver.org/news/fullstory.php/aid/3984/Clinical_trials_for_better_health_policies.html. Accessed October 2017.

Across the U.S., Industry-Sponsored Clinical Trials Contribute Significant Economic Activity

In 2013, the biopharmaceutical industry sponsored **6,199 clinical trials** of medicines around the United States, involving 1.1 million volunteer participants and supporting an estimated \$25 billion in economic activity across all 50 states and the District of Columbia.*



*Estimates reflect only those activities occurring at clinical trial sites and exclude more centralized, cross-site functions such as coordination and data analysis. Also excluded are nonclinical R&D such as basic and preclinical research and the significant economic contribution from non-R&D activities of the industry such as manufacturing and distribution.

Source 9: Battelle Technology Partnership Practice; for PhRMA. Biopharmaceutical industry-sponsored clinical trials: impact on state economies. Available at <http://phrma-docs.phrma.org/sites/default/files/pdf/biopharmaceutical-industry-sponsored-clinical-trials-impact-on-state-economies.pdf>. Published March 2015. Accessed May 2017.

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 - 8 a:** Susan B. Shurin, MD, Deputy Director, National Heart, Lung, and Blood Institute (NHLBI), National Institutes of Health, Bethesda, Maryland, United States. Clinical Trials for Better Health Policies. OECD Observer. Available at http://oecdobserver.org/news/fullstory.php/aid/3984/Clinical_trials_for_better_health_policies.html. Accessed October 2017.
9. Battelle Technology Partnership Practice; for PhRMA. Biopharmaceutical industry-sponsored clinical trials: impact on state economies. Available at <http://phrma-docs.phrma.org/sites/default/files/pdf/biopharmaceutical-industry-sponsored-clinical-trials-impact-on-state-economies.pdf>. Published March 2015. Accessed May 2017.





BETTER HEALTHCARE, BETTER OUTCOMES



CONTINUED MEDICAL INNOVATION CAN LEAD TO A REDUCTION IN HEALTHCARE SPENDING.

Medical innovation is transforming certain types of cancer from leading causes of death to long-term, manageable diseases while also reducing the overall strain on healthcare systems.

Through investment in medicines, overall healthcare spending can be reduced. By improving health, medicines can reduce hospital admissions and reduce the use of costly medical services. These advancements coupled with improved access to medicines can result in a decrease of overall healthcare costs.

Medical innovation has allowed for significant changes in the treatment for cancer care. Increasingly, cancer will be managed by preventing and controlling cancer through genetic biomarkers and precision medicine with the hope of stopping the cancer as quickly as possible.

And while new cancer therapies may have a positive impact for patients, it cannot be sustained without a balance in the virtuous cycle of medical innovation.



“ Pharmaceuticals have the effect of **improving** or **maintaining an individual's health** ... adhering to a drug regimen for a chronic condition such as diabetes or high blood pressure may prevent complications ... taking the medication may also avert hospital admissions and thus **reduce the use of medical services.**”

– Congressional Budget Office

New Therapies Are a Major Contributor to Improvements in Life Expectancy

Medicines have played an important role in helping patients live longer and healthier lives. New medicines continue to be a major contributor to improvements in life expectancy.



1986 - 2000

Overall life expectancy was almost 2 years with new therapies contributing to approximately .8 years



2000 - 2009

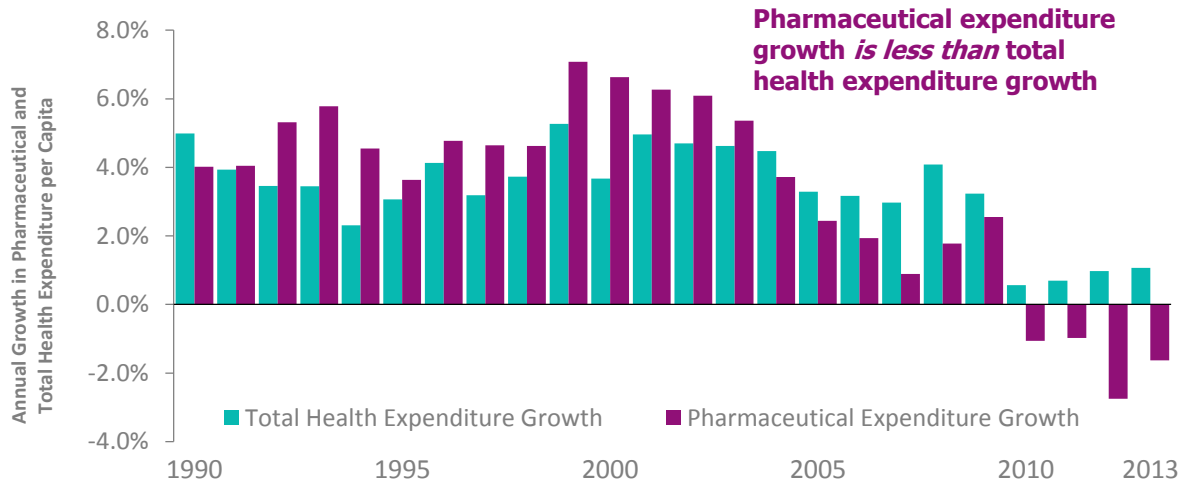
Overall life expectancy was almost 1.74 years with new therapies contributing to approximately 1.27 years

Source 1: 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 July 2017.

Source 1 a: 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 July 2017.

Total Healthcare Expenditure has Seen Constant Growth Globally, While Spending on Medicines has Decreased

Pharmaceutical expenditure has substantially decreased since 2010 while total healthcare expenditure has continued to grow across developed markets from 1990 - 2013

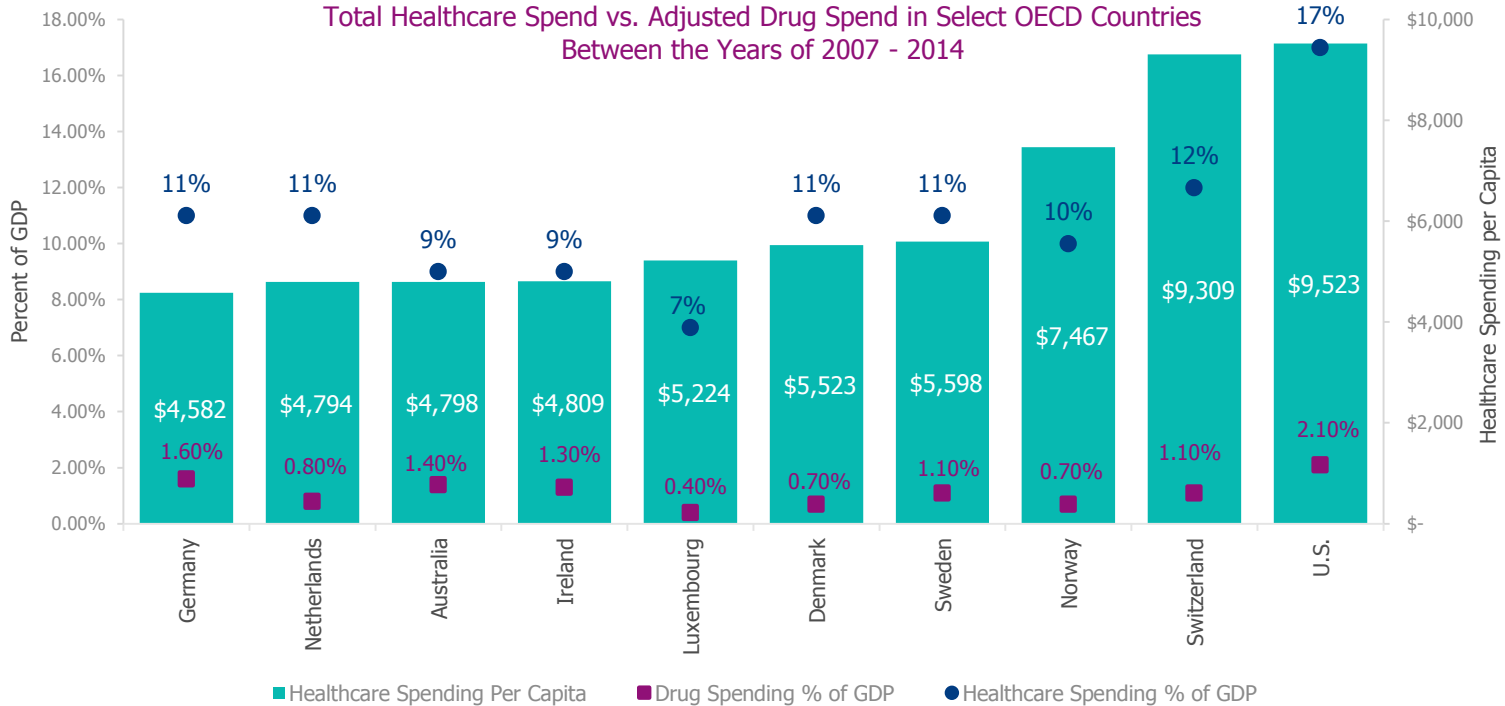


Note: Average annual growth in pharmaceutical and total health expenditure per capita, in real terms, average across OECD countries, 1990 to 2013 (or nearest year). Countries include Australia, Austria, Belgium, Canada, Czech Republic, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Iceland, Ireland, Israel, Italy, Japan, Korea, Luxembourg, Mexico, Netherlands, New Zealand, Norway, Poland, Portugal, Slovak Republic, Slovenia, Spain, Sweden, Switzerland, Turkey, United Kingdom, United States.

Source 2: EFPIA 2015, H&G evidence compendium. Available at <http://efpia.morris-chapman.net/documents/160/138/2015-Health-amp-Growth-evidence-compendium-Slide-decks>. Accessed July 2017. Calculated at OECD (2015), Average annual growth in pharmaceutical and total health expenditure per capita, in real terms, average across OECD countries, 1990 to 2013 (or nearest year), in Health at a Glance 2015, OECD Publishing, Paris. Available at http://dx.doi.org/10.1787/health_glance-2015-graph8-en. Accessed August 2017.

Drug Spending is a Small Portion of Total Healthcare Spend in Select OECD Countries

Total Healthcare Spend vs. Adjusted Drug Spend in Select OECD Countries Between the Years of 2007 - 2014



Source 3: OECD, Disposable Income Per Capita: 2013 Data; 2014: United States, Australia, Netherlands, Finland; 2012: New Zealand; 2009: Japan, Total Hospital Spending, Hospital Spending Per Capita: 2014 estimated data; 2013: Australia, Japan, and Norway; 2007 New Zealand, Total drug spend, Drug spend per capita: 2014 Data, 2013: Australia, Japan; 2007: New Zealand. Available at <https://data.oecd.org/healthres/pharmaceutical-spending.htm>. Accessed August 2017.

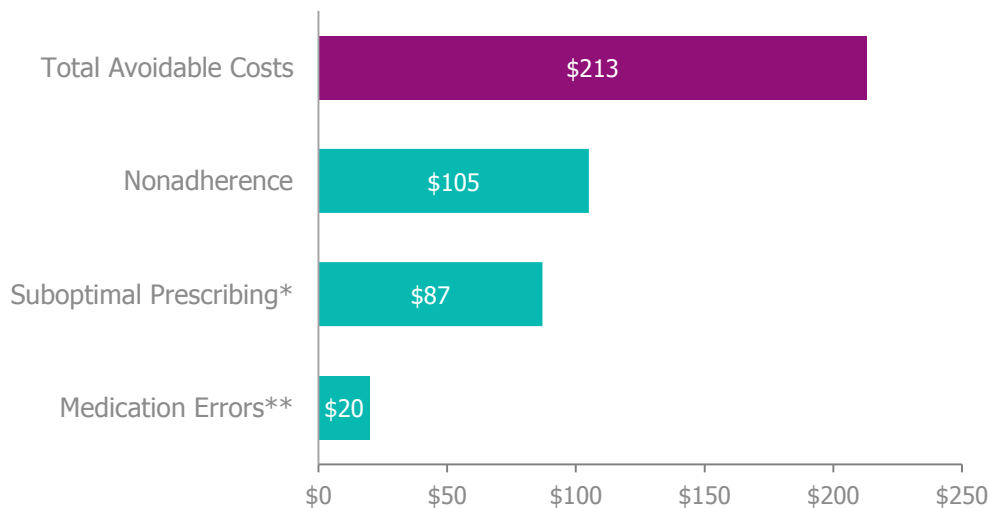


Potential Savings From Better Use of Medicines



Based on estimates from 2012, better use of medicines could eliminate up to \$213 billion in U.S. healthcare costs annually, which represents 8% of the nation's healthcare spending.

Avoidable Annual U.S. Healthcare Cost Estimates (in Billions, 2012)

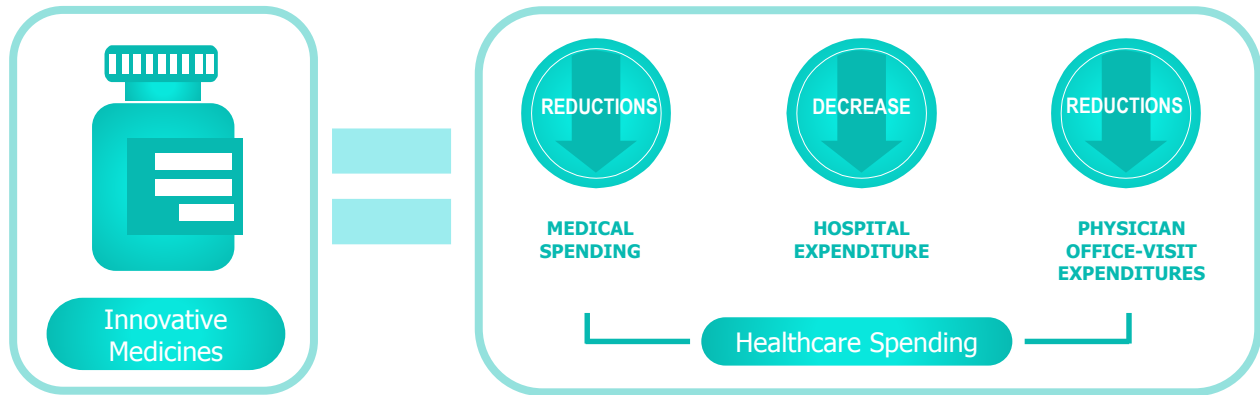


* Category includes untimely medicine use (\$40 billion), inappropriate antibiotic use (\$35 billion), and suboptimal generic use (\$12 billion)

** Category includes medication errors (\$20 billion) and mismanaged polypharmacy in the elderly (\$1 billion)

Source 4: IMS Institute for Healthcare Informatics. Avoidable costs in US healthcare: the \$200 billion opportunity from using medicines more responsibly. Available at http://www.imshealth.com/files/web/IMSH%20Institute/Reports/Avoidable_Costs_in%20US_Healthcare/IHII_AvoidableCosts_2013.pdf. Published June 2013. Accessed August 2017.

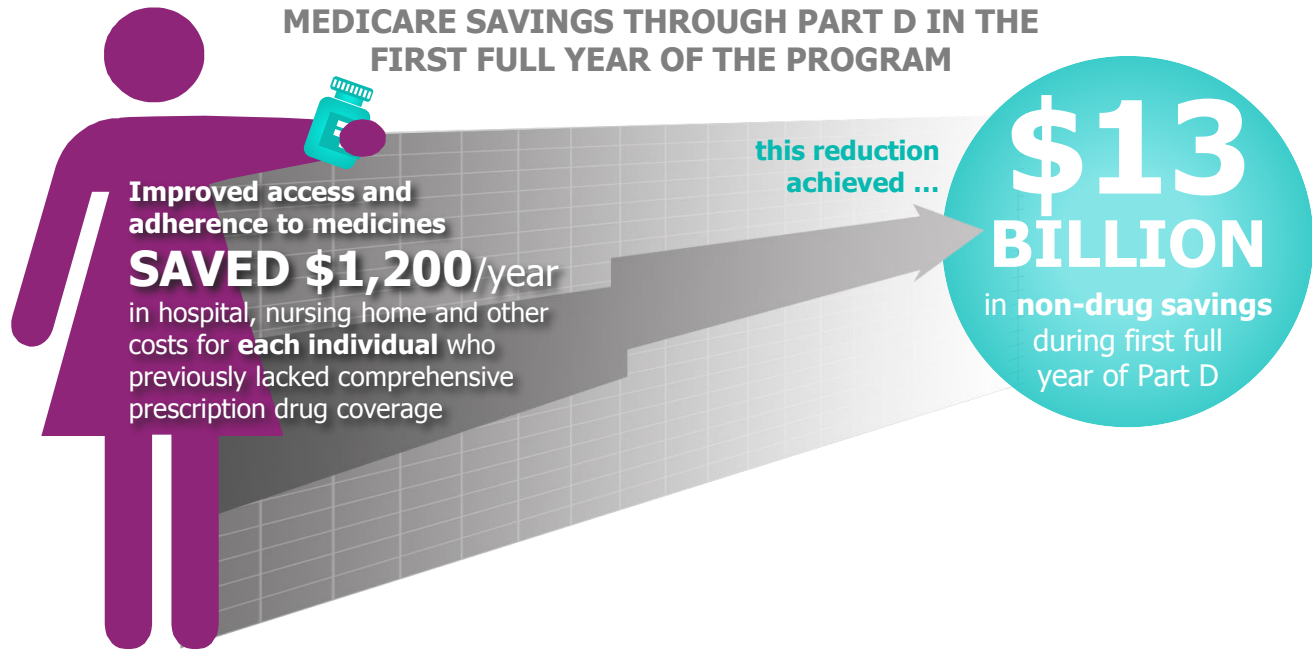
Medical Innovation Reduces Healthcare Spending While Increasing Patient Health and Survival



\$1 SPENT = \$6.20 SAVED

For every dollar spent on innovative medicines, total U.S. healthcare spending is reduced by \$6.20

Improved Access and Adherence to Medicines Through Medicare Part D Results in Non-Drug Cost Savings



Source 6: McWilliams JM, Zaslavsky AM, Huskamp HA. Implementation of Medicare Part D and nondrug medical spending for elderly adults with limited prior drug coverage. JAMA. 2011;306(4):402-409. Available at <http://jamanetwork.com/> on 08/20/2017. Accessed August 2017.

Source 6 a: Afendulis CC, Cherner ME. State-level impacts of Medicare Part D. Am J Managed Care. 2011;17 Suppl 12:S. Available at <http://www.ajmc.com/journals/supplement/2011/StateLevelImpactsofMedicarePartD/StateLevelImpactsofMedicarePartD/>. Accessed August 2017.

Spending per Medicare Beneficiaries Decreased in 2014

In 2014, the Congressional Budget Office lowered its projections of total spending on Medicare benefits from 2012 through 2021 **by \$370 billion**, excluding sequestration savings.

Between 2011-2014

APPROX.
10%

OF
MEDICARE
PART D
SPENDING
ACCOUNTED
FOR

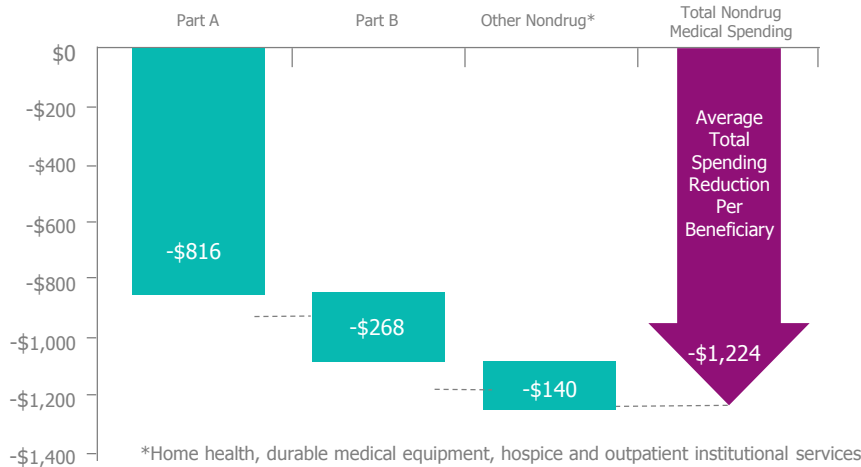
MORE THAN
60%

OF THE
SLOWDOWN IN
OVERALL
MEDICARE
BENEFITS

Gaining Drug Coverage Reduced Other Medical Spending and Resulted in an Increase in Longevity

The Medicare drug benefit increased access to medicines for those previously without drug coverage, resulting in reduced medical spending and non-drug savings of \$13.4 billion in 2007, the first full year of the benefit.

Average Reduction in Medical Spending in 2006 and 2007 for Beneficiaries Who Gained Drug Coverage Through Medicare Part D



BETWEEN THE IMPLEMENTATION OF PART D in 2006 and 2014,

an estimate of nearly

200,000 MEDICARE BENEFICIARIES

— lived at least —
1 YEAR LONGER

— with an average —
increase in longevity of

3.3 YEARS



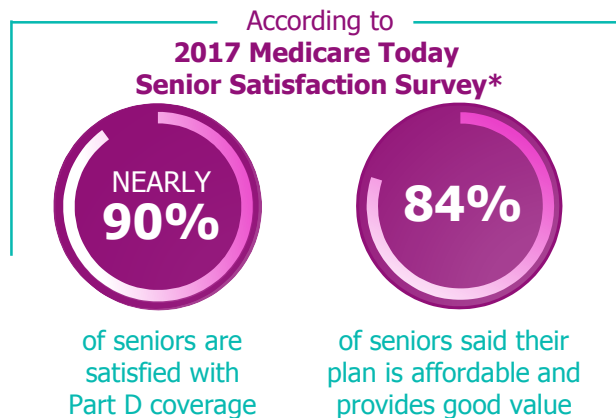
Source 8: Afendulis CC, Chernew ME. State-level impacts of Medicare Part D. Am J Managed Care. 2011;17 Suppl 12:S. Available at <http://www.ajmc.com/journals/supplement/2011/StateLevelImpactsofMedicarePartD/StateLevelImpactsofMedicarePartD/>. Accessed August 2017.

Source 8 a: Semilla AP, Chen F, Dall TM. Reductions in mortality among Medicare beneficiaries following the implementation of Medicare Part D. Am J Managed Care. 2015;21:S165-S172. Available at http://www.ajmc.com/journals/supplement/2015/a580_jul15_medicarepartd/a580_jul15_medicarepartd_web. Accessed August 2017.

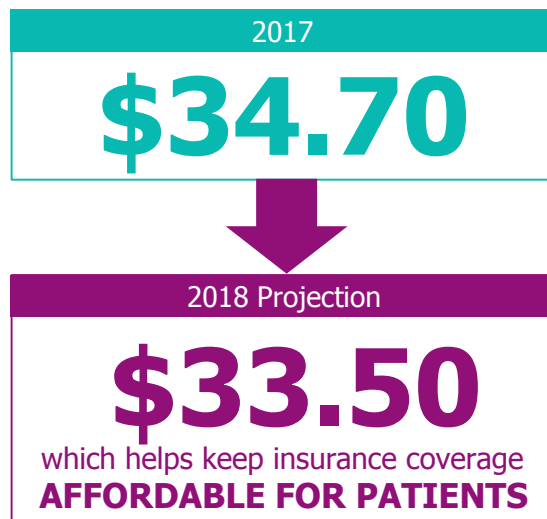
Source 8 b: McWilliams JM, Zaslavsky AM, Huskamp HA. Implementation of Medicare Part D and nondrug medical spending for elderly adults with limited prior drug coverage. JAMA. 2011;306(4):402-409. Available at <http://jamanetwork.com/> on 08/20/2017. Accessed August 2017.

Many Patients are Satisfied with Medicare Part D Coverage

Nearly **41 million people** are enrolled in Medicare Part D as of 2016



AVERAGE COST PER PATIENT FOR
MONTHLY MEDICARE PART D PREMIUMS



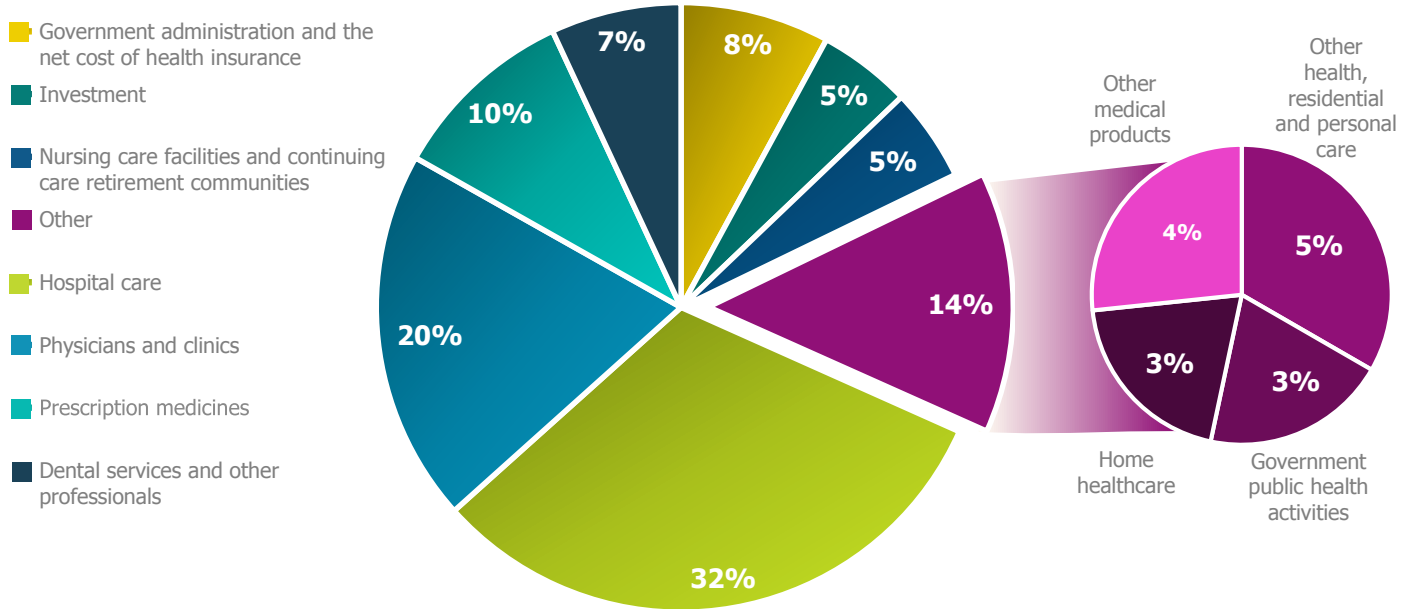
*This poll was conducted by Morning Consult from June 18 – July 6, 2017, among a national sample of 1,944 seniors who are Medicare Part D recipients. The interviews were conducted online and the data were weighted to approximate a target sample of seniors based on age, race/ethnicity and gender. Results from the full survey have a margin of error of plus or minus 2 percentage points.

Source 9: Medicare Today. Senior Satisfaction Survey. Available at <http://medicaretoday.org/wp-content/uploads/2017/08/2017-Senior-Satisfaction-Survey-Fact-Sheet.pdf>. Accessed September 2017.

Source 9 a: Center for Medicare and Medicaid Services. 2017 ANNUAL REPORT OF THE BOARDS OF TRUSTEES OF THE FEDERAL HOSPITAL INSURANCE AND FEDERAL SUPPLEMENTARY MEDICAL INSURANCE TRUST FUNDS. <http://medicaretoday.org/wp-content/uploads/2017/08/2017-Senior-Satisfaction-Survey-Fact-Sheet.pdf>. Accessed September 2017.

Source 9 b: The Kaiser Family Foundation. The Medicare Part D Prescription Drug Benefit. Available at <http://www.kff.org/medicare/fact-sheet/the-medicare-prescription-drug-benefit-fact-sheet/>. Accessed September 2017.

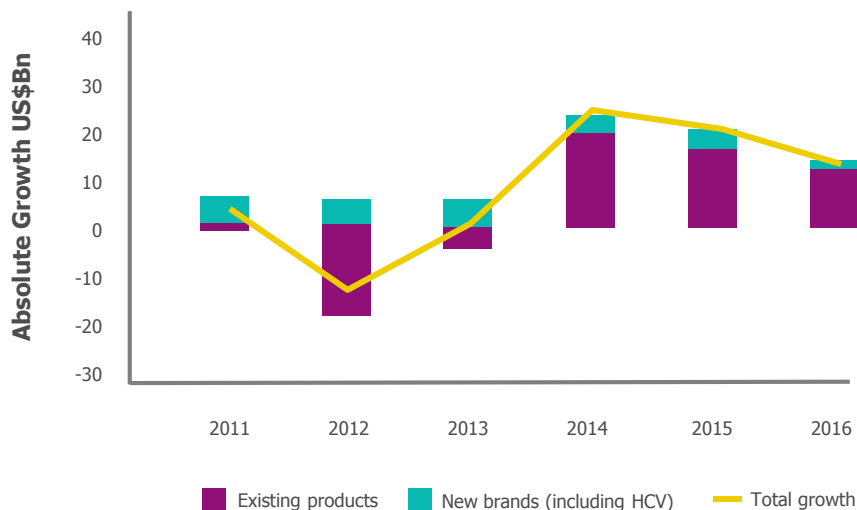
The Health Dollar in the U.S. (\$3.2 Trillion), Calendar Year 2015: Where It Went



Source 10: Centers for Medicare & Medicaid Services. National Health Expenditures 2015 Highlights. Available at <https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/downloads/highlights.pdf>. Accessed July 2017.

Net Revenue Growth for Medicines has Been Driven by New Medicines, Which Peaked in 2014 but Have Declined Since Then

Net Revenue Growth by Product Type, Segmentation Comparison (U.S. \$Bn)

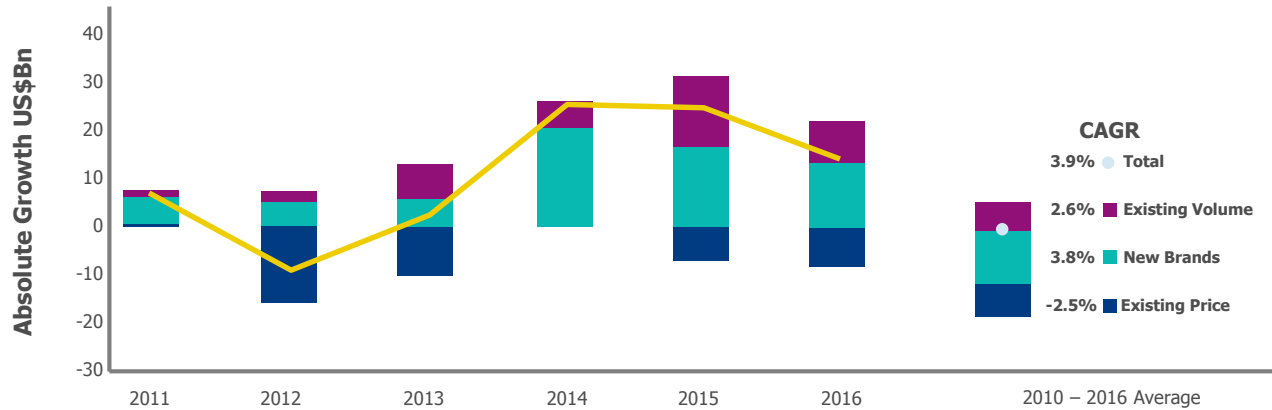


Source 11: QuintilesIMS, National Sales Perspectives, Dec 2016; QuintilesIMS Institute Aug 2017. Available at http://www.imshealth.com/files/web/IMSH%20Institute/Reports/QIHI_Understanding_the_Drivers_of_Drug_Expenditure_US.pdf. Accessed October 2017.



Manufacturer Revenue Growth has Been Driven by New Products and Volume Growth from Existing Products

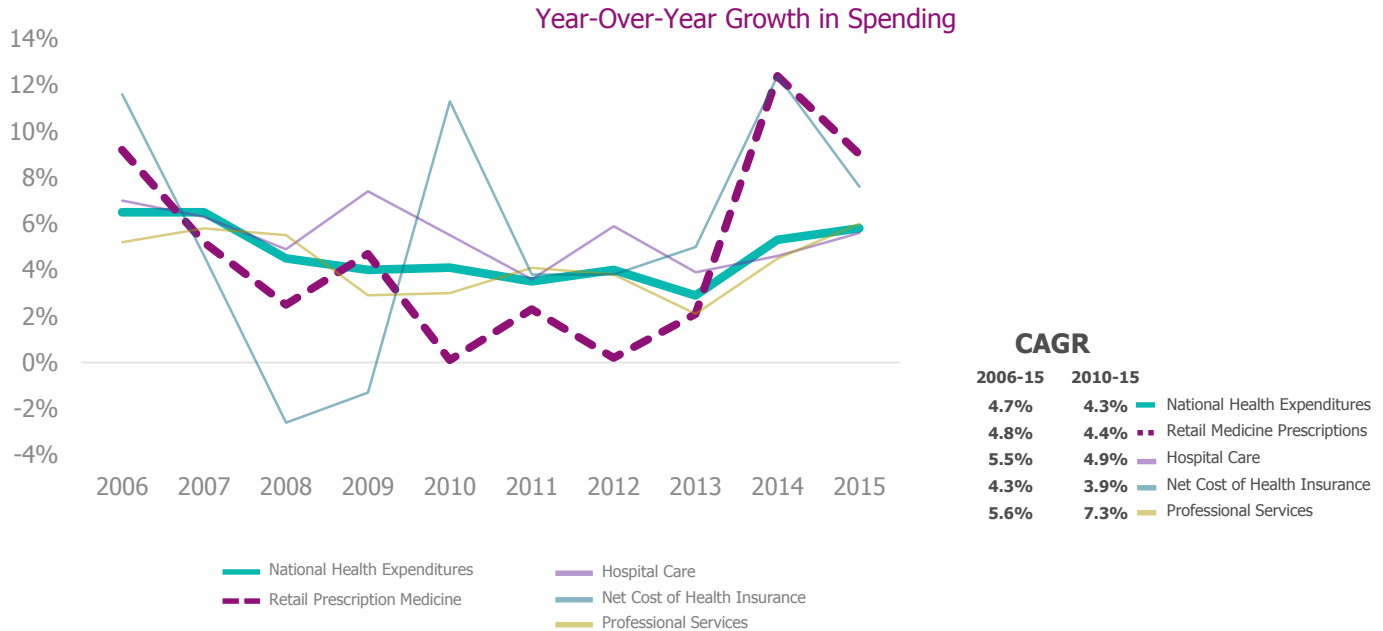
Net Revenue Growth by Product Type (U.S. \$Bn)



Existing products volume growth New brands Existing products price growth Total growth

Source 12: QuintilesIMS, National Sales Perspectives, Dec 2016; QuintilesIMS Institute Aug 2017. Available at http://www.imshealth.com/files/web/IMSH%20Institute/Reports/QIIMI_Understanding_the_Drivers_of_Drug_Expenditure_US.pdf. Accessed October 2017.

Retail Prescription Growth Has Been Lower or On Par With Other Components of Healthcare Spending



Source 13: CMS National Health Expenditure Accounts, January 2017. Available at <https://www.cms.gov/research-statistics-data-and-systems/statistics-trends-and-reports/nationalhealthexpenddata/nationalhealthaccountsprojected.html>. Accessed October 2017. QuintilesIMS, National Sales Perspectives, Dec 2016; QuintilesIMS Institute Aug 2017. Available at http://www.imshealth.com/files/web/IMSH%20Institute/Reports/QIIHI_Understanding_the_Drivers_of_Drug_Expenditure_US.pdf. Accessed October 2017.

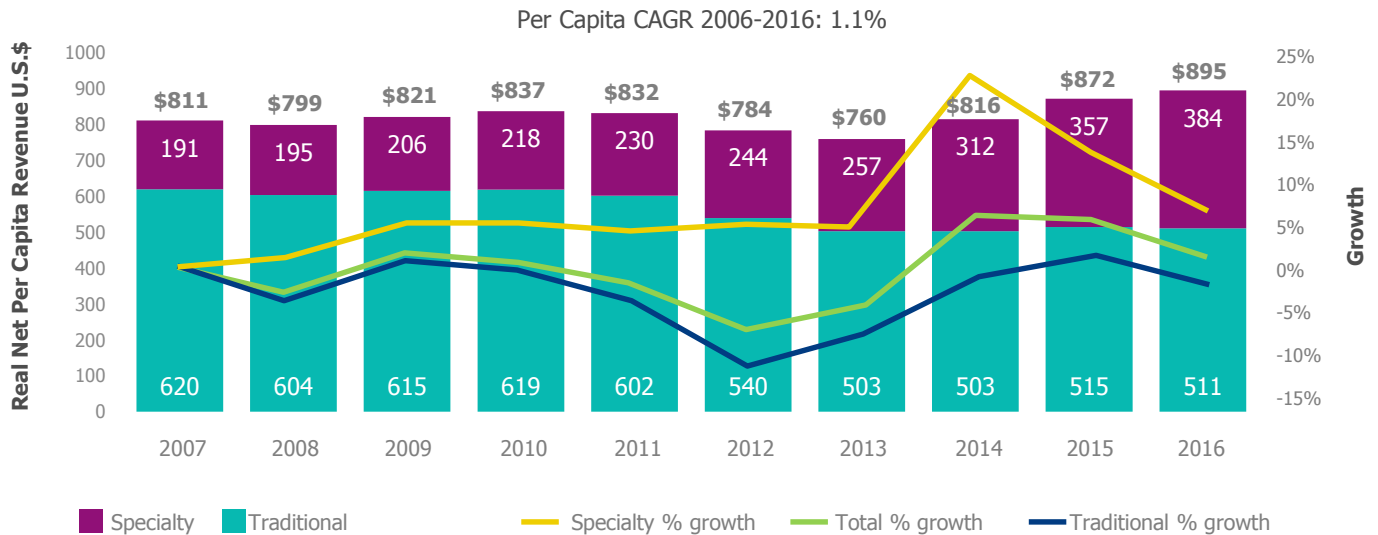


The Virtuous Cycle in Effect



Traditional medicine costs are declining due to patent expirations, creating “room” for new specialty medicines

Real Net Per Capita Medicine Manufacturer Revenue and Growth by Type, (U.S.\$)

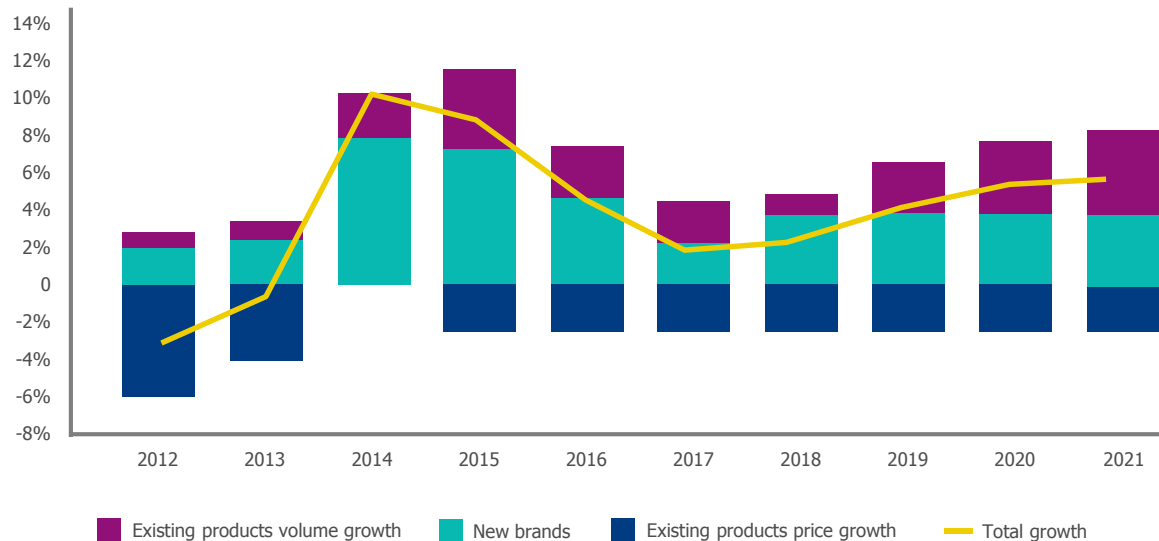


Source 14: QuintilesIMS, National Sales Perspectives, Dec 2016; QuintilesIMS Institute Aug 2017. Available at http://www.imshealth.com/files/web/IMSH%20Institute/Reports/QIHI_Understanding_the_Drivers_of_Drug_Expenditure_US.pdf. Accessed October 2017.

Prescription Net Revenue Growth is Expected to Average 2-5% Through 2021

Growth will be driven by new and existing products and offset by patent expirations

Net Medicines Revenue Growth and Contribution by Type



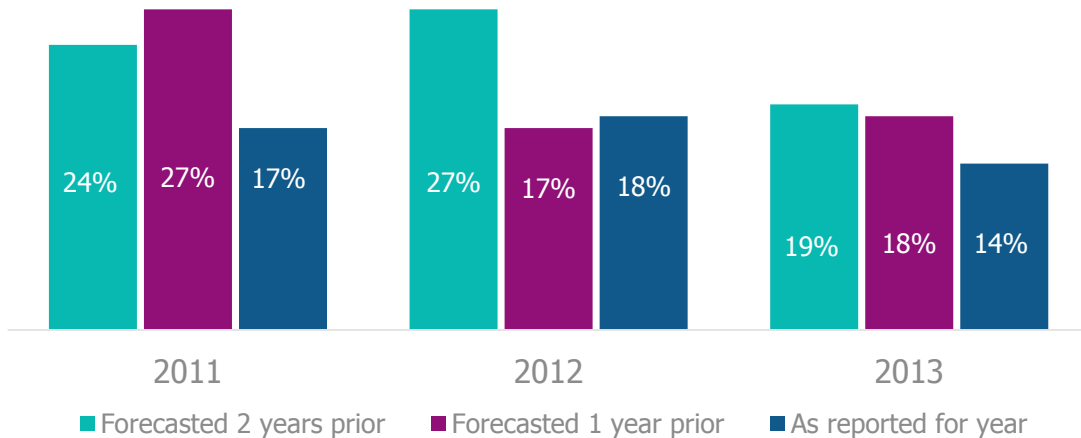
Source 15: QuintilesIMS, National Sales Perspectives, Market Prognosis, QuintilesIMS Institute, Mar 2017. Available at http://www.imshealth.com/files/web/IMSH%20Institute/Reports/QIIHI_Understanding_the_Drivers_of_Drug_Expenditure_US.pdf. Accessed October 2017.



Forecasts of Specialty Drug Spending Have Been Routinely Overstated

An analysis of annual drug trend reports found that inconsistent definitions of “specialty medicines” can bias spending projections

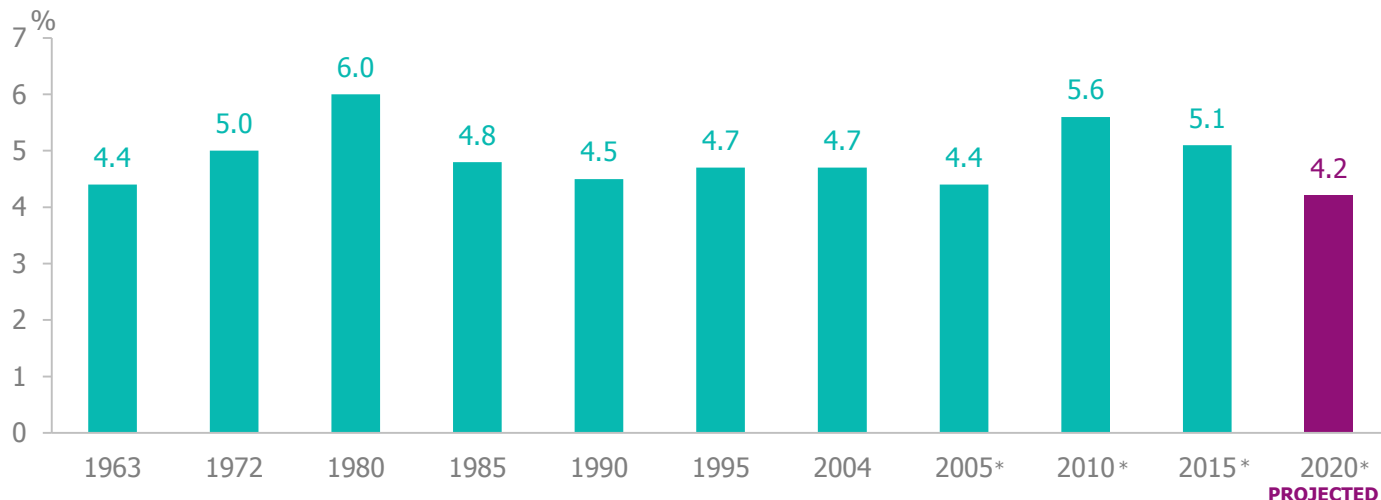
Forecast vs. Actual Growth in Specialty Medication Spending From a Major Pharmacy Benefits Management Company*



* As reported in annual Drug Trend Reports from Express Scripts.

Source 16: Dieguez G, et. al. Milliman, Inc. Understanding Specialty Drug Forecasts. Available at <http://phrma-docs.phrma.org/sites/default/files/pdf/milliman-specialty-drug-forecasts.pdf>. Accessed July 2017.

Cost of Cancer Treatment is and is Expected to Remain, a Small Portion of Total Personal Healthcare Expenditures



*Calculated based on available data, sources a through d

Source 17: (1963-2004) National Cancer Institute. Cancer Trends Progress Report – 2007 Update. Table L1: National Cancer Treatment Expenditures in Billions of Dollars (1963-2004). Available at <http://www.progressreport.cancer.gov/sites/default/files/archive/report2007.pdf>. Accessed August 2017.

Source 17 a: Centers for Disease Control. Table 111 Gross domestic product, national health expenditures, per capita amounts, percent distribution, and average annual percent change: United States, selected years 1960–2011. Available at <http://www.cdc.gov/nchs/hus/contents2012.htm#111>. Accessed August 2017.

Source 17 b: Center for Medicare and Medicaid Services. National Health Expenditure Projections 2012-2022. Available at <https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/Downloads/Proj2012.pdf>. Accessed August 2017.

Source 17 c: Mariotto A. et al. Journal of the National Cancer Institute. Available at <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3107566/table/tbl5/>. Accessed August 2017.

Source 17 d: National Cancer Institute. National Expenditures for Cancer Care. Available at <https://costprojections.cancer.gov/expenditures.html>. Accessed August 2017.

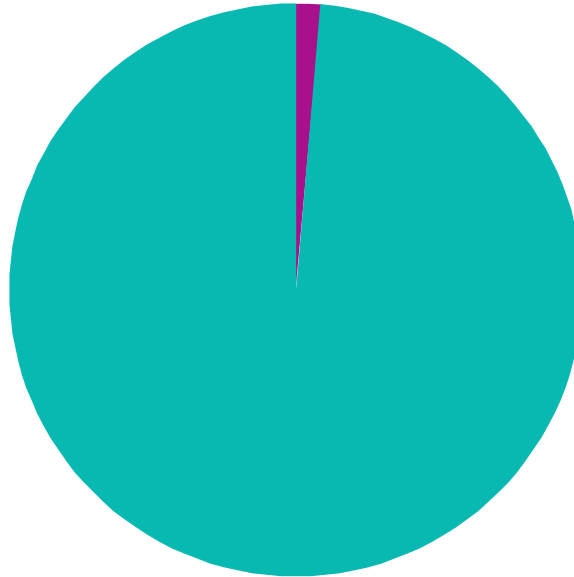
Source 17 e: Cutler, D. Are we Finally Winning the War on Cancer? *J Econ Persp* 2008;22(4):3-26. Mariotto A, Yabroff K, Shao Y, et al. Projections of the Cost of Cancer Care in the United States: 2010–2020. *J Natl Cancer Inst.* 2011;103(2):117-128. Centers for Medicare and Medicaid Services. Available at Accessed August 2017.



New Cancer Therapies: Small Cost, Large Impact for Patients and Society

Spending on cancer medicines represents about 1% of overall healthcare spending

Cancer medicines*
as a portion of
National Health
Expenditures
Projected Total
U.S. Healthcare
Spending, 2016**



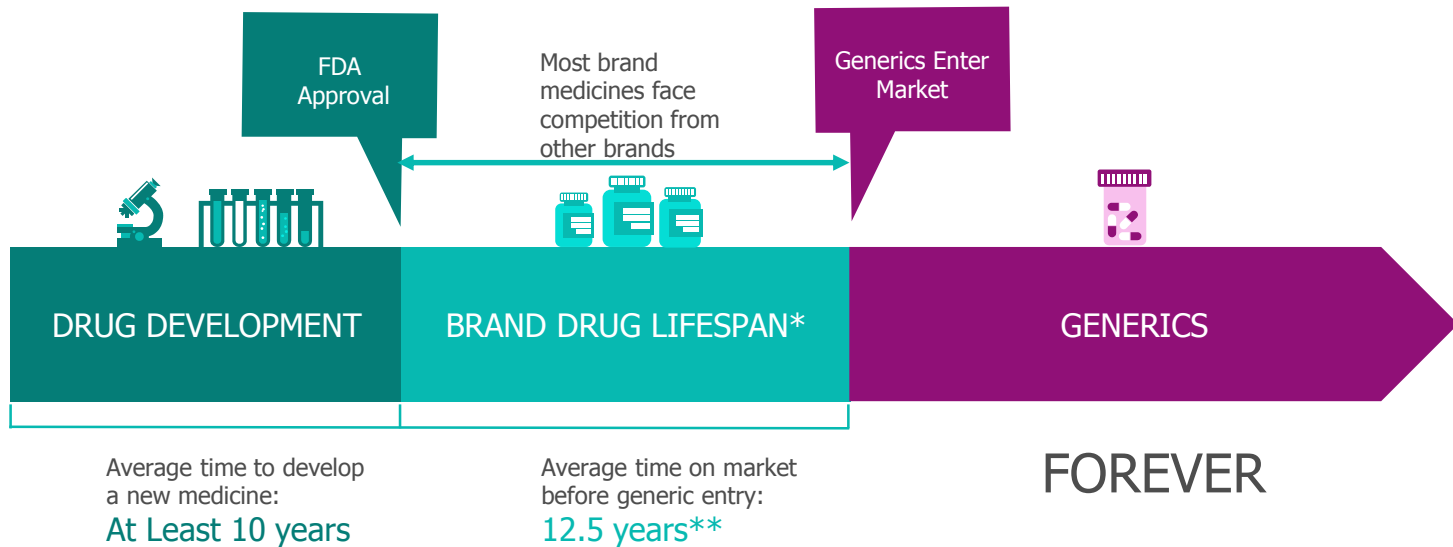
■ Cancer Treatment Spending
■ All Remaining Healthcare Spending

*Cancer medication invoice spending and does not include discounts
**2016 CMS total National Health Expenditures is a projection

Source 18: QuintileIMS Institute. Medicines Use and Spending in the U.S. A Review of 2016 and Outlook to 2021. Available at https://structurecms-staging-psyclone.netdna-ssl.com/client_assets/dwcnk/media/attachments/590c/6aa0/6970/2d2d/4182/0000/590c6aa069702d2d41820000.pdf?1493985952. Page 32. Accessed July 2017.

Source 18 a: CMS Projected National Healthcare Expenditure Data. Available at <https://www.cms.gov/research-statistics-data-and-systems/statistics-trends-and-reports/nationalhealthexpenddata/nationalhealthaccountsprojected.html>. Accessed November 2017.

Additional Cost Savings to the Healthcare System are Realized Once New Medicines Become Generic



*Brand drug market share generally declines rapidly after generic entry

**For brand medicines with more than \$250 million in annual sales in 2008 dollars, which account for 82% of the brand medicines analyzed

Source 19: Pharmaceutical Research and Manufacturers of America. Drug Discovery and Development: Understanding the R&D Process. Available at http://www.pfma.org/sites/default/files/pdf/rd_brochure_022307.pdf. Accessed August 2017.

Source 19 a: Grabowski H, Long G, Mortimer R, Boyo A. Updated trends in US brand-name and generic drug competition. J Med Economics. 2016;19(9):836-844. Available at <http://www.tandfonline.com/doi/abs/10.1080/13696998.2016.1176578?journalCode=ijme20>. Accessed August 2017.

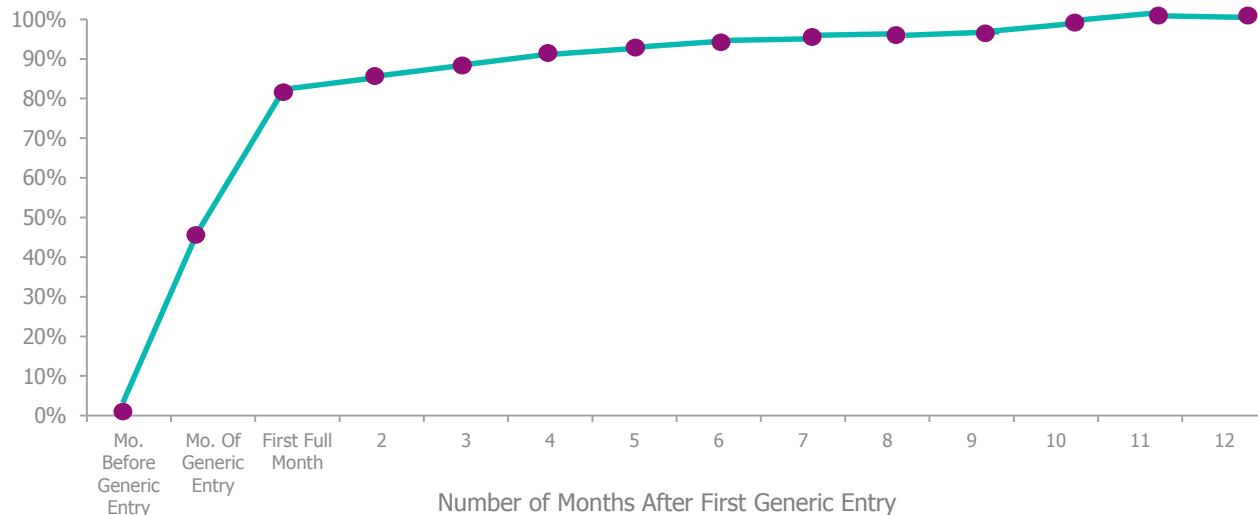


Newly Introduced Generics are Adopted Rapidly



When a generic version of a medicine becomes available for the first time, it captures an average of three-quarters of the market within 3 months

Average Generic Share of Total Use Following Launch of a Brand Medicine's First Generic, 2013-2014*



* Average monthly generic share of total standardized units of unique molecule/form combination

Source 20: Pharmaceutical Research and Manufacturers of America. Biopharmaceuticals in Perspective, Spring 2017. Available at <http://phrma-docs.phrma.org/files/dmfile/Biopharmaceuticals-in-Perspective-2017.pdf>. Calculated from Grabowski H, Long G, Mortimer R, Boyo A. Updated trends in US brand-name and generic drug competition. J Med Economics. Accessed August 2017.

The Virtuous Cycle of Medical Innovation at Work

Percent share of prescriptions

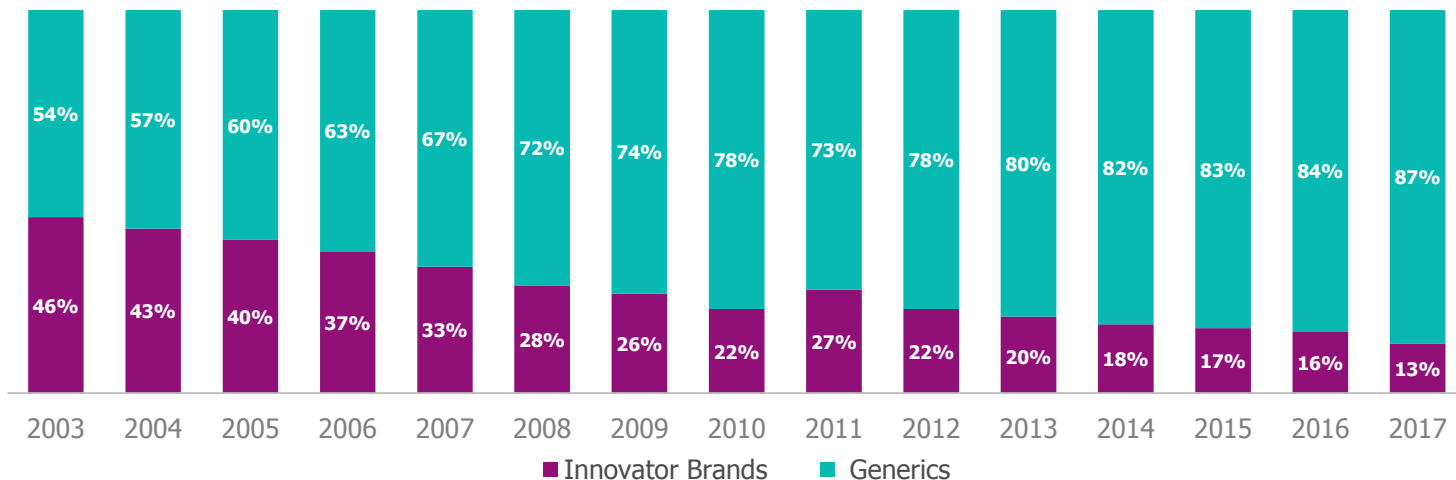


Chart Notes: Includes all prescriptions dispensed through retail pharmacies, including independent and chain drug stores, food store pharmacies and mail order as well as long-term care facilities. Generics include branded and unbranded generic medicines. Prescription counts are not adjusted for length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

Source 21: 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 August 2017.

Source 21 a: Long D. The US Pharmaceutical Market: Trends and Outlook. April 2017. Accessed August 2017.

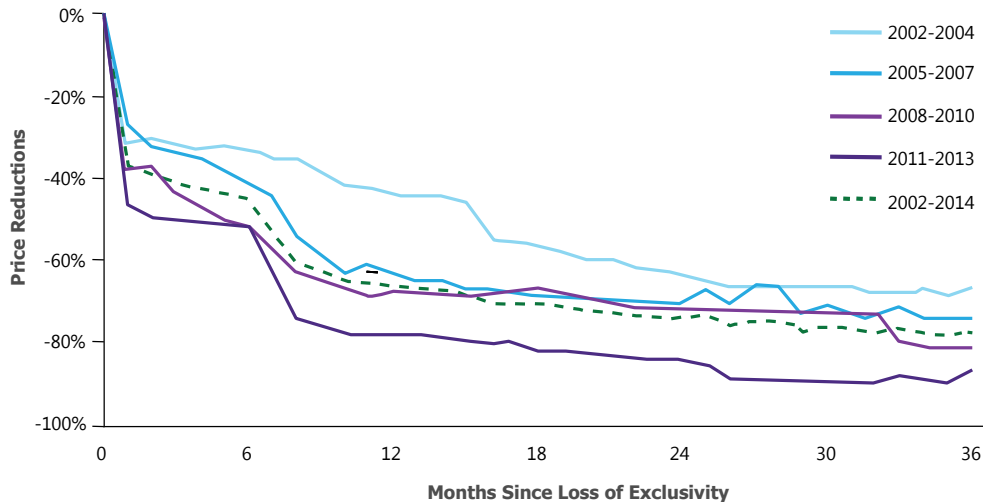


Generic Medicines Generated \$253 Billion in Savings for Patients in 2016



Generic entries have exhibited steeper and faster priced reductions over time

Monthly Price Reductions for Oral Medicines after Loss of Exclusivity



“The savings created by generic copies free up resources to invest in new treatments – creating headroom for innovation – and resulting in significant progress against some of the most costly and challenging diseases.”

– Pharmaceutical Research and Manufacturers of America (PhRMA)

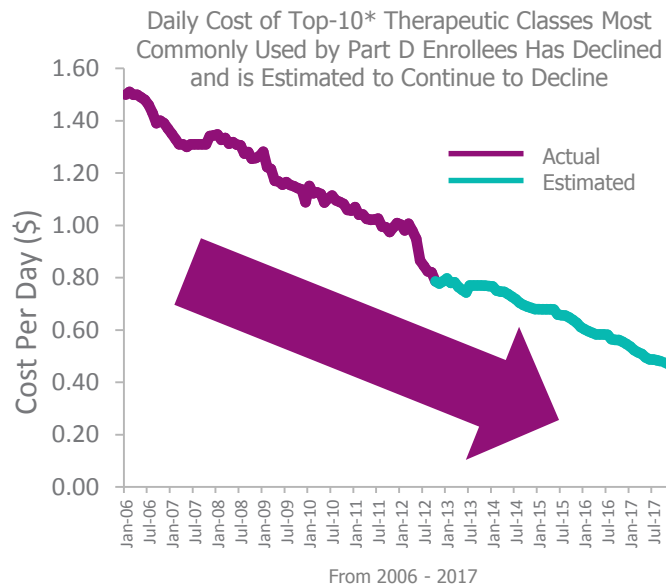
Source 22: IMS Health, National Sales Perspectives, March 2015. Available at <http://stateofreform.com/featured/2017/06/generic-drugs-generated-253-billion-savings-2016/>. Accessed July 2017.

Source 22 a: IMS Health. Price Declines after Branded Medicines Lose Exclusivity in the U.S. Available at <https://www.imshealth.com/files/web/IMSH%20Institute/Healthcare%20Briefs/PhRMA%20Generic%20Price%20Brief%20January%202016.pdf>. Accessed August 2017.

U.S. Prescription Drug Prices Fall Significantly Over Time Once Generic, Promoting Innovation and Affordability

Medicine	Year	Brand Price	Generic Price	% Change
DOXIL® Doxorubicin	2013	\$5,594	\$162	-97%
XELODA® Capecitabine	2013	\$3,401	\$1,249	-63%
FEMARA® Letrozole	2011	\$686	\$15	-98%
GEMZAR® Gemcitabine	2011	\$1,600	\$102	-94%
ELOXATIN® Oxaliplatin	2008	\$11,354	\$341	-97%

Figures represent the monthly cost of the brand name product versus the generic



*The 10 therapeutic classes most commonly used by Part D enrollees in 2006 were: lipid regulators, angiotensin converting-enzyme inhibitors, calcium channel blockers, beta blockers, proton pump inhibitors, thyroid hormone, angiotensin II, codeine and combination products, antidepressants, and seizure disorder medications.

Source 23: IMS Analysis of PhRMA, May 2015; Kleinrock M. Daily cost of Medicare Part D. December 2013 Update. December 2013. IMS Institute for Healthcare Informatics.

Source 23 a: Celgene data on file.



Accelerations of Loss of Exclusivity Continue to Drive Substantial Price Decreases and Expected Resulting Savings in the Next 5 Years, Including Biosimilars

The impact of patent expiries has been relatively unchanged for the past three years but is expected to increase sharply

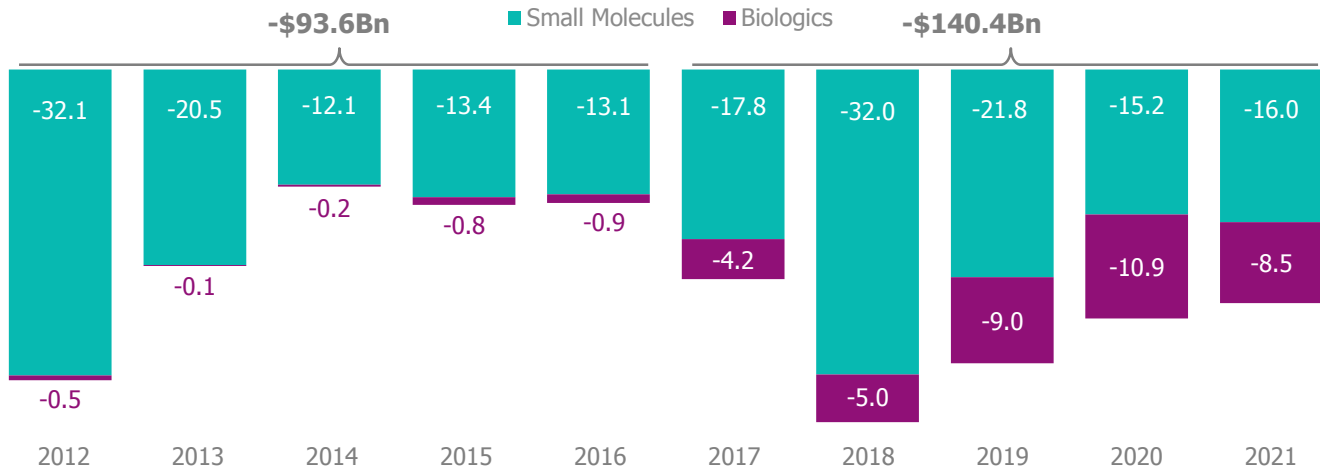


Chart notes: Lower brand spending based on invoice prices. Historic impacts from QuintilesIMS National Sales Perspectives, forecast impacts are modeled by projecting individual products sales growth to the point of patent expiry and then modeling expected impact based on historical analogues and actual data for in-progress events.

Source 24: QuintilesIMS Institute. Medicines Use and Spending in the U.S. A Review of 2016 and Outlook to 2021. Available at https://structurecms-staging-psyclone.netdna-ssl.com/client_assets/dwonk/media/attachments/590c/6aa0/6970/2d2d/4182/0000/590c6aa069702d2d41820000.pdf?1493985952. Page 32. Accessed July 2017.

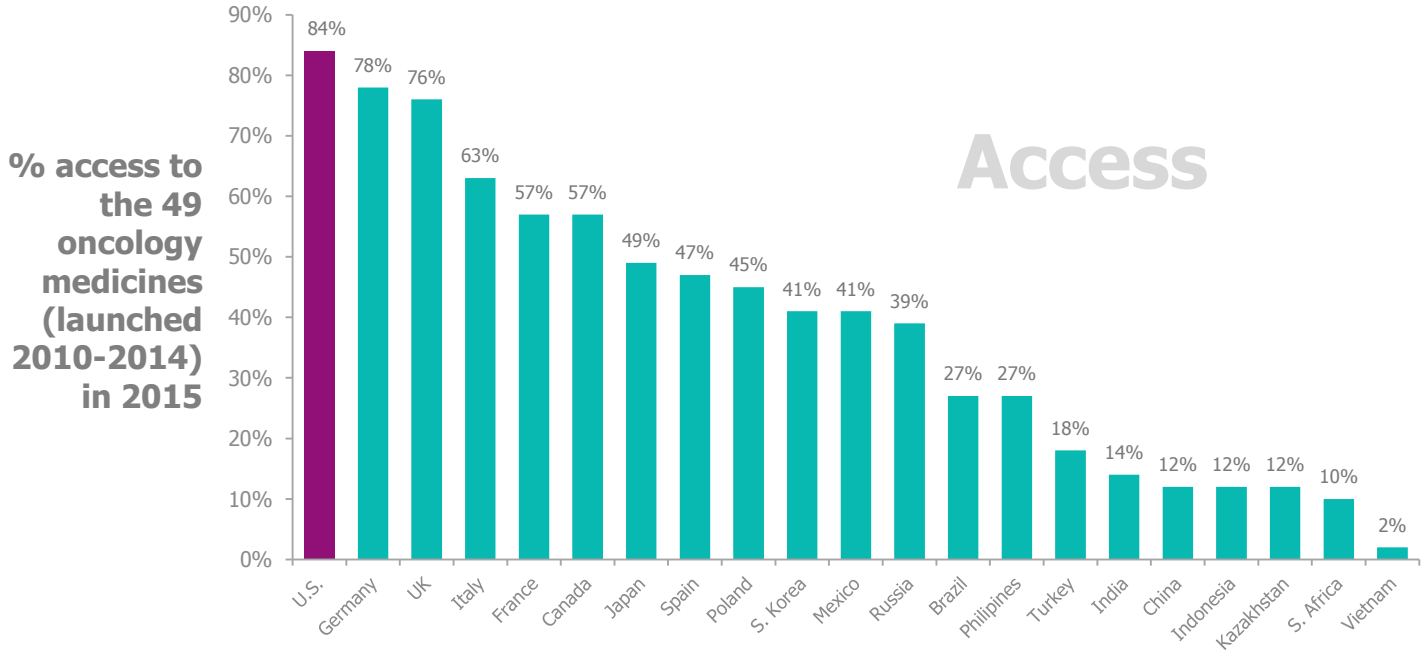


Barriers to Access

Patients in the U.S. Have Greater Access to Medicines When Compared to Other Highly Developed Countries



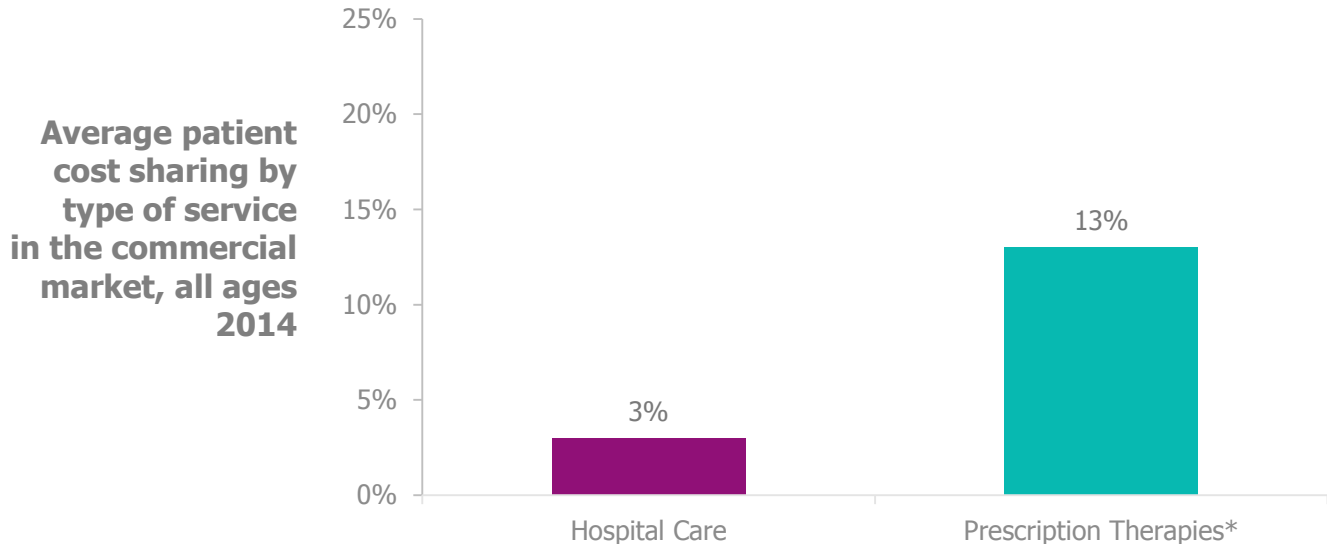
2015 Availability of Oncology Medicines Launched 2010-2014



Access

Source 25: IMS Institute for HealthCare Informatics. "Global Oncology Trend Report. A Review of 2015 and Outlook to 2020." Available at <https://morningconsult.com/wp-content/uploads/2016/06/IMS-Institute-Global-Oncology-Report-05.31.16.pdf>. Accessed August 2017.

Patients Pay More For Prescription Medicines Than Other Medical Services



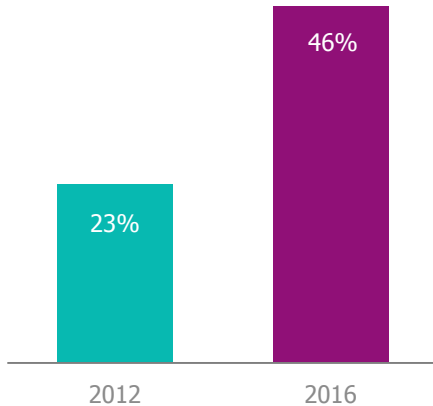
* **Note:** Prescription drug spending includes brand and generic ingredients, pharmacy and distribution costs for retail prescriptions. Hospital care includes inpatient and outpatient care.

Source 26: Pharmaceutical Research and Manufacturers of America. Biopharmaceutical Research in Perspective, Spring 2017. Calculated through Avalere Health analysis of the U.S. Department of Health and Human Services, Agency for Healthcare Research and Quality, Medical Expenditure Panel Survey, 2014. Available at <https://meps.ahrq.gov/mepsweb>. Accessed May 2017. Analysis includes individuals with any source of health care coverage, public or private; this includes individuals who had health coverage without coverage for prescription drugs, which can be expected to account for less than 2% of those with health coverage. Accessed August 2017.

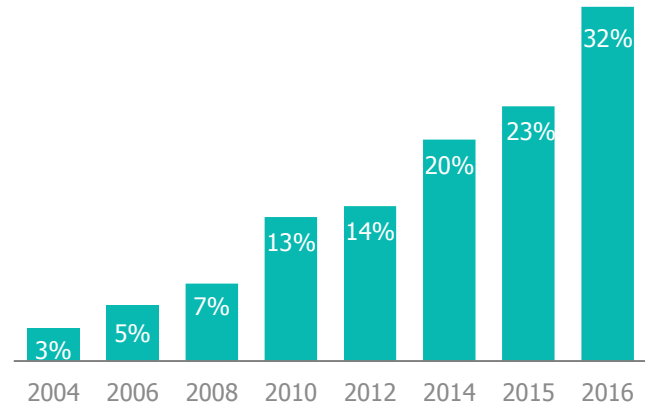


Patients in the U.S. are Facing Rising Out-of-pocket Costs and Other Barriers to Care

Percent of plans with deductibles on prescription medicines



The use of four or more cost sharing tiers is becoming more common on employer plans



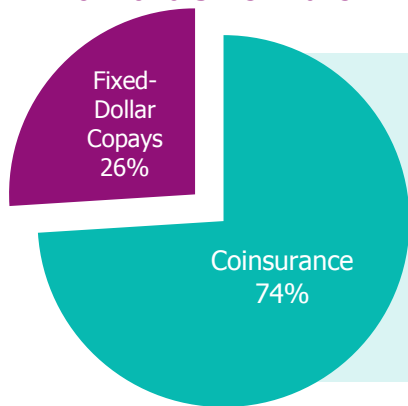
Source 27: PWC, Health and Well-Being Touchstone Survey, June 2016. Available at <https://www.pwc.com/us/en/hr-management/publications/assets/pwc-touchstone-2017.pdf>. Accessed August 2017.

Source 27 a: Kaiser Family Foundation/Health Research & Educational Trust, Employer Health Benefits: 2015 Annual Survey. Available at <http://files.kff.org/attachment/Report-Employer-Health-Benefits-2016-Annual-Survey>. Accessed October 2017.

Plans Often Charge Patients a Percentage of a Medicine's Total Cost Rather Than Fixed-Dollar Copays

In the most frequently purchased type of Health Insurance Exchange plan, coinsurance for certain medicines is common: 74% of these plans require enrollees to pay a percentage of a specialty tier medicine's total cost, with 36% of these plans requiring patients to pay coinsurance of more than 30% of the cost.

Cost Sharing in Specialty Tiers of 2016 Silver Plans*



COINSURANCE

Is a percentage of costs a patient is responsible for paying with his or her own money (out of pocket). Coinsurance can make a patient's out-of-pocket costs difficult to predict – and potentially much higher – than fixed-dollar copays.

* Silver Plans are shown here because they account for a majority of Health Insurance Exchange enrollment. Plans subject different medicines to different levels of cost sharing, or "tiers". Medicines assigned to a "specialty tier" typically require the highest level of cost sharing.

Source 28: Pearson C F. Avalere Health Release Majority of Drugs Now Subject to Coinsurance in Medicare Part D Plans. Available at <http://avalere.com/expertise/managed-care/insights/majority-of-drugs-now-subject-to-coinsurance-in-medicare-part-d-plans> data conducted using Avalere Health's Data Frame database accessed here <http://avalere.com/business-intelligence>. Accessed August 2017.

Source 28 a: Pharmaceutical Research and Manufacturers of America. Biopharmaceuticals in Perspective. Spring 2016. Available at <http://phrma-docs.phrma.org/sites/default/files/pdf/chart-pack-biopharmaceuticals-in-perspective.pdf>. Calculated with Avalere Health PlanScape©, <http://go.avalere.com/acton/fs/blocks/showLandingPage/a/12909/p/p-00ed/t/page/fm/0> a proprietary analysis of exchange plan features, December 2015. This analysis is based on data collected by Managed Markets Insight & Technology, LLC. Accessed August 2017.



High Cost Sharing Reduces Adherence



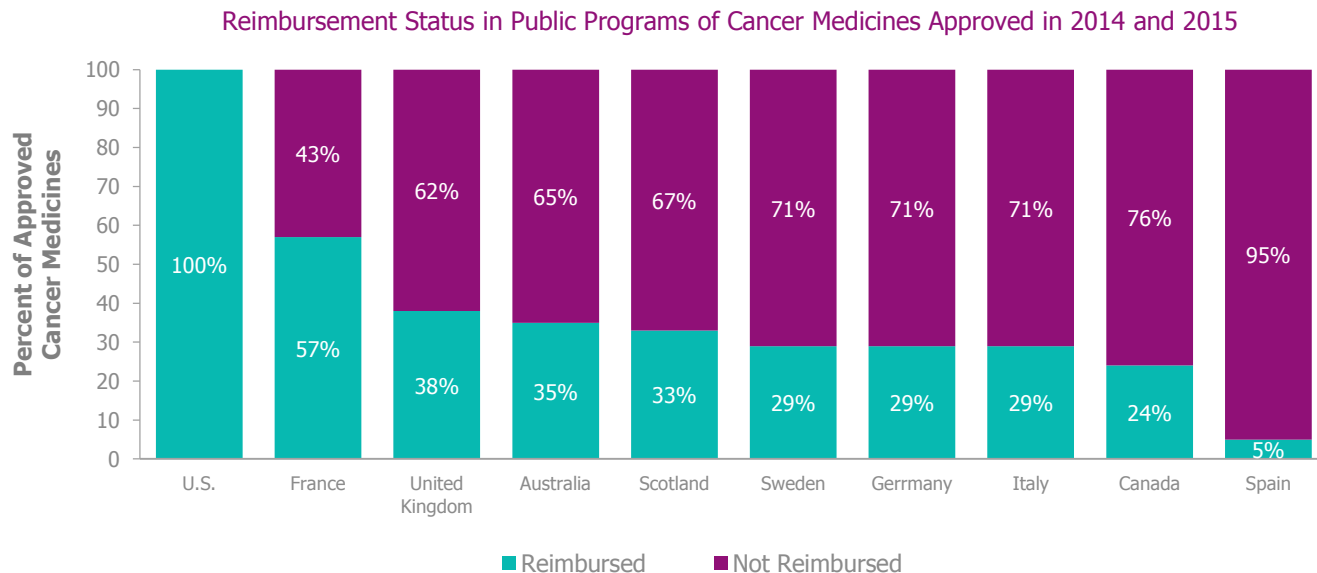
Percentage Change in Adherence From Doubling Medicine Copays from 1997 - 2000*



*Based on information from claims database

Source 29: Goldman D P, Joyce G F, Escarce J J, et al. Pharmacy benefits and the use of drugs by the chronically ill. JAMA. 2004;291(19):2344-2350. Available at <http://jamanetwork.com/journals/jama/fullarticle/198761>. Accessed September 2017.

Not All Cancer Medicines are Reimbursed Under Public Insurance Programs



Note: The categorization of “not reimbursed” does not mean that there is no patient access to these medicines, and there may be non-standard means for obtaining access to new medicines through special funds and submission of applications for approval outside of standard guidelines.

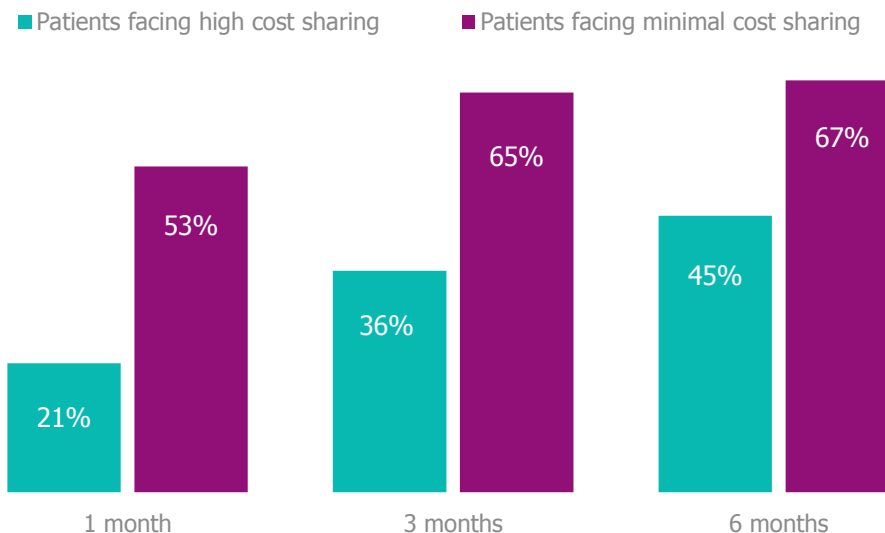
Source 30: IMS Institute for HealthCare Informatics. “Global Oncology Trend Report. A Review of 2015 and Outlook to 2020”. Available at <http://studyles.com/doc/2762501/cancer-medicines>. Accessed August 2017.



Patients Facing High Cost Sharing Commonly Do Not Initiate Treatment



Percentage of Chronic Myeloid Leukemia Patients Initiating Treatment

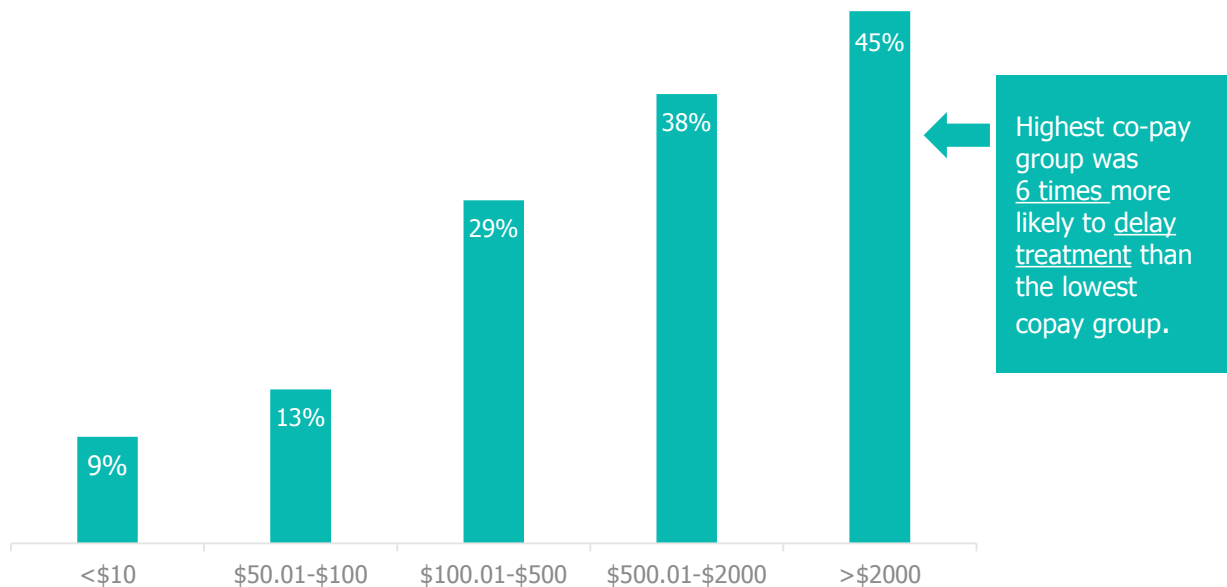


Source 31: Pharmaceutical Research and Manufacturers of America. Spring 2016. Available at <http://phrma-docs.phrma.org/sites/default/files/pdf/biopharmaceutical-industry-profile.pdf>. Cost Sharing and TKI Adherence. Journal of Clinical Oncology. 2016;22(3):188-197file:///C:/Users/nicole.ferrito/Downloads/jco.2013.52.9123.pdf. Accessed August 2017.

Source 31 a: Doshi JA, Li P, Ladage VP, Pettit AR, Taylor EA. Impact of cost sharing on specialty drug utilization and outcomes: a review of the evidence and future directions. Am J Managed Care. 2016;22(3):188-197. <http://www.ajmc.com/journals/issue/2016/2016-vol22-n3/Impact-of-Cost-Sharing-on-Specialty-Drug-Utilization-and-Outcomes-A-Review-of-the-Evidence-and-FutureDirections>. Accessed March 2016.

Cancer Patients Abandon Treatment Due to High Cost Sharing Hurdles

Oral Oncolytic Abandonment Rate by Patient Out-of-Pocket Amount



Source 32: Doshi JA, Li P, Huo H, Pettit AR, Armstrong K. Higher patient cost sharing is associated with prescription abandonment and delay in fills of novel oral oncolytic prescriptions. Proceedings of the 22nd Annual International Meeting International Society of Pharmacoeconomics and Outcomes Research; 2017 May; Boston, MA. Abstract available at <https://www.ispor.org/ScientificPresentationsDatabase/Presentation/73657?pdfid=49504>. Accessed September 2017.



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5

WORKING TOWARD A WORLD FREE FROM CANCERS


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 create the negative impact of halting medical progress and advancements in cancer, including its effect on survival, death, prosperity and progress.

The cost and commitment necessary to keep our promise of a world free from cancers is increasing – and the investment required is not keeping up.

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 medical progress in the 21st 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

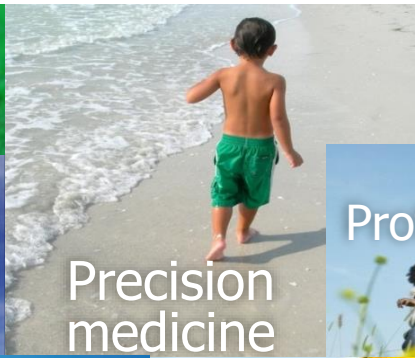
A Collaborative Ecosystem is Necessary to Accelerate Innovation



What's at Stake ...



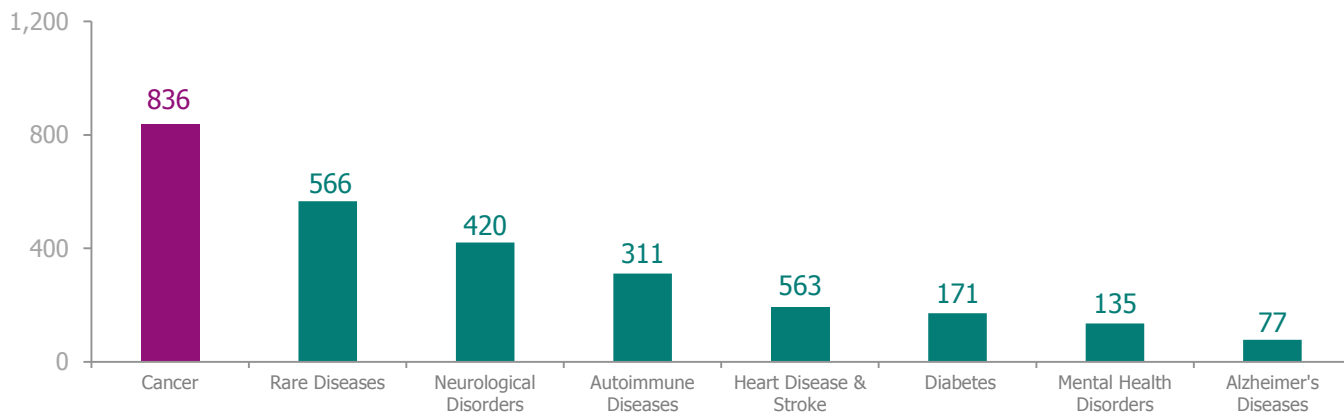
Today's investments in healthcare and R&D are working toward a world **free from cancers** as we know them today for our children and our children's children.



Maximizing the Promise of Science: 7,000+ Medicines in Development Globally in 2016

Biopharmaceutical researchers are working on new medicines* for many diseases, including:

Number of medicines



*Defined as single products that are counted exactly once regardless of the number of indications pursued

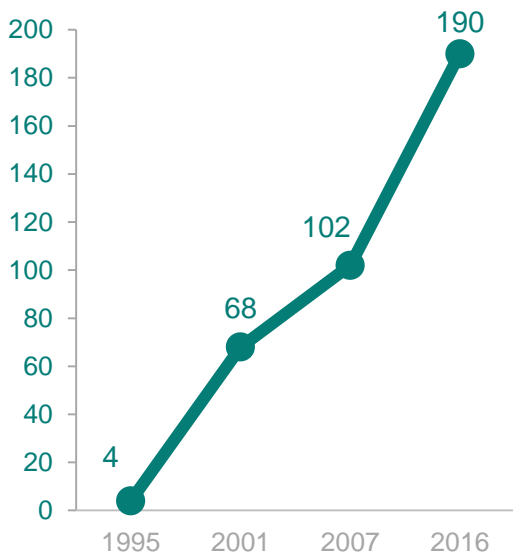
Source 1: Biopharmaceuticals in Perspective, Spring 2017. Available at <http://phrma-docs.phrma.org/files/dmfile/Biopharmaceuticals-in-Perspective-2017.pdf>. Calculated from Adis R&D Insight Database. Accessed August 2017.



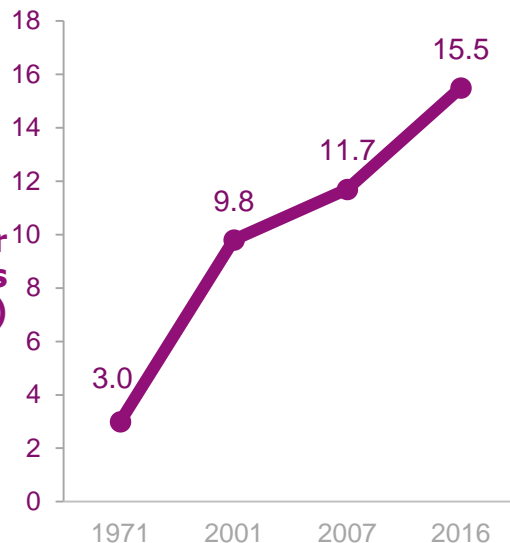
More New Therapies May Lead to More Survivors




Number of cancer therapies approved by year*




Cancer survivors (millions)



*Cumulative number of cancer therapies approved by year starting from 1995 through November 2016

Source 2: FDA. Approved Drugs for Oncology. Available at <https://www.centerwatch.com/drug-information/fda-approved-drugs/therapeutic-area/12/oncology>. Accessed August 2017.

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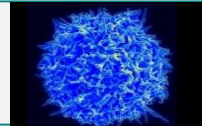
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Major Scientific Advances in Cancer Treatment Pipeline Give Patients Hope

The cancer pipeline is ripe with innovative therapeutic options. Emerging combinations of medicines hold particular promise for controlling and killing cancer cells.

Chimeric Antigen Receptor (CAR) T-cell Adoptive Cell Therapy

involves the modification of individuals' immune-boosting T-cells to target and kill blood cancer cells



Cancer Metabolism-Targeting Drugs

disrupt cancer cell metabolism and can impede cancer cell growth.

Oncolytic Viral Therapies

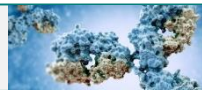
zero in on cancer cells, replicate and cause them to rupture stimulating an immune response

CRISPR Gene Editing

allows researchers to manipulate cancer cell function

Immunotherapies

help target and kill cancer cells by "releasing the breaks" on the immune system



“ We’re really seeing the fruits of many years of research into what drives cancer and how it interacts with the immune system to defeat it and survive. ”

Dr. Louis Weiner
Director of the Georgetown Lombardi Comprehensive Cancer Center

Source 3: World Medical innovation Forum: Cancer. "Disruptive Dozen 2016." http://worldmedicalinnovation.org/wp-content/uploads/2016/04/Partners-FORUM-2016-BROCHURE-D12-Cancer-160422_0942_FREVI-WEB-X3-SM-SPREAD.pdf:

Source 3 a: McGinley, L. "The list of cancers that can be treated by immunotherapy keeps growing." The Washington Post. <https://www.washingtonpost.com/news/to-your-health/wp/2016/04/19/breakthrough-cancer-therapy-shows-growing-promise/>

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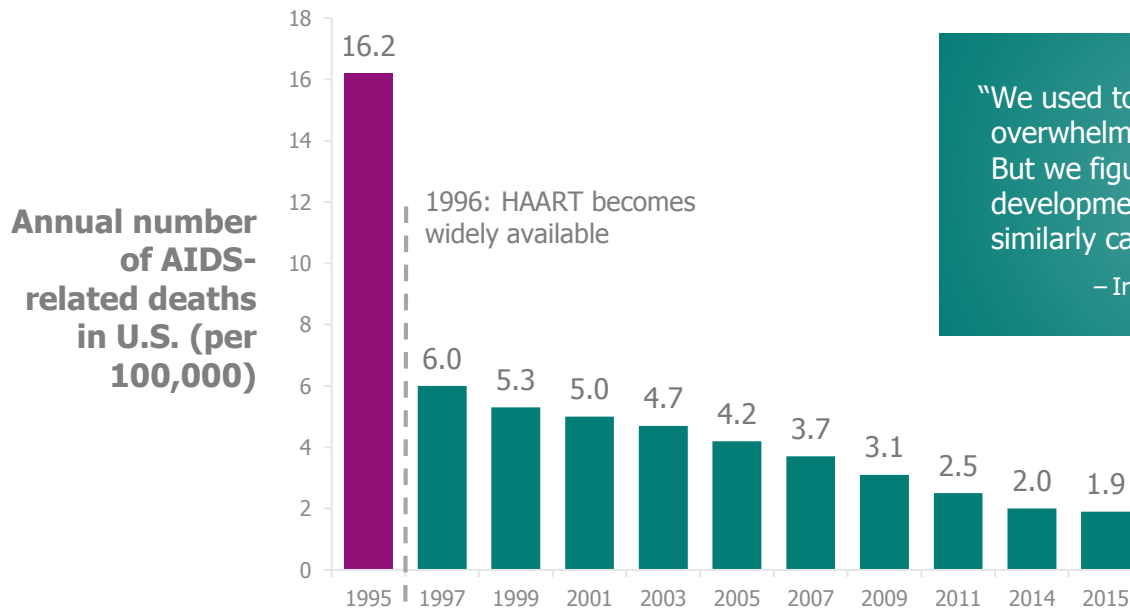
Source 3 c: National Cancer Institute. Oncolytic Virus Therapy Shows Benefit in Patients with Melanoma. Available at . <https://www.cancer.gov/news-events/cancer-currents-blog/2015/oncolyticvirus-melanoma>. Accessed August 2017.



Innovations in Cancer Treatment Could Echo HIV/AIDS Successes



As HIV/AIDS treatments improved, total cost of care decreased



“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, MD, MBA, FACP, Aetna

Source 4: Pharmaceutical Research and Manufacturers of America. PhRMA Chart Pack: Cancer Medicines Value in Context. Spring 2014. Chapter 1, slide 21. Available at <http://www.phrma.org/sites/default/files/pdf/cancer-chart-pack-5-22-14.pdf>. Accessed August 2017.

Source 4 a: Pharmaceutical Research and Manufacturers of America. PhRMA Chart Pack: Biopharmaceuticals in Perspective. Available at <https://www.noexperiencenecessarybook.com/XGxbw/microsoft-powerpoint-cancer-chart-pack-5-22-14-final.html>. Accessed August 2017.

Source 4 b: Centers for Disease Control. Health United States, 2016. Available at <https://www.cdc.gov/nchs/data/hus/hus16.pdf>. Accessed August 2017.

Hepatitis C (HCV): Cure Rates are Rising

HCV GENOTYPE 1 PREVALENCE

2.4 million people

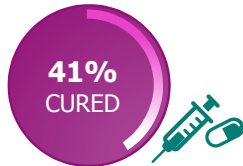
HAVE GENOTYPE 1 HCV



1ST GENERATION

2001-2010

Interferon and Ribavirin
(IF-R)

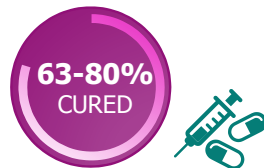


48-week treatment

2ND GENERATION

2011-2013

Protease Inhibitors with
FIN



24-48-week treatment

3RD GENERATION

2013-2014

Polymerase Inhibitors with
IFN



12-week treatment

4TH GENERATION

2014-2015

Combination Antiviral
Therapies



8-12-week treatment
INTERFERON FREE

Nearly **60 more medicines** are in development offering to bring more coherent treatment options for patients with shorter treatment duration and few side effects. **INTERFERON FREE.**

Source 5: Pharmaceutical Research and Manufacturers of America. PhRMA Chart Pack: Biopharmaceuticals in Perspective. Spring 2017. Available at <http://phrma-docs.phrma.org/files/dmfile/Biopharmaceuticals-in-Perspective-2017.pdf>. Accessed August 2017.

Source 5 a: Pharmaceutical Research and Manufacturers of America. Decades of Innovation in Chronic Diseases. 2006 – 2016. Available at <http://phrma-docs.phrma.org/sites/default/files/pdf/decade-of-innovation-chronic-disease.pdf>. Calculated with Adis R&D Insight Database. Accessed January 2016. Accessed August 2017.



Novel Targeted Chronic Myeloid Leukemia (CML) Therapies Help Patients Live Longer and Healthier

Over the period 2001-2013:



Three New CML Therapies Contributed to



50%
decline in people
dying from CML*



*Number of new deaths per 100,000 people, age-adjusted

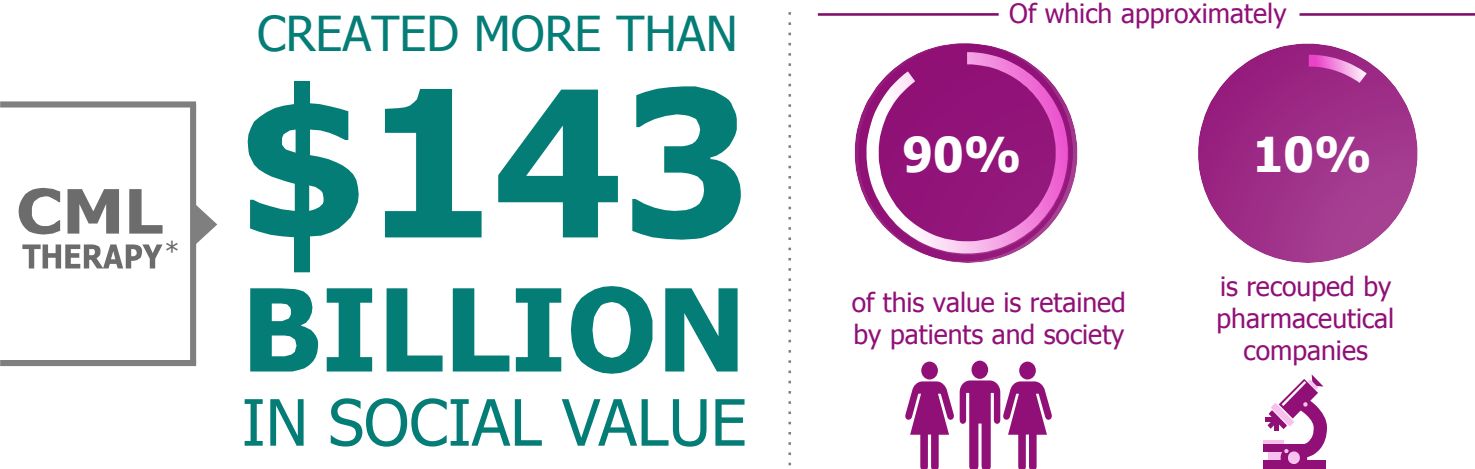
Source 6: National Cancer Institute, Surveillance Epidemiology and End Results (SEER). Available at <http://seer.cancer.gov/statfacts/html/ld/cmly.html>. Accessed August 2017.

Source 6 a: U.S. Food and Drug Administration. Gleevec. Available at <http://www.accessdata.fda.gov/scripts/cder/daf/index.cfm?event=overview.process&AppNo=021335>. Accessed August 2017.

Source 6 b: U.S. Food and Drug Administration. Sprycel. Available at <http://www.accessdata.fda.gov/scripts/cder/daf/index.cfm?event=overview.process&AppNo=021986>. Accessed August 2017.

Source 6 c: U.S. Food and Drug Administration. Tasigna. Available at <http://www.accessdata.fda.gov/scripts/cder/daf/index.cfm?event=overview.process&AppNo=022068>. Accessed August 2017.

Innovative Chronic Myeloid Leukemia (CML) Therapies Yielded Large Economic Returns



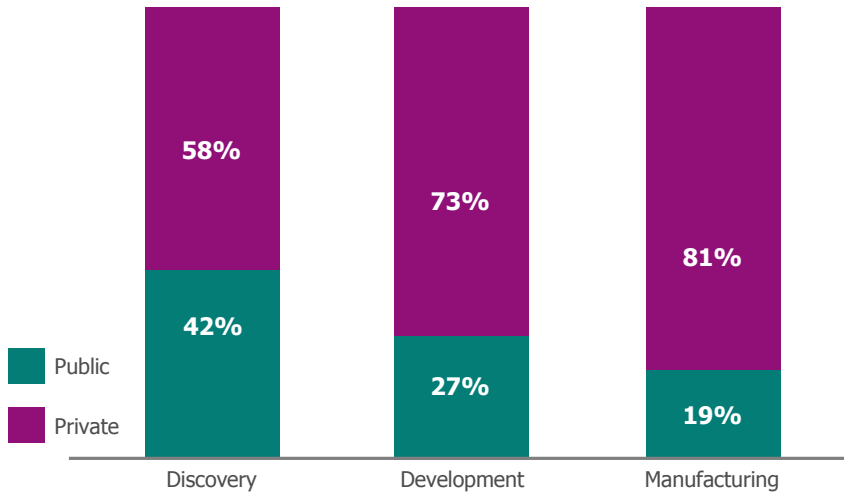
*Based on first- and second-line tyrosine kinase inhibitors

Source 7: Yin W, Penrod JR, Maclean JR, et al. Value of survival gains in chronic myeloid leukemia. *Am J Manag Care* 2012;18(11Suppl):S257-64. Available at http://www.ajmc.com/journals/supplement/2012/a386_12nov_oncology/a386_12nov_onclogy_yin_s257to64/P-3. Accessed August 2017.

Biopharmaceutical Research Companies Play a Pivotal Role in Drug Discovery and Development

The private sector makes the largest R&D contributions in translating basic research findings into therapeutic advances for patients

Share of Contributions to Key R&D Milestones for the Most Transformative Medicines of the Past 25 Years



“Today, most important developments in medical science typically begin in laboratories, such as the discovery of specific new biological molecules, processes, or pathways, or innovative applications of existing knowledge ... [The] real impact for public health generally comes after several more significant steps – including further R&D, testing, approval by appropriate regulatory bodies (such as the FDA), manufacturing and distribution.”

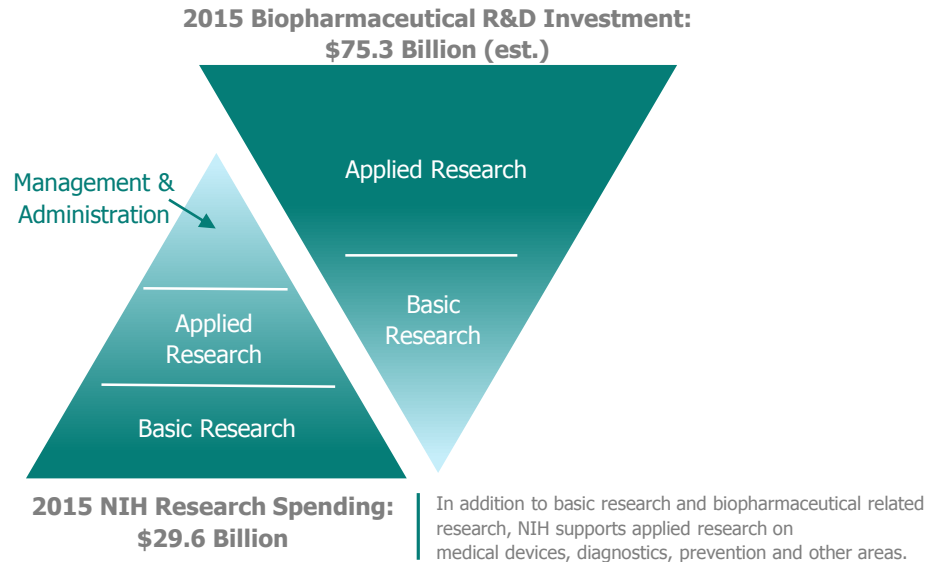
– NIH, Office of Intramural Research (OIR), Office of Technology Transfer (OTT)

Source 8: Chakravarthy R, Cotter K, DiMasi J, Milne C-P, Wendel N; Tufts Center for the Study of Drug Development. Public and private sector contributions to the research & development of the most transformational drugs of the last 25 years. Available at <http://csdd.tufts.edu/files/uploads/PubPrivPaper2015.pdf>. Published January 2015. Accessed August 2017.

Source 8 a: Biopharmaceutical Research and Development. The Process Behind New Medicines. Available at http://phrma-docs.phrma.org/sites/default/files/pdf/rd_brochure_022307.pdf. Accessed August 2017.

Biopharmaceutical Companies do the Vast Majority of Research to Translate Basic Science into New Medicines

While basic science is often initiated in government and academia, it is biopharmaceutical firms that provide the necessary critical mass, expertise and experience needed to develop new medicines.



Source 9: Chakravarthy R, Cotter K, DiMasi J, Milne C-P, Wendel N; Tufts Center for the Study of Drug Development (CSDD). Public and private sector contributions to the research & development of the most transformational drugs of the last 25 years. <http://csdd.tufts.edu/files/uploads/PubPrivPaper2015.pdf>. Published January 2015. Accessed August 2017.

Source 9 a: Pharmaceutical Research and Manufacturers of America. Biopharmaceuticals in Perspective, Spring 2017. Available at Accessed August 2017.



Biopharmaceutical Companies are Committed to Advancing Precision Medicine

In recent years we have seen remarkable advances in targeted therapy, and the R&D pipeline has never been more promising.

IN THE PIPELINE

73% & 42%

OF CANCER MEDICINES

OF NEW MEDICINES

have potential to be
**PRECISION
MEDICINES**



**MORE THAN
25%
OF NEW
MEDICINES**



**APPROVED BY FDA
IN 2016
WERE
PRECISION
MEDICINES**

“Oncology is on fire with [precision medicine], with treatment selections based on individual molecular characteristics.”

—Janet Woodcock

Director of Food and Drug Administration’s
Center for Drug Evaluation and Research

Source 10: Pharmaceutical Research and Manufacturers of America. Value of Personalized Medicines. Spring 2015. Calculated with Tufts Center for the Study of Drug Development (CSDD). Personalized medicine gains traction but still faces multiple challenges. Tufts CSDD Impact Rep. 2015;17(3). Accessed August 2017.

Source 10 a: Salazar D. PhRMA: Over 800 cancer treatments in pipeline. Available at <http://www.drugstorenews.com/article/phrmaover00cancer treatments pipeline>. Accessed August 2017.

Source 10 b: Precision Medicine Coalition. Personalized Medicines at FDA 2016 Progress Report. Available at <http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PM-at-FDA.pdf>. Accessed August 2017.

Ongoing Research and Use of a Medicine Over Time Reveal Additional Value That May Not Have Been Recognized Initially



Additional value may be realized over time

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

“The relative value of a given cancer treatment is likely to change over its lifetime ... the assessment of the value of any treatment must be dynamic and adapt to new medical information that may better inform its use, mitigate its toxicity, or modify its place in the treatment landscape.”

– American Society of Clinical Oncology

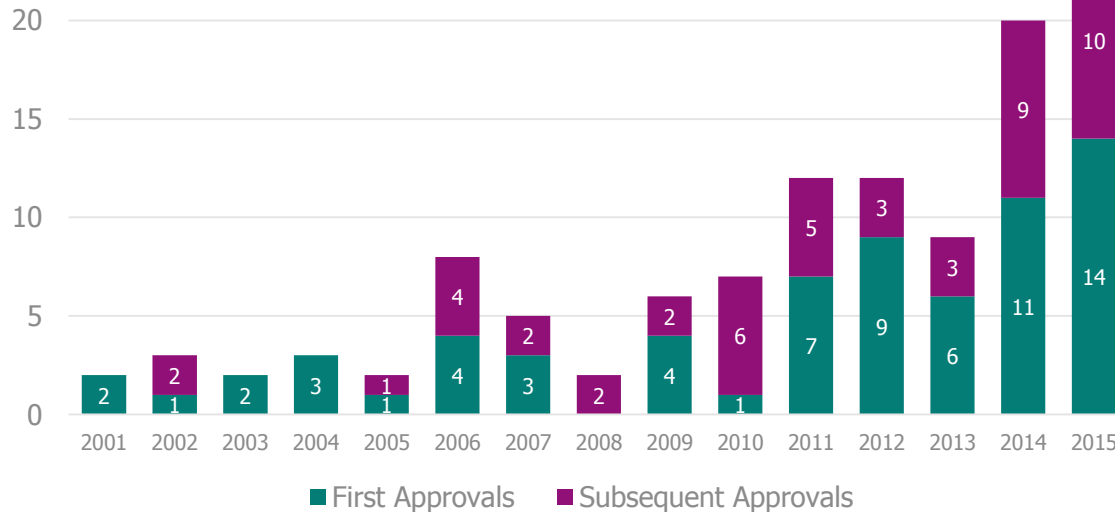
Source 11: Sweeney N Gross T F. Boston Associates. The Value of Innovation in Oncology: Recognizing Emerging Benefits Over Time White Paper May 2015. Available at <http://studyres.com/doc/2762501/cancer-medicines>. Accessed August 2017.

Source 11 a: Schnipper L E et al. American Society of Clinical Oncology Statement: A Conceptual Framework to Assess the Value of Cancer Treatment Options. Journal of Clinical Oncology. Available at <http://ascopubs.org/doi/full/10.1200/jco.2015.61.6706>. Accessed November 2017.

Over Time, Targeted Oncology Medicines Generate Additional Treatment Options for New Indications

Through ongoing research, our understanding of the genetic and molecular mutations driving cancer cell growth is uncovering commonalities among cancer types and the medicines used to treat them.

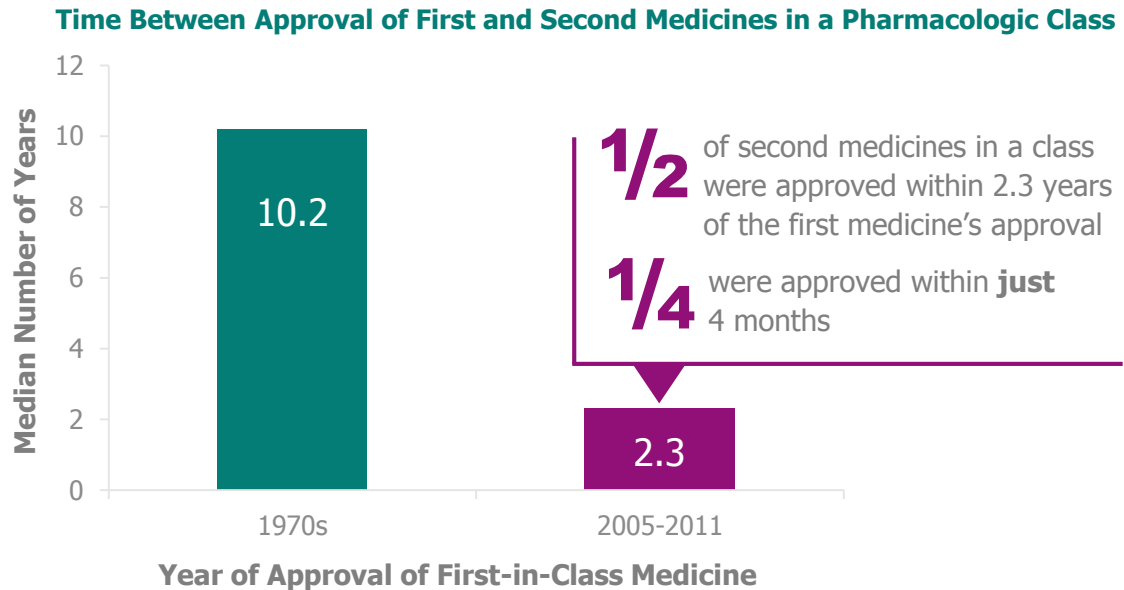
Targeted Oncology Medicine FDA Approval Type by Year



Source 12: IMS Institute for Healthcare Informatics, "Medicines Use and Spending in the U.S.: A Review of 2015 and Outlook to 2020"; CMS National health Expenditures Projections. April 2016. Available at file:///C:/Users/nicole.ferrito/Downloads/IMS_Institute_Medicines_Use_and_Spending_in_2015%20(2).pdf. Accessed August 2017.

Increasing Competition Within Therapeutic Categories

The time a medicine is the only therapy available in its pharmacologic class has declined from a median of more than 10 years in the 1970s to close to 2 years in the 2000s.



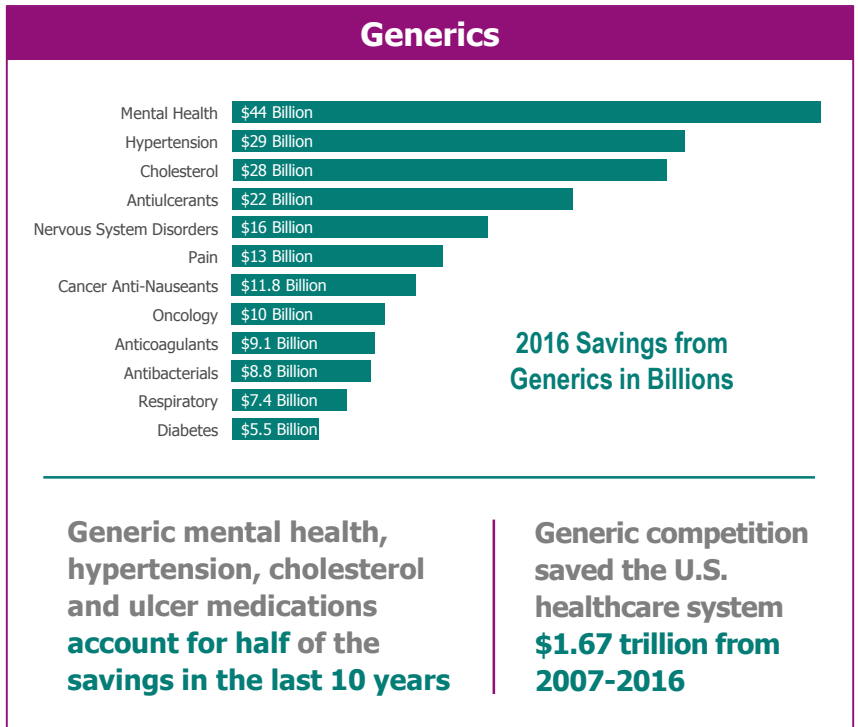
Source 13: 1970s data: Tufts Center for the Study of Drug Development, unpublished data, March 2010; 2005-2011 data: Tufts Center for the Study of Drug Development. First-in-class drugs in competitive development races with later entrants. Tufts CSDD Impact Rep. 2015;17(6). Accessed August 2017.

Source 13 a: Statista.com. Time between approval of first and second drugs in a therapeutic class. Available at <https://www.statista.com/statistics/215468/time-taken-for-approval-of-drugs-in-a-therapeutic-class-in-the-us/>. Accessed August 2017.

Source 13 b: Biopharmaceuticals in Perspective, Spring 2016. Available at <http://phrma-docs.phrma.org/files/dmfile/chart-pack-biopharmaceuticals-in-perspective4.pdf>. Calculated from Adis R&D Insight Database. Accessed August 2017.



Increased Competition Among Innovator Brands and Generics Helps to Reduce Costs for the Healthcare System



Source 14: QuintilesIMS. Treating hepatitis C virus infection. Available at <http://www.healthline.com/health/hepatitis-c/full-medication-list#5>. Accessed August 2017.

Source 14 a: Britt R. MarketWatch. Gilead to discount its pricey Sovaldi drug. Available at <http://www.marketwatch.com/story/gilead-to-discount-its-pricey-sovaldi-drug-2015-02-04>. Accessed August 2017.

Source 14 b: Gilead FDA Approval Timeline. Available at <http://www.gilead.com/medicines/productapprovaltimeline>. Accessed August 2017.

Continuing Research Reveals Greater Benefit in Targeted Patient Population: Gefitinib for Non-Small Cell Lung Cancer (NSCLC)

After approval, clinical studies in broader NSCLC patient population showed limited efficacy. However, ongoing research revealed that patients with a specific mutation demonstrated significant survival gains.

Accelerated approval as second- or third-line treatment based on tumor shrinkage

Indication limited to those currently benefiting from treatment due to ongoing clinical studies that fail to show significant survival

Approved for first-line use in patients with specific mutations (EGFR exon 19 deletions or exon 21 L858R substitutions)

2003

2005

2015

“Gefitinib is a textbook example of how much oncology has changed over the past decade. The trial to follow up and get full approval of gefitinib wasn’t designed with smart selection of patients based on mutations.”

–Dr. Lecia Sequist,
Medical Oncologist, Massachusetts
General Hospital

Source 15: National Cancer Institute. Timeline: “FDA Approval for Gefitinib.” <http://www.cancer.gov/about-cancer/treatment/drugs/fda-gefitinib> (Accessed May 2016). Accessed August 2017.

Source 15 a: Gefitinib Label. 2015. Available at https://www.accessdata.fda.gov/drugsatfda_docs/label/2015/206995s000lbl.pdf. Accessed August 2017.

Source 15 b: *Cancer Discovery* September 2015 5.896. Gefitinib Approved for EGFR-Mutated NSCLC. Available at <http://cancerdiscovery.aacrjournals.org/content/5/9/896.2>. Accessed August 2017.

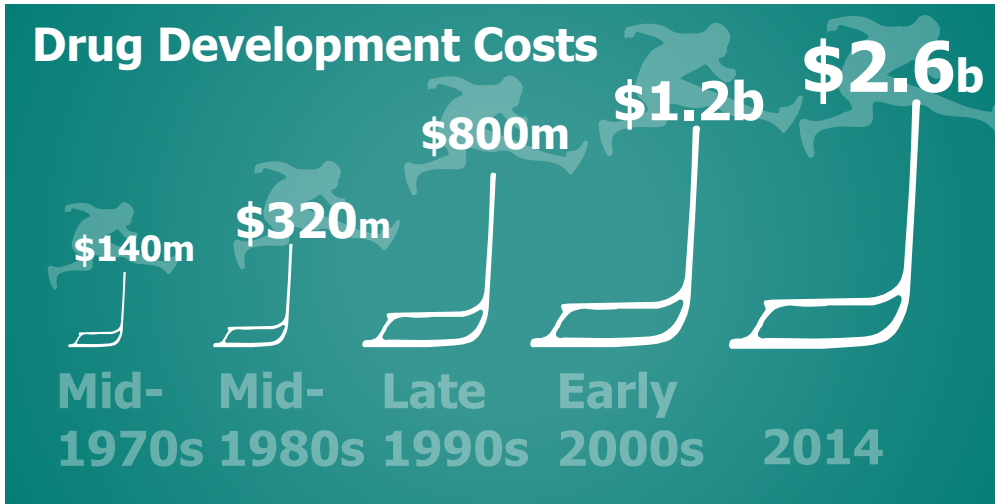
Source 15 c: Cancer Medicines: Value in Context, Summer 2016. Available at http://phrma-docs.phrma.org/files/dmfile/cancer_chart_pack3.pdf. Calculated from Adis R&D Insight Database. Accessed August 2017.



Drug Development Costs Continue to Climb



The average cost to develop one new approved therapy **more than tripled** between the late 1990s and 2014



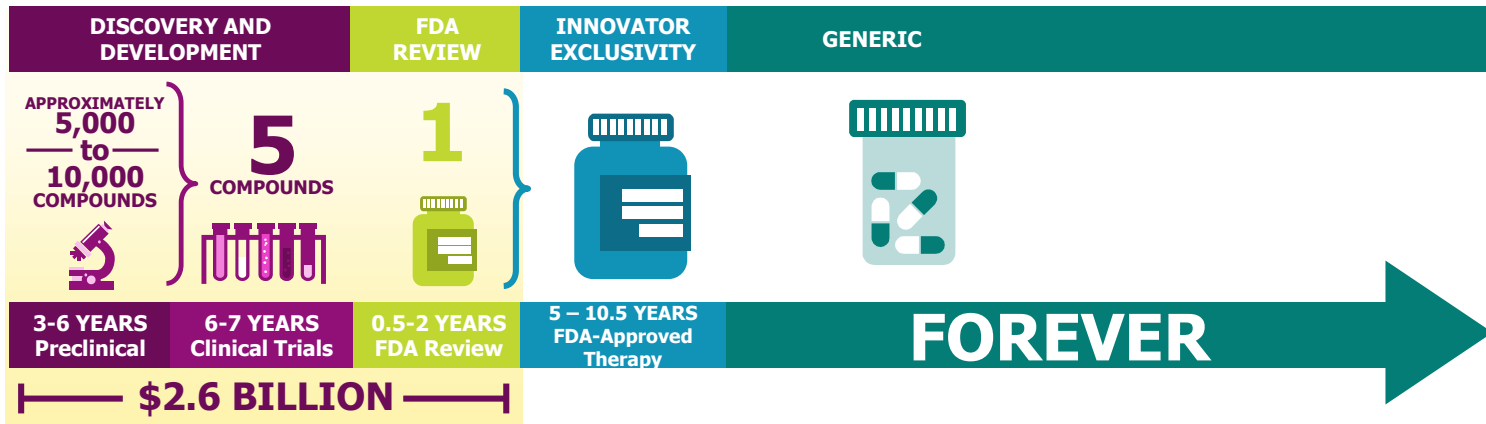
Source 16: Pharmaceutical Research and Manufacturers of America. 2013 Profile: biopharmaceutical research industry. Available at <http://www.phrma.org/sites/default/files/pdf/PhRMA%20Profile%202013.pdf>. Accessed August 2017

Source 16 a: DiMasi J, et al. Innovation in the pharmaceutical industry: New estimates of R&D costs. *Journal of Health Economics*. Available at http://csdd.tufts.edu/news/complete_story/pr_tufts_csdd_2014_cost_study. Accessed August, 2017.

Source 16 b: DiMasi J, et al. The price of innovation: new estimates of drug development costs. Available at <http://www.sciencedirect.com/science/article/pii/S0167629602001261?via%3Dihub>. Accessed August 2017.

Society Benefits from New Treatments and, Ultimately, Lower Costs Forever

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 17: Pharmaceutical Research and Manufacturers of America. Drug Discovery and Development: Understanding the R&D Process. Available at http://www.pfma.org/sites/default/files/pdf/rd_brochure_022307.pdf. Accessed August 2017.

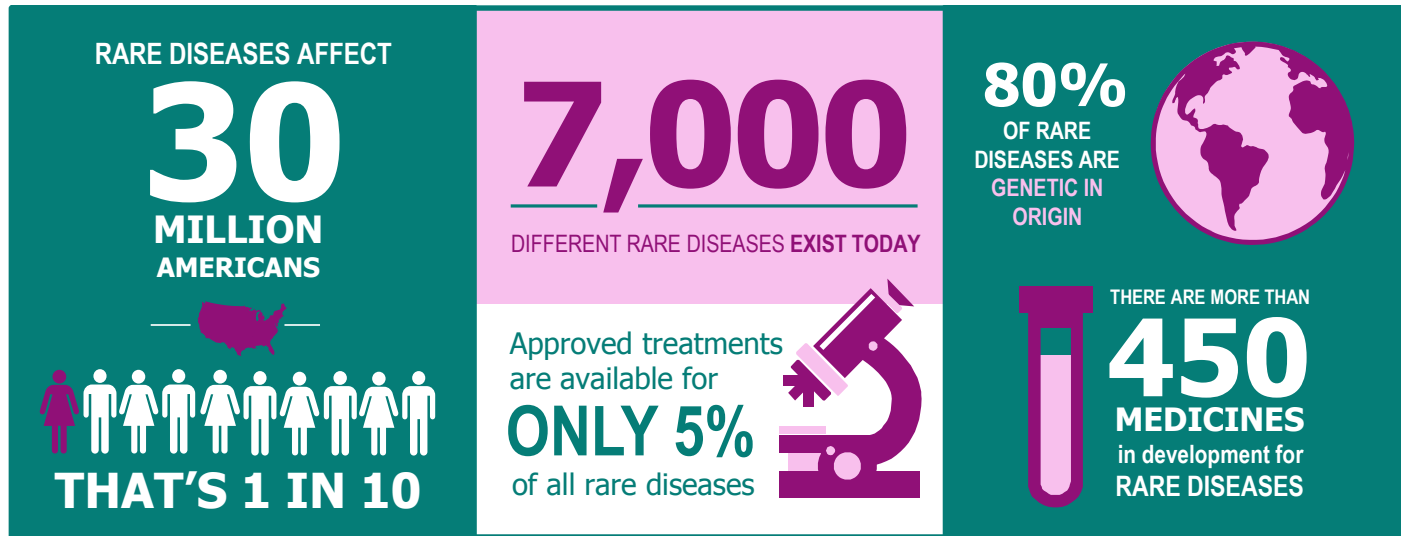
Source 17 a: DiMasi J. Journal of Health Economics. Innovation in the pharmaceutical industry: New estimates of R&D costs. Available at <http://dx.doi.org/10.1016/j.jhealeco.2016.01.012>. Accessed August 2017.

Source 17 b: U.S. Food and Drug Administration. Frequently Asked Questions on Patents and Exclusivity. Available at <https://www.fda.gov/drugs/developmentapprovalprocess/ucm079031.htm#howlongpatentterm>. Accessed September 2017.

Harnessing Innovation in Rare Disease



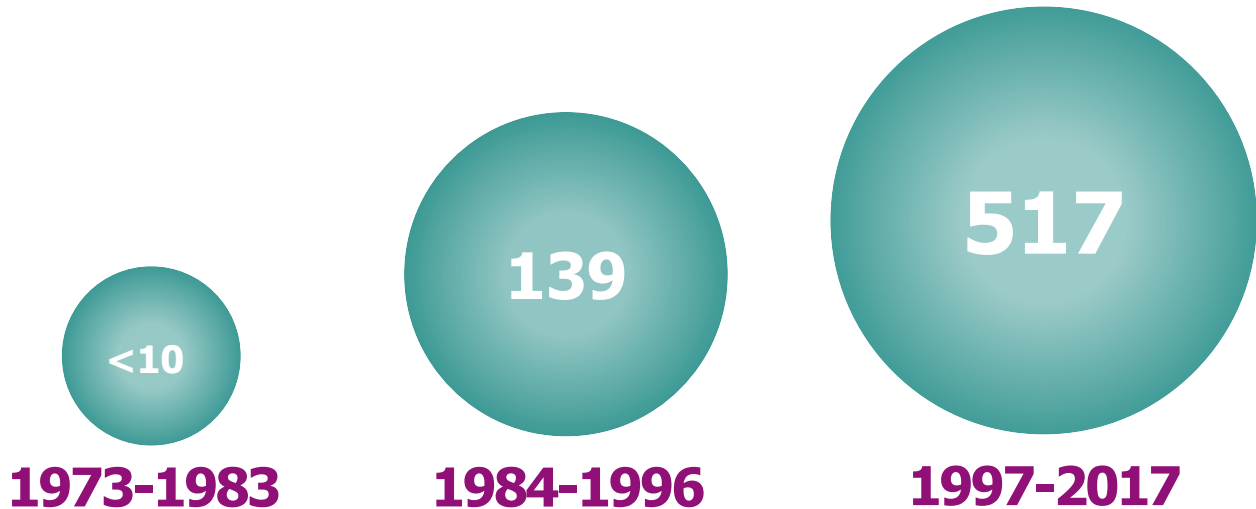
Since the passage of the Orphan Drug Act in 1983, we have seen tremendous advances in treatments for rare diseases. The rapid pace of the science holds significant promise for the future.



Source 18: Pharmaceutical Research and Manufacturers of America (PhRMA). "A Decade of Innovation in Rare Diseases." Available at <http://phrma-docs.phrma.org/sites/default/files/pdf/PhRMA-Decade-of-Innovation-Rare-Diseases.pdf>. Accessed July 2017.

U.S. Orphan Drug Act (1983) Impact on Innovation

Number of Drug Approvals for Rare Diseases

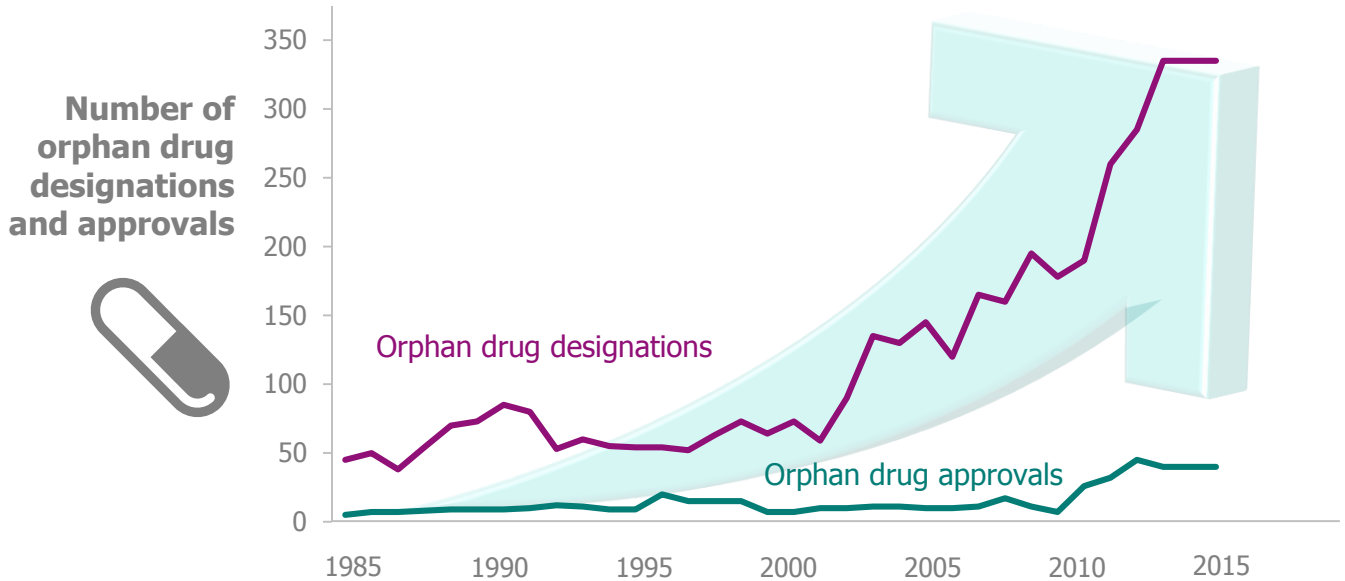


Source 19: U.S. Food and Drug Administration, Developing Products for Rare Diseases & Conditions. Available at <http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/default.htm>. Accessed August 2017.

Source 19 a: U.S. Food and Drug Administration, Office of Orphan Product Development, Orphan Drug Designations and Approvals Database, 1983-1996. Available at <https://www.accessdata.fda.gov/scripts/opdlisting/oopd/listResult.cfm>. Accessed August 2017.

Source 19 b: U.S. Food and Drug Administration, Office of Orphan Product Development, Orphan Drug Designations and Approvals Database, 1996-2017. Available at <https://www.accessdata.fda.gov/scripts/opdlisting/oopd/listResult.cfm>. Accessed August 2017.

Number of New Orphan Drug Designations Climb But Approvals Not Keeping Pace in the U.S.

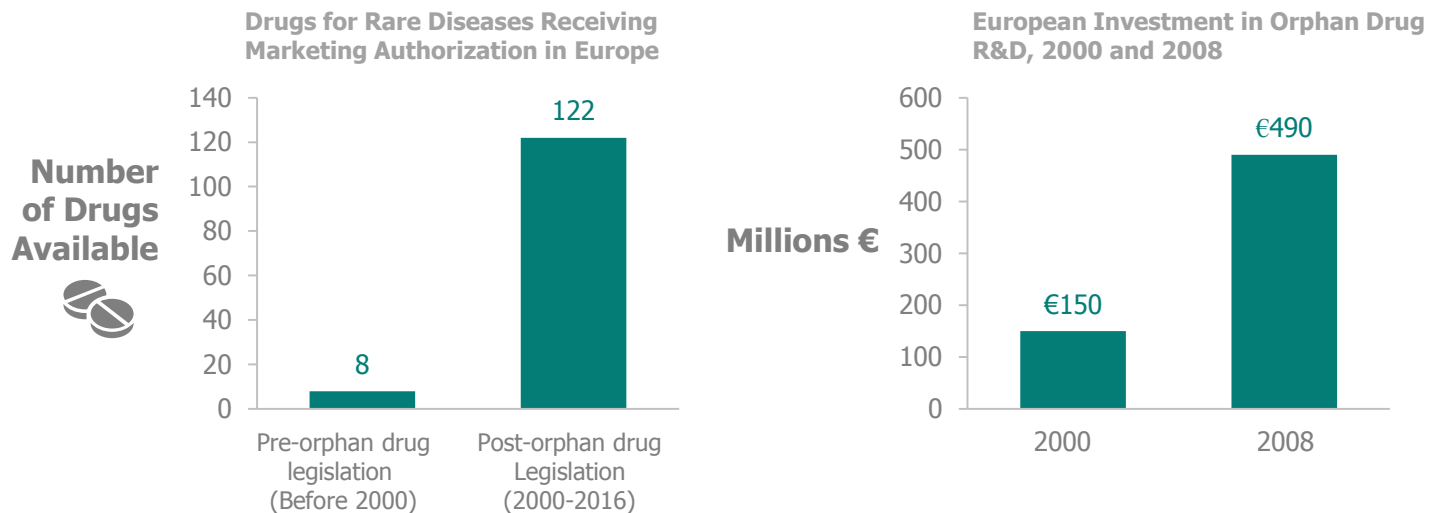


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Source 20 a: U.S. Food and Drug Administration. Office of Orphan Drug Products. Available at <https://www.fda.gov/downloads/AboutFDA/ReportsManualsForms/Reports/BudgetReports/UCM488554.pdf>. Accessed August 2017.

Source 20 b: Orphan Drug Report 2014. Available at <http://info.evaluategroup.com/rs/evaluatepharmaltd/images/2014OD.pdf>. Accessed August 2017.

Growth in European Orphan Drug Development



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 21: European Medicines Agency. Committee for Orphan Medicinal Products (COMP) meeting report on the review of applications for orphan designation. November 2016. Available at http://www.ema.europa.eu/docs/en_GB/document_library/Committee_meeting_report/2016/11/WC500216127.pdf. Accessed August 2017.

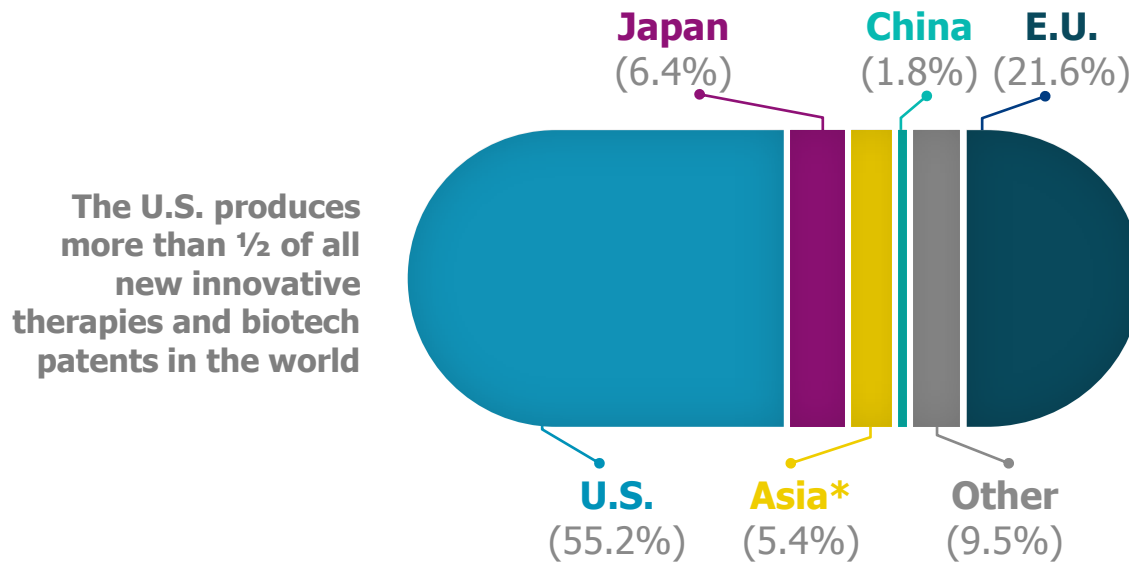
Source 21 a: Mestre-Ferrandiz J, Garau M, O'Neill P, Sussex J; OHE Consulting. Assessment of the Impact of Orphan Medicinal Products on the European Economy and Society. November, 2010. Available at <http://www.ohe.org/publications/article/assessment-of-the-impact-of-orphan-medicinal-products-oneurope-15.cfm>. Accessed August 2017.

Source 21 b: Schey C, et al. Estimating the budget impact of orphan medicines in Europe: 2010 - 2020. Orphanet J Rare Dis. 2011 Sep 27;6:62. doi: 10.1186/1750-1172-6-62. Available at <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3191371/>. Accessed August 2017.

U.S. is the Leader in Biopharmaceutical Innovation, 1998-2014



U.S. Patents Granted in Pharmaceutical Technology by Region/Country of Inventor, 2014



*Asia includes India, Malaysia, Singapore, South Korea, Taiwan and others.

Source 22: Pharmaceutical Research and Manufacturers of America <http://phrma-docs.phrma.org/files/dmfile/Biopharmaceuticals-in-Perspective-2017.pdf>. Analysis of National Science Foundation, National Science Board, data. USPTO patents granted in pharmaceuticals, by region/country/economy: selected years, 1998–2014. Available at <http://www.nsf.gov/statistics/2016/nsb20161/uploads/1/9/at06-50.pdf>. Accessed August 2017.

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

Notes and Sources



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2 a: American Cancer Society. Cancer Treatment and Survivorship Facts & Figures 2016-2017. Available at <http://www.cancer.org/acs/groups/content/@research/documents/document/acspc-048074.pdf>. Accessed August 2017.
2 b: Center for Disease Control and Prevention. Cancer Survivors. United States, 2007. CenterWatch. Available at <http://www.cdc.gov/mmwr/preview/mmwrhtml/mm6009a1.htm>. Accessed August 2017.
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3 a: McGinley, L. "The list of cancers that can be treated by immunotherapy keeps growing." The Washington Post. <https://www.washingtonpost.com/news/to-your-health/wp/2016/04/19/breakthrough-cancer-therapy-shows-growing-promise/>
3 b: Batra S. et al. Cancer metabolism as a therapeutic target. Available at <https://www.ncbi.nlm.nih.gov/pubmed/25184270>. Accessed August 2017.
3 c: National Cancer Institute. Oncolytic Virus Therapy Shows Benefit in Patients with Melanoma. Available at <https://www.cancer.gov/news-events/cancer-currents-blog/2015/oncolyticvirus-melanoma>. Accessed August 2017.
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6

CELGENE CREATING THE FUTURE THROUGH MEDICAL INNOVATION



Passion, innovation and courage have been in the company's genes since its founding. Celgene's unwavering focus on medical innovation underscores its position in a healthcare ecosystem that has delivered longer, healthier lives to patients.

From helping people obtain their medications to using cutting-edge scientific technology to discover new treatments, Celgene's entrepreneurial spirit, collaborative culture and commitment to rare diseases creates a unique platform for transforming patient outcomes. At every level, it is growing and evolving.

Celgene has become a leader in discovering, developing and delivering innovative medicine to patients with unmet needs, by digging deeper to resolve unanswered scientific questions and working tirelessly to improve the lives of people worldwide.



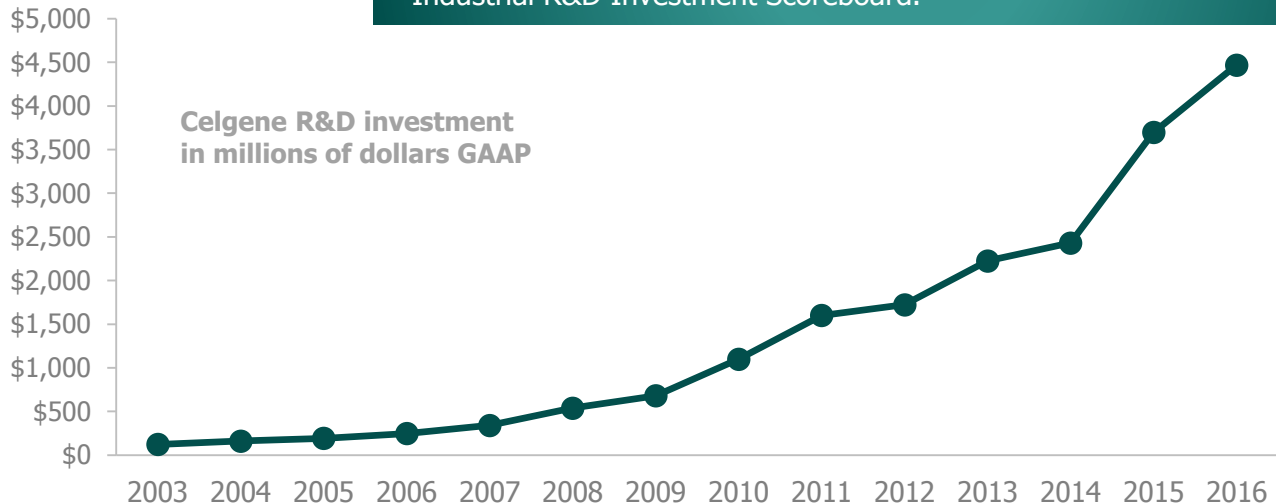


“In my many years at Celgene, I have been proud to work for a company that consistently **puts patients first** in all aspects of its business, whether it's focusing on research and development of **innovative therapies** with the **highest unmet need**, or **supporting patients** through the various stages of their disease journey.”

Chad Saward
Patient Advocacy

Delivering on Our Promise to Bring New Treatments to Patients in Need

Over the past 5 years, Celgene has invested an average of 36 percent* of total revenue on research and development. In fact, over the same period, Celgene committed **\$14.5 billion** to research and development (R&D). Celgene also ranked **#1 in R&D intensity** according to the 2016 EU Industrial R&D Investment Scoreboard.



*Based on U.S. Generally Accepted Accounting Principles.

Source: Celgene data on file.

Source: 2016 EU Industrial R&D Investment Scoreboard.



Sustaining a Deep and Diverse Group of Innovative Centers of Excellence



**Protein Homeostasis;
Biologics**
San Diego, CA



**Immuno-Oncology
Research;
Research Informatics/
Knowledge Utilization**
Seattle, WA



**Medicinal Chemistry;
Inflammation &
Immunology**
Cambridge, MA



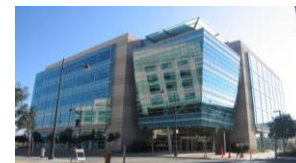
**Inflammation &
Immunology**
San Diego, CA
(Receptos)



**Nonclinical & Early
Clinical Development**
Summit, NJ



**Research Informatics/
Knowledge Utilization;
EU Clinical Operations;
Tissue Bank**
Seville, Spain (CITRE)



**Translational Development
Epigenetics;
Quantical Research**
San Francisco, CA

Source: Celgene data on file.

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

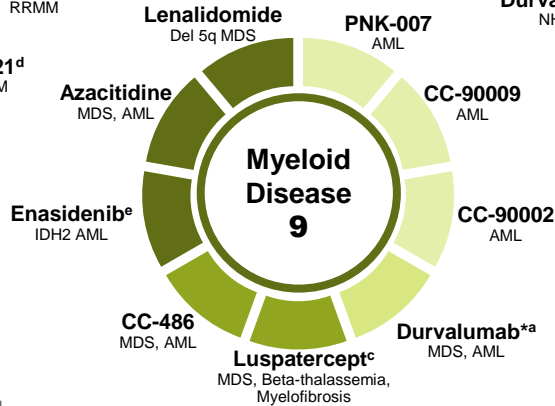
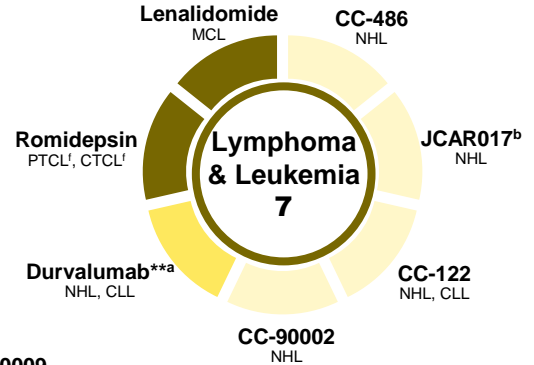
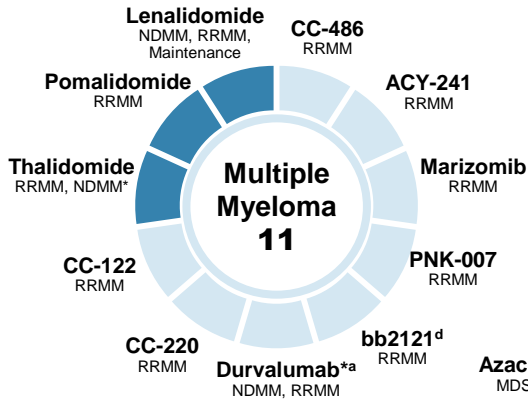
Celgene's Hematology franchise is the foundation on which our company was built. From our earliest efforts in myeloma to ongoing clinical studies in multiple diseases, our therapies are transforming the landscape of the treatment of certain blood cancers.

Our hematology portfolio includes epigenetic therapies that impact tumor cells' ability to survive and reproduce. This mechanism of action has allowed us to contribute to the great strides in diseases such as myelodysplastic syndromes and T-cell lymphomas.

Our research focuses on treating the underlying disease using a combination of actions to attack cancer cells and support the body's own defense system.

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

Celgene Continuously Enhances its Strong Hematology Pipeline



LEGEND



Ph I Post-Approval Research

*1 MM clinical study is on full hold; 3 MM clinical studies are on partial hold
 ** The NHL/CLL clinical studies are on partial clinical hold.

As of November 2017

a In collaboration with MedImmune Limited, a wholly owned subsidiary of AstraZeneca PLC.

b In collaboration with Juno Therapeutics. Celgene has commercial rights outside of North America and China.

c In collaboration with Acceleron Pharma, Inc.

d In collaboration with bluebird bio, Inc.

e In collaboration with Agios Pharmaceuticals, Inc.

f Filing for regulatory approval based on pivotal phase 2 data.

Source: Celgene data on file.

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

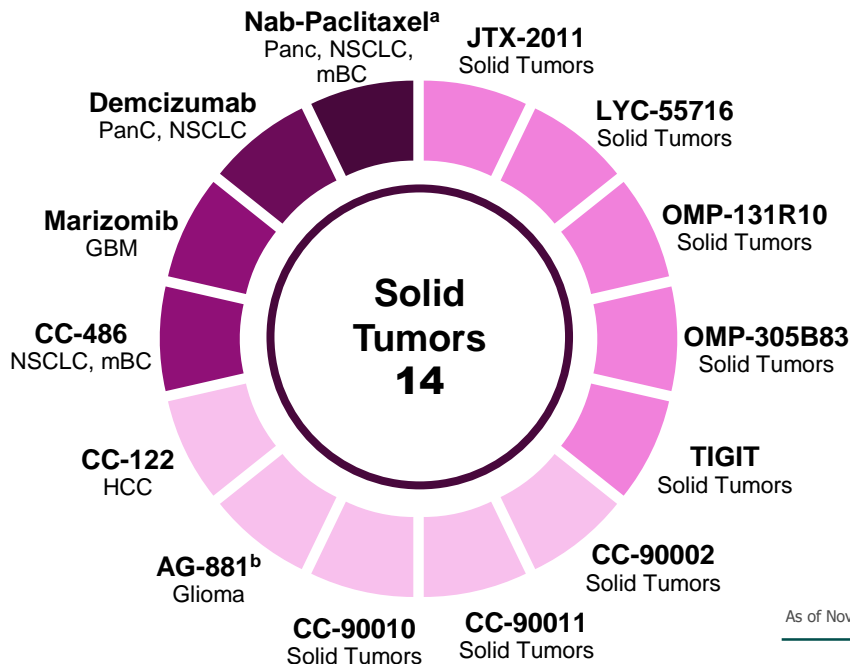
With the global growth of our company has come an expanded view of indications in which we believe we can make a significant contribution. In particular, solid tumors are a natural extension of the success we have achieved in hematology.

Our compounds are showing promise in a range of tumor types and have entered phase III trials in pancreatic cancer as an adjuvant treatment – 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.

Our Celgene Oncology franchise is moving into new disease areas with the potential to help even more patients.

Celgene's Oncology Pipeline Continues to Advance



LEGEND



As of November 2017

^a Trial conducted by licensee partner, Taiho Pharmaceuticals Co. Ltd.

^b In collaboration with Agios Pharmaceuticals, Inc.

For information on approved uses, please refer to approved product labeling. The safety and efficacy of the agents and/or uses under investigation have not been established. There is no guarantee that the agents will receive health authority approval or become commercially available in any country for the uses being investigated.

Source: Celgene data on file.



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

Alan Kay
American Computer Scientist



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