New Medicines Yield Significant Progress against Serious Diseases

The U.S. health care system faces great challenges which must be addressed. At the same time, outcomes continue to improve, with the use of new medicines playing a large role in achieving better results. This fact sheet provides examples for a few major disease areas.

In the last ten years more than 300 new medicines have been approved by the FDA. These medicines are helping patients live longer, healthier lives. They are transforming HIV and many cancers into treatable conditions, reducing the impact of chronic diseases like cardiovascular disease, diabetes, osteoporosis and rheumatoid arthritis, and fighting even the rarest conditions.

“Many examples exist of major therapeutic gains achieved by the industry in recent years... anecdotal and statistical evidence suggests that the rapid increases that have been observed in drug-related R&D spending have been accompanied by major therapeutic gains in available drug treatments.”
—Congressional Budget Office, 2006¹

“FDA approved 35 innovative drugs in FY 2011, many of them groundbreaking. This is among the highest number of approvals in the past decade, surpassed only by 2009 (37). But few years have seen as many important advances for patients.”
—Food and Drug Administration, 2011²

Increasing life expectancy
- Since 1950 life expectancy for men and women in the US has increased by a full decade, from 68.2³ to 78.7.⁴ Life expectancy is continuing to rise steadily; just since 2000 we have seen an increase of 1.9 years for males and 1.6 years for females.⁵
- In the last 75 years the death rate has fallen by 60%, according to the Centers for Disease Control and Prevention.⁶ According to the report author, “In the medical field, there have been advances and changes in behavior over time.”⁷
  - **Medicines play an important role in this progress.** For example, according to CDC, “Factors contributing to the decline in heart disease and stroke mortality include better control of risk factors, improved access to early detection, and better treatment and care, including new drugs and expanded uses for existing drugs.”⁸

Decreasing Disability
- **Disability among seniors sharply down.** A 2008 study by Harvard University researchers found that between 1984 and 2004/05 disability in the elderly population fell by 1/5.⁹ For cardiovascular disease the researchers report that medicines and other treatments increased the chances an elderly patient would survive a cardiovascular event without becoming disabled by 50%.
Cancer

- Life expectancy is increasing for cancer patients. Medicines are an important part of life expectancy gains. Since 1980 life expectancy for cancer patients has increased about 3 years and 83% of those gains are attributable to new treatments, including medicines. Another study found that medicines specifically account for 50-60% of increases in survival rates since 1975.

“From Killer to Chronic Disease: Drugs Redefine Cancer for Many”
—Washington Post headline, 2003

“There is no question that when we talk about turning the tide against cancer, the most exciting opportunities, the new opportunities in fact, are understanding the biology and applying that biology to new treatments. We are certainly at a turning point.”
—J. Leonard Lichtenfeld, M.D., MACP, Deputy Chief Medical Officer, American Cancer Society, 2012

- Cancer death rates began to fall for the first time in the 1990s and are continuing to decline. According to the National Cancer Institute’s (NCI) SEER database, since its peak in 1991, the cancer death rate has fallen by 20%. According an NCI report decreases in death rates “indicate real progress in cancer control, reflecting a combination of primary prevention, early detection, and treatment.”

The cancer death rate fell 22% for men and 14% for women between 1990 and 2007, which translated to 898,000 fewer deaths from the disease in this period, according to the American Cancer Society.

- Decline in cancer death rate is tremendously valuable. Research findings by University of Chicago economists Kevin Murphy (a MacArthur fellow) and Robert Topel show that reducing cancer death rates by 10% would be worth roughly $4.4 trillion in economic value to current and future generations.

- Five-year survival is rising. The chances that a cancer patient will live 5 years or more has increased by 36% across cancers. In 1975 the 5-year survival rate was 49%. By 2004 (the most recent data available) survival rose to 67%.
  - Survival is increasing dramatically for many forms of cancer. Between 1975 and 2004 (the most recent data available) 5-year survival went up 19% for women with breast cancer (75.5% to 89.9%), 50% for men with prostate cancer (66.4% to 99.9%), 35% for patients with colon and rectum cancer (48.7% to 65.9%), and 46% for lung and bronchus cancer (11.5% to 16.8%).

- The chance of survival for children with cancer has “improved markedly over the past 30 years, from less than 50% before the 1970s to 80% today, due to new and improved treatments,” according to the American Cancer Society.

“We have squeezed what we can out of conventional therapy." [New findings that personalized medicines can help children with cancer suggest that] "we’re beginning to have the tools to address the other 20%.”
—Michael P. Link, Stanford University School of Medicine, President of ASCO, 2012

- New cancer medicines significantly advanced treatment in 2011. The American Society of Clinical Oncology identified 12 major cancer treatment advances in 2011 that had the potential to reduce cancer mortality. Of these, 10 are related to new medicines, better ways to use existing medicines, or newly discovered benefits of approved medicines.

“Scientifically, we have never been in a better position to advance cancer treatment. ... We now understand many of the cellular pathways that can lead to cancer. We have learned how to develop drugs that block these pathways. And increasingly, we know how to personalize therapy to the unique genetics of the tumor, and the patient.”
—Richard L. Schilsky, MD, Professor, University of Chicago, and former President, ASCO, 2008
Cardiovascular Disease

- **Overall death rates from cardiovascular disease are falling.** According to a 2011 statistics update by the American Heart Association (AHA), death rates for cardiovascular disease fell 30.6% between 1998 and 2008.25
  - According to the lead researcher of another AHA report there would have been an additional 190,000 deaths in 2006 if death rates had remained at 1999 levels.26
- **Death rates for diseases of the heart are falling significantly.** Between 2000 and 2008 (the most recent data), death rates fell by 28% according to the Centers for Disease Control and Prevention.27
  - The death rate for one common type of disease of the heart, coronary heart disease, fell by 40% between 1980 and 2000 according to a 2007 study in the *New England Journal of Medicine*.28 This drop resulted in 341,745 fewer deaths in 2000. The researchers attribute approximately half of this decrease to new medical therapies, including medicines.
  - Death rates for heart failure and heart attack (two outcomes that can result from coronary heart disease) fell by nearly half 1999-2005. A 2007 study published in the *Journal of the American Medical Association* reports that the death rate for heart attack patients in hospitals decreased from 8.4% in 1999 to 4.6% in 2005. The study also showed that congestive heart failure – often a debilitating and costly condition – developed in 11% of heart attack patients in 2005, down from 19.5% just six years earlier. The researchers point to the increased use of cholesterol drugs, blood thinners, and angioplasties as a direct cause of lowered rates of death and heart failure.29
- **According to the Centers for Disease Control and Prevention, new medicines contribute to improving trends in cardiovascular disease:** “Factors contributing to the decline in heart disease and stroke mortality include better control of risk factors, improved access to early detection, and better treatment and care, including new drugs and expanded uses for existing drugs.”30
- **Blood pressure medicines save lives and prevent hospitalizations, but even more could be saved if all patients received recommended care.** A 2007 study in *Health Affairs* found that use of antihypertensive medicines prevented 86,000 premature deaths from cardiovascular disease in 2001 and, 833,000 hospitalizations for heart attack and stroke in 2002.31 If all untreated patients received recommended care an additional 89,000 deaths and 420,000 hospitalizations could be prevented each year.
- **Despite rising obesity levels, Americans have reached a milestone in controlling high cholesterol.** The Centers for Disease Control and Prevention reported at the end of 2007 that U.S. adults reached an average cholesterol level in the ideal range (below 200) for the first time in 50 years.32 Authors of the report attribute the drop to the increased use of cholesterol-lowering medicine in the over-60 population, but warn that increasing obesity rates could wipe out the gains.33

“**Protein enzymes, receptors, or channels identified by the pharmaceutical industry as “drugable targets” have led to striking, remarkable, and repeated achievement.”**
—Drs. Myron Weisfeldt and Susan Zieman, Johns Hopkins University, “Advances in the Prevention and Treatment of Cardiovascular Disease,” *Health Affairs, 2007*34

HIV/AIDS

- **Life with HIV/AIDS has changed dramatically.** Patients diagnosed with AIDS in 1990 could expect to live only months,35 during which time they would be likely to contract a number of opportunistic infections. The only treatment available had to be taken every four hours—around the clock—and had serious side effects. Since
the approval of the anti-retroviral treatments (ART) in 1995 and more classes in subsequent years the AIDS death rate has dropped by 79%. If diagnosed today, a range of treatment options, including different combinations of drugs, often keep patients symptom-free for years.

“One of the most impressive success stories in the translation of basic biomedical research into interventions that positively affect the lives of millions of people has been the delineation of vulnerable targets in the replication cycle of HIV. This achievement has led to the development of highly effective therapies for HIV-infected persons. To understand the enormity of the effect of ART on the lives of HIV-infected persons, it is important to consider that in the early years of the AIDS epidemic before ART was available, the median survival after an AIDS diagnosis was measured in weeks to months and patient care was confined to diagnosing and treating a complex array of opportunistic infections and AIDS-related types of cancer.

“Beginning with the use of zidovudine monotherapy in 1987, 5 classes of antiretroviral drugs have been developed. Combinations of these agents safely and reliably suppress HIV replication in the body below the limits of detection in most HIV-infected persons receiving this therapy. In stark contrast to the early and mid-1980s, if a person aged 20 years is newly infected with HIV today and guideline recommended therapy is initiated, researchers can predict by using mathematical modeling that this person will live at least an additional 50 years—that is, a close-to-normal life expectancy.”

—Carl W. Dieffenback, PhD and Anthony D. Fauci, MD, National Institute of Allergy and Infectious Disease, Annals of Internal Medicine, 2011

- New treatments are keeping ahead of the constantly changing HIV virus. Researchers are always in a race against time with HIV because the virus adapts to existing treatments making them less effective. In recent years new HIV treatments have continued to be approved, including a new personalized HIV treatment which represents a new class of medicines. New treatments such as this one represent important options for patients whose infection is not responding to available medicines.

- Thanks in part to continued treatment advances, HIV death rates are continuing to fall. The most dramatic drops in HIV death rates occurred following the introduction of ART with decreases averaging 33% per year between 1995 and 1998. From 1999 to 2009, death rates continued to drop by 5% per year. The most recent data has shown continued decline in death rates: between 2009 and 2010, death rates fell another 13%.

- Hospitalizations have dropped. Since ART became available the number of people with HIV increased by 28% between 1996 and 2000 primarily because of rising survival rates. Hospital rates, however, fell by 32% over the same period.

- In recent years hospitalization rates have continued to fall. Between 2002 and 2007 the hospitalization rate fell from 35 per 100 HIV patients to 27 per 100 patients, a 23% drop, according to researchers at Johns Hopkins University School of Medicine. Before effective treatments existed HIV/AIDS was very expensive, with repeated hospitalizations a major cost driver. In 1985, it cost the U.S. Army an estimated $500,000 to treat each AIDS patient in its care, and experts warned that the disease had “the potential to bankrupt the system.”

- Earlier use of medicines recommended. Based on growing evidence that unchecked HIV virus is a greater risk than adverse reactions to the medicine, new guidelines issued by the International AIDS Society are recommending earlier initiation of ART even in many cases when the patient is asymptomatic. These guidelines recommend treatment when the CD4 cell count drops below 500/microlitre and should even be considered before blood count drops to the level. Previous guidelines had recommended treatment when the CD4 count fell to 350/microlitre.

- Use of HIV medicines helps prevent transmission. A recent study reported in The Lancet and carried out in Africa found that initiation of ART reduces the risk of transmission from an infected individual to their sexual partner by 92%. A large new study sponsored by the National Institute of Allergy and Infectious Disease echoes this result, finding that early initiation of ART reduced transmission by 96%. The international study was stopped and unblinded four years early because the findings were so robust.
• **Medicines have become easier to use – adding to their effectiveness.** The first once-daily one-pill combination tablet was approved for treatment of HIV in 2006.\textsuperscript{48} This medicine combines the active ingredients of three widely used antiretroviral drugs into a single dose. The single-pill treatment regimen is a marked improvement over previous HIV/AIDS treatments, which often require patients to take multiple pills every day. Missing doses can lead the virus to mutate and become resistant to medicines. Because this new pill will make it easier for patients to remember to take their medicine it is believed that “widespread use of the combination drug could potentially even slow the spread and evolution of the AIDS epidemic itself.”\textsuperscript{49}

“...The development of [antiretroviral therapy (ART)] has been one of the greatest accomplishments of basic and translational research: approximately 30 anti-HIV agents are licensed and evidence-based guidelines have been developed for their optimal use. Combination ART with at least 3 drugs has resulted in substantial reductions in morbidity and mortality in both rich and poor countries. Antiretroviral therapy has been simplified to the point where treatment with a single, multidrug pill once a day is possible with generally manageable adverse effects. With improvements in ART, the estimated life expectancy of certain HIV-infected patients now approaches that of uninfected individuals. Antiretroviral therapy also has proven efficacious in HIV prevention, reducing the risk of mother-to-child transmission and serving as postexposure prophylaxis for individuals exposed to HIV.”

—Gregory K. Folksers, MS, MPH; Anthony S. Fauci, MD, National Institute of Allergy and Infectious Diseases, 2010\textsuperscript{50}

“Antiretroviral therapy of HIV infection has changed a uniformly fatal into a potentially chronic disease.... Patients who can access and adhere to combination therapy should be able to achieve durable, potential lifelong suppression of HIV replication.”

—Paul Volberding, MD and Steven Deeks, MD, University of San Francisco, *The Lancet*, 2010\textsuperscript{51}

“...There is a stunning contrast between how I felt as a physician-scientist in the 1980s and the optimism I feel today as more infections are prevented and lifesaving drugs increasingly become available throughout the world.”

—Anthony S. Fauci, MD, Director, National Institute of Allergy and Infectious Diseases, 2011\textsuperscript{52}

“We’re at some sort of turning point in the AIDS epidemic... It’s not a single thing going on. It’s the culmination of what’s happened for 30 years. ... Each of them is moving the political world to start thinking about an AIDS-free generation.”

—Myron Cohen, MD, University of North Carolina-Chapel Hill, 2012\textsuperscript{53}

**Diabetes**

- **Death rates for people with diabetes fell substantially between 1997 and 2006** according to a report by CDC and NIH researchers.\textsuperscript{54} All-cause mortality fell 23% and deaths due to heart disease and stroke fell 40%.

- **Many new treatments for diabetes.** In recent years **8 new classes** of diabetes medicines have been approved, giving patients and providers powerful new tools to treat the condition.

- **Taking diabetes medicines as directed reduces the risk of hospitalizations.**
  - Improved use of diabetes medicines cuts risk of hospitalization by half. Patients who are less than 80% adherent to their diabetes medicines are 2-3 times more likely to be hospitalized in the next year compared with patients who are more adherent. The less often a patient took their medication as directed, the more likely the patient was to be hospitalized.\textsuperscript{55}
  - Adherent diabetes patients are less likely to use hospital and ER care. A recent study found that emergency room visits among patients who took their diabetes medicines as directed was 46% lower than for patients who took their medicines less than 50% of the time. Similarly, the hospitalization rate and the number of days spent in the hospital were 23% and 24% lower, respectively, for adherent patients.\textsuperscript{56}
• Diabetes patients treated with medicines are less likely to develop other health problems. Diabetes patients who are treated with diabetes medicines are 31% less likely to develop lipid disorders and 13% less likely to develop high blood pressure than untreated patients.57

Rheumatoid Arthritis

• Clinical remission is now possible for patients with severe rheumatoid arthritis (RA).58 A recent study found that patients treated with combination therapy consisting of both a new and older medicine had a 50% chance of complete clinical remission after 52 weeks of treatment, compared with 28% taking only the older medicine. These results would have been “unthinkable” prior to new disease-modifying biological medicines.59
• In 2008 the American College of Rheumatology (ACR) rewrote rheumatoid arthritis progress measures to account for biopharmaceutical advances in treating RA.60 The magnitude of symptom improvement for patients on biologic medicines largely surpassed the 20% improvement threshold – using a measure known as ACR20 to assess improvements in tender and swollen joint count and other indicators. In 2007, the ACR proposed a revision to the ACR20 standard that would create a hybrid outcome measure that includes 50% and 70% improvements (ACR50 and ACR70).61
• Treatment of rheumatoid arthritis has changed significantly in recent years:

  “Only 20 years ago, rheumatoid arthritis (RA) was regarded as a relentlessly progressive disease. Treatments provided little hope of significantly modifying long-term disease outcome. The literature painted “a grim picture” suggesting “that both premature death and marked functional morbidity occur even in population-based analyses” and that “the long-term prognosis of rheumatoid arthritis is bad”....

  “During the ensuing 20 years, we have witnessed significant advances. In clinical trial design and daily practice, widespread use is made of a variety of disease activity measures and response criteria that have been developed and validated.... In parallel, unique advances were made in the therapeutic arena: recognition of the importance of early treatment, emergence of MTX [methotrexate] as a recognized DMARD [disease-modifying anti-rheumatic drug] with far greater effectiveness and safety than previously available DMARDs; and new biologic agents successfully expanded the results of treatment of RA.”
  —Drs. Joseph Smolen and Daniel Aletaha, Clinical and Experimental Rheumatology, 200662

  “Today a new class of anti-rheumatic drugs dramatically slows disease progression.”
  —Dr. Elias Zerhouni, Former Director, National Institutes of Health, 200863

Hepatitis C

• Sustained virologic response rates fell improved from 10% in the 1990s to 80% today among hepatitis C patients.64 Sustained virologic response, defined as the suppression of the virus below detectable levels for 24 weeks after treatment, rose as understanding of the disease grew and treatment moved to today’s triple therapy regimens which include recently direct acting antivirals.

Rare Diseases

• New treatment options for rare diseases give new hope to patients with few existing treatment options.
  Since the passage of the Orphan Drug Act in 1983 more than 400 medicines have been approved to treat rare diseases.65 This includes 11 orphan approvals in 2011. Examples include:
  o The first treatment for ALS or Lou Gehrig’s disease.
o Five new treatments for pulmonary hypertension.
   o A genetically engineered antibody that is the first treatment for Crohn’s disease.
   o The first medicine that treats the cause of Fabry disease, rather than its symptoms.
   o The first in a new class of medicines to treat acromegaly, a disorder in which excess growth hormone causes enlarged hands feet and facial features.
   o The first self-administered treatment for life threatening swelling in patients with the genetic disorder hereditary angioedema.
   o A personalized medicine, which is the first medicine to specifically treat a subset of patients with non-small-cell lung cancer.

**Recent Approvals**

- **New medicines offer promise to continue and accelerate the progress we have seen in recent years.**
  Between 2001 and 2011 the FDA approved 340 new medicines. In the last year we have seen many valuable new medicines approved:
    o **Cancer:** Two new personalized medicines for lung cancer and melanoma now provide effective options for patients with tumors expressing certain genetic markers.66 The personalized melanoma treatment and another new melanoma medicine became the first new approvals for the disease in 13 years.
    o **Rare Diseases and Orphan Indications:** Eleven new medicines were made available to patients for rare diseases such as the genetic defect congenital factor XIII deficiency, several cancers, and scorpion poisoning.57
    o **Lupus:** The first new medicine for lupus since 1955 takes a new approach to treating this serious and potentially fatal autoimmune disease.68
    o **Hepatitis C:** Two new medicines approved last year are the first in a new class and offer a greater chance of cure for some patients, compared with existing therapies.69
    o **Cystic Fibrosis:** The first drug available that targets the underlying cause of cystic fibrosis. This personalized medicine treats a subset of patients with CF with the mutation known as G551D.70

---

New Medicines Yield Significant Progress against Serious Diseases
New Medicines Yield Significant Progress against Serious Diseases


57. PharMetrics, Examination of Treatment Patterns and Effects of Medication-Taking Behaviors Among Patients with Diabetes, (Watertown, MA: PharMetrics, 2004) (research supported by PhRMA).


70. Cystic Fibrosis Foundation, Kalydeco, 8 February 2012, http://www.cff.org/treatments/Therapies/Kalydeco/