

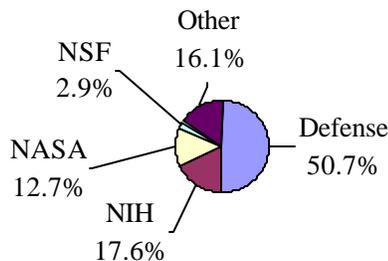
**GOAL 6: STRENGTHEN THE NATION'S HEALTH SCIENCES RESEARCH ENTERPRISE and ENHANCE ITS PRODUCTIVITY.**

*Improvements in health are grounded in knowledge acquired through research.* HHS sets the pace for the world in medical, epidemiological (incidence, distribution, and control of disease), behavioral, and health services research. We sponsor and conduct public and private research through strong, sustained public support for health sciences.

Today we are at the brink of discoveries that have the potential to revolutionize the prevention, diagnosis, and treatment of disease as well as the delivery of quality health care in America and around the world. As a Department, HHS has recognized the potential for health research advances and continues to pursue a focused and balanced approach to funding research and the infrastructure necessary to take advantage of research opportunities.

This year NIH established a formal working group of the Advisory Committee of the Director that conducted an independent assessment of NIH performance under this goal.

**Federal FY 1999 Research Outlays**



Source: President's Budget for Fiscal Year 2001, Historical Table 9.8

*NIH accounted for 17.6% of the entire Federal research budget in FY 1999.*



*HHS recognizes the importance and benefit of basic research.*

**❖ We improved the understanding of normal and abnormal biological processes and behaviors.**

This year again brought news of significant gains in biomedical research. Research findings about normal and abnormal biological functions constitute an essential knowledge base to support advances in prevention and treatment science and to determine what efforts are possible and effective across the population.

*Human cancer cells were created in the laboratory* by altering the expression of a defined set of genes and affecting at least four cellular pathways. The ability to introduce specific genetic alterations to transform normal cells paves the way for more precisely defining the biochemical pathways in the cell that must be disrupted in the development of cancer. This information will open new avenues for exploring the roles of various cellular pathways that become disrupted and for determining the sequence of events that must occur as cancer develops.

Studies in rodents resulted in the first evidence that adult neural stem cells can be used to *repair damage from a broad array of brain accidents/diseases where cell dysfunction is "global"* or spread throughout the brain. Other researchers demonstrated that bone marrow stem cells could give rise to liver cells and that neural stem cells become blood-forming cells. This new knowledge changes the way we think about the brain and treatment for brain disease and

injury and has obvious implications for the development of new treatment modalities for a number of devastating illnesses and injuries.

A family of *proteins (toll-like receptors) that are involved in the body's immune response* to bacteria was discovered. When these proteins detect and signal the presence of the bacteria, they trigger a severe immune reaction that can lead to septic shock. This new knowledge could facilitate development of new vaccine strategies and new approaches to the treatment of septic shock. Drugs that could interfere with the activation of toll-like receptors by bacteria during an acute infection could save thousands of lives.

The working group determined that this target was exceeded after a review of nearly 300 descriptions of research outcomes published in FY 1999.



The *human genome project* seeks to understand the genetic instructions that make us unique. Critical genomic resources continued to be developed by achieving a FY 1999 U.S. annual production rate of human genomic sequence of 173 million base-pairs, a world-wide rate of 265 million base-pairs, a total of 442 million completed world-wide and completing the sequence of the 97 million base-pairs of the *C. elegans* genome.

The *C. elegans* is a roundworm and its genetic sequencing marked a historic accomplishment since it provides biologists with a powerful tool to experiment with and learn how whole genomes function. The ability to compare the sequence of genes across multiple species and develop model systems in simpler organisms will significantly enhance the ability of researchers to identify the functional roles of the encoded proteins and thereby contribute to a better understanding of the molecular basis for human health and disease.

All of the publicly funded U.S. centers sequencing the human gene are meeting and in many cases, exceeding the standards of quality assurance for their data. The working group determined that these targets were exceeded based upon public databases.



❖ **We improved the prevention, diagnosis, and treatment of disease and disability.**

*Heart disease* is the nation's number one killer among men and women of all racial and ethnic groups. More than 40 percent of all deaths in

**Goal (NIH):** Add to the body of knowledge about normal and abnormal biological functions and behavior.

**FY 1999 Target:** Progress in advancing scientific understanding in key fields bearing on our knowledge of biological functions and behavior in their normal and abnormal state.

**FY 1999 Actual:** Exceeded the target.



**Goal (NIH):** Develop critical genomic resources, including the DNA sequences of the human genome, and the genomes of important model organisms and disease-causing microorganisms.

**FY 1999 Targets:**

- 1) U.S. annual production rate of human genomic sequence: 90 million base-pairs.
- 2) Worldwide rate: 220 million base-pairs.
- 3) Total completed worldwide at the end of FY 1999: 400 million base-pairs.
- 4) Complete the sequence of the *C. elegans* genome.

**FY 1999 Actual:** Exceeded the targets for 1) - 3) and met the target for 4).



**Goal (CDC):** Reduce morbidity and mortality attributable to behavioral risk factors by building nationwide programs in chronic disease prevention and health promotion and intervening in selected diseases and risk factors.

**1999 Target:** 85 % of states participating in the Behavioral Risk Factor Surveillance System communicate the findings of their annual behavioral risk factor data collected.

**1999 Actual:** Data is not available yet.

the United States, 900,000 each year, are directly attributable to heart disease and stroke. Associated annual costs exceed \$286 billion.

In FY 1999 CDC expanded the first state-based program for developing policies and conducting research to prevent cardiovascular disease—the leading cause of death of men and women across all racial and ethnic groups—from 8 to 11 states and strengthened the capacity of the initial 8 programs to address racial and ethnic disparities in cardiovascular disease.

In September, the National Heart, Lung, and Blood Institute and the National Institute of Diabetes and Digestive and Kidney Diseases of NIH issued an alert on the increasing importance of *diabetes mellitus* as a major risk factor for cardiovascular disease.



In addition, NIH's research program resulted in the following accomplishments in FY 1999:

- Development of a promising new technique for detecting lung cancer at an earlier and potentially more curable stage.
- Development of an improved approach for preventing mother-to-child transmission of HIV.
- Development of a new test for diagnosing a particularly devastating aggressive cancer that can involve the brain, spinal cord, and the eye.
- Identification of an effective non-surgical treatment for fistulas, a serious complication associated with the chronic inflammatory bowel disease known as Crohn's disease.
- Progress towards the Administration's goal of developing an AIDS vaccine by 2007 by increasing the number and dollar value of awards made for vaccine discovery.
- Development and implementation of the Clinical Trials database, a consolidated source of information related to federally and privately funded clinical trials for drugs used for serious or life threatening diseases and conditions.

Progress toward the various performance goals of new methods, technologies or approaches for diagnosing, preventing, or treating disease and the NIH working group also assessed disability. All FY 1999 targets were met, successfully met, or substantially exceeded. Suggestions were also provided for improving one of the goal areas.



❖ **We actively supported the research capacity of the country.**

Through its *Research Training and Career Development Program*, NIH supports a critical aspect of scientific research:

- The development of a talent base capable of producing advances in science.

To evaluate its success in attracting, developing, and retaining a diverse group of scientists, NIH has established several performance goals to assess the agency's success in attracting qualified applicants. For example, in FY 1999 NIH met its goal to maintain an application flow consistent with success rates close to historical levels of 40 percent for fellowships and 60 percent for research training grants and entry-level career awards.

NIH increased the pool of clinical researchers who can conduct patient-oriented research by issuing 85 mentored Patient-Oriented Research Career Development awards, 83 mid-career Investigator awards in Patient-Oriented research, and 35 curriculum development awards.

NIH also encouraged interest in scientific research careers by making information on training and career development opportunities widely available to students and post-doctorates (e.g., Independent Scientist Award, Minority and Disability Research supplements, Mentored Clinical Scientist Development Award).

AHCPR also supported 69 pre- and 86 post-doctoral National Research Service Award trainees to ensure that investigators will exist to perform the research necessary to improve quality and cost effective health care.



NIH supports *construction of facilities* on the NIH campus, as well as grants to fund facility improvements at institutions outside of NIH.

- Completed 56.4 percent of Louis Stokes Laboratory Building although the FY 1999 target was 65 percent completion. The NIH assessment revealed that this shortfall could be attributed to the need to make space adjustments to support current and projected research requirements. Construction is expected to be complete in December 2000, rather than the end of FY 2000.
- Completed design and over 66 percent of the construction for the Dale and Betty Bumpers Vaccine Research Center.
- Made major progress in the design and site work for the Mark O. Hatfield Clinical Research Center.



*Research capacity must keep pace  
with research priorities and  
technological advances.*