Government Performance and Results Act

FY 2004 Final Annual Performance Plan
FY 2003 Revised Final Performance Plan
FY 2002 Annual Performance Report

U.S. Department of Health and Human Services

February 3, 2003

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From the Director

In accordance with the Government Performance and Results Act of 1993, I am pleased to present the National Institutes of Health's Performance Plans for Fiscal Years 2004 and 2003 and Performance Report for Fiscal Year 2002. In keeping with HHS and OMB guidance, the GPRA plan and report are consolidated and submitted as part of the Congressional Justification.

Begun as a one-room Laboratory of Hygiene in 1887, the National Institutes of Health (NIH) today is one of the world's foremost medical research centers. An agency of the Department of Health and Human Services, the NIH is the Federal focal point for health research and the steward of medical and behavioral research for the Nation.

The NIH mission is to uncover new knowledge that will lead to better health for everyone. NIH works toward that mission by supporting the research of non-federal scientists in universities, medical schools, hospitals, and research institutions throughout the country and abroad; conducting research in its own laboratories; helping in the training of research investigators; supporting the development of research resources and facilities; and fostering communication of medical information.

The record of payoffs from the national investment in health research is profound. For example, 30 years after the signing of the National Cancer Act, we are able to cure more than half of all cancers; specifically, nearly half of all high-grade lymphomas are cured, the overwhelming majority of all testicular cancers are cured, and we're curing more breast and ovarian cancer now than ever before. Similarly, coronary heart disease mortality is half of what it was several decades ago, and death rates from stroke have declined 60 percent. Furthermore, the development of anti-HIV drugs (highly active antiretroviral therapy or "HAART") have dramatically reduced the numbers of new AIDS cases and AIDS deaths, allowing patients to live productive lives for many years after contracting HIV.

Today, the confluence of generous budgets and extraordinary scientific opportunity poise NIH to make contributions to improvements in health that throw past accomplishments into the shadows. The draft of the DNA sequence of the human genome is the best known of the new tools that are revolutionizing biomedical research and generating hope where none existed before. But there are several additional new lines of research that are changing the way biomedical research is done. These include proteomics – the analysis of large sets of proteins with the goal of understanding their function; combinatorial chemistry – a new way to generate large libraries of molecules that can be screened for use as drugs; and new, advanced imaging techniques that enable scientists to see within the body as it carries out various functions. As a result, progress in the biomedical sciences is moving at a speed that could only be dreamed of just five years ago.

I am proud to report to you and the Nation on the results of fiscal year 2002 NIH endeavors related to GPRA goals and, in the spirit of transparency and accountability, to set forth Performance Plans for fiscal years 2003 and 2004.

Dr. Elias A. Zerhouni
Director, National Institutes of Health

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National Institutes of Health

Government Performance and Results Act

Final FY 2004 Annual Performance Plan; Revised Final FY 2003 Annual Performance Plan;
FY 2002 Annual Performance Report

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Executive Summary

Agency Mission

Founded in 1887, the National Institutes of Health (NIH) is the Federal focal point for medical research in the United States. Composed of 27 separate Institutes and Centers, NIH is one of eight health agencies of the Public Health Service, a major component of the U.S. Department of Health and Human Services (DHHS).

The NIH mission is to uncover new knowledge that will lead to better health for everyone.

NIH funds research on diseases and conditions ranging from the rarest genetic disorder to the common cold. NIH supports research of non-federal scientists in universities, medical centers, hospitals, and research institutions throughout the country and abroad; conducts research in its own laboratories; helps to train research investigators; and fosters communication of medical information to the public, health care providers, and the scientific community.

Medical innovation is one of the principal foundations on which America's past successes in improving healthcare have been built. It is where hope for the future resides. History provides abundant evidence that medical progress rarely occurs without the sustained pursuit of advances in basic and behavioral science. Through the conduct and support of medical research, NIH seeks to expand fundamental knowledge about the nature and behavior of living systems; to improve and develop new strategies for the diagnosis, treatment, and prevention of disease; and to reduce the burdens of disease and disability.

About This Document

The Government Performance and Results Act. In 1993, Congress passed and the President signed into law the Government Performance and Results Act (GPRA), P.L. 103-62. This legislation's broad intent is to enhance the effectiveness, efficiency, and accountability of government programs by directing Federal agencies to more sharply focus their management efforts on the results that program spending yields.

Linkage to HHS and Other Strategic Plans. For the strategic planning component of GPRA, an agency is defined as a cabinet-level department or independent agency. Thus, NIH is not considered an agency for GPRA purposes. Rather, its parent organization, HHS, is the unit required to submit the governing strategic plan.

The ways in which the NIH Performance Plan links to the HHS Strategic Plan and other planning documents are indicated in Part II of the Plan in the Summaries of Performance for each Program. In each summary, the far right column is used for reference. Reference codes indicate linkage to various HHS Strategic Plan goals and linkage with other important priority setting

activities, including the Department's 10-year health objectives for the Nation known as "Healthy People 2010" and the President's Management Agenda.

Finally, the relationship between the NIH GPRA Performance Plan and the plans of other HHS agencies is noted. Where NIH goals link to goals of other HHS Operating Divisions, a code has been placed in the summary table reference column and the link is discussed in Appendix 1.

Budget and Performance Integration. This document, NIH's Final FY 2004 Annual Performance Plan, Revised Final FY 2003 Annual Performance Plan, and FY 2002 Annual Performance Report under GPRA, is provided as Volume II of the FY 2004 NIH Congressional Justification. Budget information (a crosswalk between GPRA programs and the NIH budget mechanisms table) is provided at the end of Part I of this document (see page 13).

Point of Contact. The Office of Science Policy and Planning, Office of Science Policy, Office of the Director, National Institutes of Health (NIH) prepared this document. If you have questions or comments, please contact Robin Kawazoe, Director, Office of Science Policy and Planning on (301) 496-1454.

An electronic version of this document will be available in March 2003 from NIH's World Wide Web server at the following address: http://www1.od.nih.gov/osp/ospp/gpra/gpra_nih.htm. Previous performance plans and reports are available now.

Overview of NIH Plan and Performance Report

NIH invests the public's resources and support for medical science in three basic and interrelated ways. First and foremost, NIH conducts and supports medical research. Second, it contributes to the development and training of the pool of scientific talent. Third, it participates in the support, construction, and maintenance of the laboratory facilities necessary for conducting cutting-edge research.

NIH has organized its performance goals under the following three core GPRA Programs, corresponding to the three means NIH uses to achieve its mission:

- Research Program
- Research Training and Career Development Program
- Research Facilities Program

In the Revised Final FY 2003 Annual Performance Plan, NIH has 58 goals and 91 targets; in the Final FY 2004 Annual Performance Plan, NIH has 51 goals and 77 targets.

Research Program

The 42 FY 2004 performance goals of the *Research Program* focus on enhancing research outcomes across the medical research continuum by supporting research in NIH's own intramural laboratories as well as the research of non-federal scientists working in universities, medical schools, hospitals, and research institutions throughout the country. Through its Research Program, NIH also focuses on communicating scientific results, promoting the efficient transfer of new drugs and other technologies, and providing effective research leadership and administration. NIH organizes the performance goals for this Program under five functional areas:
1) Research Outcomes,
2) Communication of Results,
3) Technology Transfer,
4) Grants Administration and Peer Review, and
5) Agency Management and Administrative Support.

Research Outcomes. This goal area addresses the heart of the NIH mission, its research outcomes. In FY 2002 NIH had seven goals in this area:

FY 2002 Performance Highlights

Discovering controls of gene expression and other cellular activities

- NIH-funded research has shown that molecules called small RNAs-cousins of DNA-play an active role in turning genes on and off, including during development.
- Implications to be examined in further study of small RNAs include possible roles in development, disease, and protection of the genome against instability. The basic science holds promise as well for developing new treatments to battle diseases.

Science declared this advance the top scientific breakthrough of the year.

Battling anthrax toxins

- NIH-supported researchers revealed that an anthrax toxin called lethal factor (LF) allows anthrax to evade the immune system by inhibiting immune cells called macrophages that normally would signal the immune system to attack the anthrax bacteria.
- Researchers also determined that the structure of another anthrax toxin, called edema factor, contains a deep, narrow pocket when activated, making it an ideal drug target.

Developing a vaccine for West Nile Virus and other flaviviruses

- NIH-supported researchers benefited from earlier basic research on flaviviruses, the group of viruses to which West Nile belongs. In 1999, NIH funded a fast-track project to develop a candidate vaccine with a private company based on existing vaccine for another similar virus.
- Vaccine now is being produced and an investigational new drug (IND) application will be filed with the Food and Drug Administration. Phase I trials are expected to begin in early 2003.

Improving Alzheimer's disease diagnosis and treatment with imaging techniques

- Investigators identified specific metabolic changes in the brain that are characteristic of Alzheimer's disease. One study demonstrated that measuring patterns of these brain changes can be highly effective for an accurate diagnosis of Alzheimer's disease.
- Researchers also found that the atrophy of several areas of the brain, as measured by MRI, correlates with decline in brain activity and cognitive ability.

a) Add to the body of knowledge about normal and abnormal biological functions and behavior.

b) Develop new or improved instruments and technologies for use in research and medicine.

c) Develop new or improved approaches for preventing or delaying the onset or progression of disease and disability.

d) Develop new or improved methods for diagnosing disease and disability.

e) Develop new or improved methods for treating disease and disability.

f) Develop critical genomic resources, including the DNA sequences of the human genome and the genomes of important model organisms and disease-causing microorganisms.

g) Develop an HIV/AIDS vaccine by 2007.

These goals will be superseded in FY 2003. Under NIH's new approach to research outcome goals, there will be a greater number of goals, but the goals will be more specific. See page 91 for a list of the NIH GPRA research outcome goals for FY 2003 and beyond.

Minimizing brain damage after stroke

- The blood-brain barrier protects the brain from toxic substances, but also prevents most potentially therapeutic drugs from entering.
- NIH-supported scientists developed a "Trojan horse" strategy to bypass the protective blood-brain barrier and introduce a drug directly into the brain. In animal studies, this delivery helped reduce stroke-induced brain damage by 70 percent.

Turning off a cancer gene leads to tumor regression without toxicity

- Scientists showed in animal studies that brief inactivation of a particular oncogene (a cancer-related gene) during cancer treatment may lead to sustained regression of tumors without inducing significant toxicity.
- This study provides a promising strategy for fighting cancer—drugs that intermittently and briefly inactivate oncogenes may be effective, nontoxic cancer therapeutics.

Regenerating neurons with adult neural stem cells

- NIH-supported researchers demonstrated in animal studies that neural stem cells derived from adult brains can make functional neurons and integrate into the circuitry of some brain regions.
- These stem-cell-derived neurons made functional connections, called synapses, with normal neurons and with each other, and released neurotransmitters, the chemical mediators of neuronal communication.
- Clinical implications include the potential to regenerate damaged brain tissue and to replace dying neurons.

Treating hypertension with traditional diuretics

- Results from the largest clinical trial ever conducted on reducing high blood pressure revealed that traditional diuretics work better than newer, more expensive medicines. Diuretic use would reduce the estimated annual cost of $15.5 billion for hypertension drugs by about $300 million per year.
- The study demonstrates the need for large clinical trials to reveal critical information that significantly impacts the health of a large number of Americans afflicted with common conditions such as heart disease.

Communication of Results. Communicating scientific results and health information to the medical research community, health care providers, patients, and the public has long been recognized as a critical NIH function. Currently, NIH has four performance goals that focus on communicating the results of NIH supported research:

a) Increase awareness of NIH-sponsored research among health care providers to promote research application.

b) Increase awareness of NIH-sponsored research results among racial/ethnic minorities and high-risk, underserved, and/or affected publics.

c) Increase awareness of NIH-sponsored research results among the general public.

d) Increase awareness of clinical research and support participation in clinical trials.

Increasing public access to health information

Through the campaign, *Know Stroke. Know the Signs. Act in Time.*, NIH is increasing the number of people who are aware of the major symptoms of stroke and rapidly seek treatment.

For details see page 137.

Technology Transfer. NIH facilitates development of new drugs and other products to benefit human health by promoting efficient transfer to the private sector of new technology emanating from NIH research. In addition to improving public health, technology transfer contributes to the global competitiveness of the nation's businesses and to the nation's economic prosperity. NIH has two performance goals that seek to enhance its technology transfer activities:

Increasing the number of scientists who receive training in technology transfer.

Almost two-thirds of NIH scientists have received training in technology transfer. Approximately 2,450 NIH scientists have attended technology training seminars and NIH made completion of the On-line Technology Training Module a requirement for employees.

For details see page 144.

a) Increase the number of scientists who have received training in technology transfer.

b) Develop a system to identify and measure the health outcomes of technologies licensed by NIH.

Grants Administration and Peer Review. Excellence in the stewardship of Federal biomedical research funding means ensuring that NIH research is responsive to emerging public health needs, scientific opportunities, and new technologies and maintaining effective and efficient processes for reviewing, selecting, and administering extramural research grants. NIH has five goals in this area:

a) Improve electronic Research Administration (eRA) technology by developing capability for end-to-end electronic research administration by 2004.

b) Ensure proper stewardship of public funding for research.

c) Ensure that the NIH peer review process keeps pace with current advances in research and that the expertise of peer reviewers is appropriate for the needs of modern science.

Ensuring that the NIH peer review system keeps pace with advances in research.

The Center for Scientific Review (CSR) convened and received study section guidelines from 12 of 17 Study Section Boundaries teams, which are charged with redesigning CSR's peer review panel structure to keep pace with the rapidly changing landscape of biomedical research.

For details see page 158.

d) Develop innovative business practices to facilitate government/public interactions.

e) Improve grantee reporting of inventions developed with Federal funds.

Agency Management and Administrative Support. Successful administration of NIH requires maintaining effective internal management systems and providing strong administrative support to the research community. There are four goals in this area:

a) Improve the efficiency of the simplified acquisition process by continuing to expand the Purchase Card Program.

b) Expand the use of Performance Based Contracting (PBC).

c) Recruit and retain highly qualified extramural investigators to biomedical/behavioral research through the use of student loan repayment programs.

Expanding the use of performance based contracting (PBC).
NIH allocated over $417 million in PBCs in FY 2002. Only $36.5 million had been allocated to PBCs in the previous fiscal year. The size of the increase represents a major shift in the way NIH writes contracts and reflects a commitment to the Administration's PBC goals.

For details see page 170.

d) Implement government-wide initiative on delayering management levels and streamlining organization.

Research Training and Career Development Program

The six goals for the *Research Training and Career Development Program* support research training and outreach designed to ensure a continuing supply of well-trained scientists. NIH's Research Training and Career Development Program addresses the objectives of promoting development of a talent base of well qualified, highly trained, and diverse investigators capable of yielding the scientific discoveries of the future. NIH's performance goals for this Program are organized under one functional area: Training Support and Outreach. The goals demonstrate NIH's efforts to enhance training programs at the predoctoral, postdoctoral, and early career developmental levels:

a) Respond to the National Academy of Sciences quadrennial report on the future needs for health-related researchers.

b) Maintain adequate application and award rates in key training support areas.

c) Increase the pool of clinician researchers trained to conduct patient-oriented research.

d) Increase the participation of underrepresented racial and ethnic groups, women, and individuals with disabilities in NIH training and research programs.

e) Expand capabilities for electronic administration of research training and career development activities.

f) Improve the capabilities for career outcome tracking for NIH training and career development programs.

Research Facilities Program

The ten goals for the *Research Facilities Program* focus on modernizing and improving intramural and extramural research facilities to ensure that the nation's scientists have adequate facilities in which to conduct their work. NIH's Research Facilities Program addresses its long-term goal to "secure facilities for research that are modern, efficient, and safe." NIH's performance goals for this Program are organized under two functional areas: 1) Intramural Modernization and Improvements and (2) Extramural Assistance.

Intramural Modernization and Improvements. NIH has nine performance goals that demonstrate NIH efforts to construct, renovate, and maintain NIH research facilities located on the Bethesda campus and at off-campus field stations. The modernization and improvement of these facilities will enable NIH intramural researchers to continue to conduct state-of-the-art medical research in a safe and environmentally responsible environment.

a) Improve the operating conditions and environment of intramural facilities and the availability and reliability of NIH utility distribution systems to support intramural research.

b) Complete the Mark O. Hatfield Clinical Research Center.

c) Complete the Warren Grant Magnuson Clinical Center Revitalization Program.

d) Maintain the quality of the NIH Animal Care Program and construct a Central Vivarium.

e) Complete the John Edward Porter Neuroscience Research Center on the NIH Bethesda Campus.

f) Establish a systematic process to manage and account for NIH's Real Property Inventory.

g) Enhance NIH's ability to deter and respond to security threats by implementing campus and facility management initiatives.

NIH broke ground for construction of the Porter Neuroscience Research Center in September 2001.

The Porter Center will bring multiple disciplines and institutes working together, sharing lab space and sharing ideas to improve the pace of neuroscience discovery.

For details see page 251.

h) Provide research facilities to support biodefense by implementing design and construction actions.

i) Increase incorporation of feasible, cost-effective, environmental sustainability strategies into NIH planning, development, and operations by empowering a senior management

team accountable for the management and implementation of the Strategic Plan for Environmental Sustainability (SPES).

Extramural Assistance. NIH has one performance goal that focuses on key NIH extramural assistance activities related to the construction and modernization of non-federal facilities at academic institutions and other centers of research excellence to enhance their ability to initiate and continue to conduct high-quality research:

a) Approve an optimal percent of construction designs by the end of the third year that are in compliance with Federal and NIH design regulations and guidelines, and with other relevant local, national, and international codes and standards.

Program Performance Report Summary Table

This summary of performance as of November 1, 2003, is provided only as a descriptive indicator of the status of our targets from one year to the next. Because goals and targets vary with regard to degree of importance to the NIH mission, summary data does not accurately reflect NIH's overall success.

Program Performance Report Summary Table					
Fiscal Year	**Goals**	**Targets**	**Results Reported**	**Targets Met**	**Targets Extended**
1999	46	86	86	80	1
2000	44	88	88	78	3
2001	36	90	90	83	2
2002	40	80	79	65	12
2003	60	97	To be reported in February 2004.		
2004	53	81	To be reported in February 2005.		

Part I.
Overview of Performance Measurement

1.1 NIH Mission and Objectives

The mission of the National Institutes of Health (NIH) derives from Section 301 of the Public Health Service Act.

The NIH mission is to uncover new knowledge that will lead to better health for everyone.

At a finer level of articulation, this mission is expressed in the following objectives:

- *Increase understanding of normal and abnormal biological functions and behavior.*
- *Improve prevention, diagnosis, and treatment of diseases and disabilities.*
- *Promote development of a talent base of well qualified, highly trained, and diverse investigators capable of yielding the scientific discoveries of the future.*
- *Secure facilities for research that are modern, efficient, and safe.*

The agency's activities and strategies discussed throughout this plan are directed at realizing these overarching objectives.

1.2 Report/Plan Roadmap

For purposes of planning and performance assessment under GPRA, NIH organizes its activities into three core programs that correspond with the agency's objectives. The three core GPRA programs are the Research Program, the Research Training and Career Development Program, and the Research Facilities Program. This aggregated approach is implemented due to the crosscutting nature of disease and scientific discovery. Although each of the institutes and centers (ICs) has a specific research orientation, there are many commonalties. Multiple ICs often address different aspects of the major health problems faced by our citizens. Disease is typically systemic, influenced by multiple factors and affects more than one organ or body system. Diverse expertise is usually required to fully understand a disease's etiology, diagnosis, treatment, and prevention and the efforts of many ICs need to be brought to bear on a particular disease or disability. By aggregating activities that are intrinsically collaborative and complementary, NIH neither omits nor minimizes the significance of any particular activity that contributes to a major function or operation for the agency as a whole. This aggregated approach also is taken to integrate the performance plan with the NIH budget.

The ***Research Program***, which corresponds with the first two objectives, encompasses the support of investigations across the full range of the medical research continuum, including basic research, which may be disease-oriented; observational and population-based research; behavioral research; health services research; and clinical research. Clinical research includes research to understand both normal health and disease states, to move laboratory findings into medical applications, and to assess new treatments or to compare different treatment approaches. In addition, the timely dissemination of medical and scientific information is a key component of the Research Program, as is the expeditious transfer of the results of NIH-funded medical research to benefit human health.

The ***Research Training and Career Development Program***, which corresponds with the third objective, addresses the need for creative and capable personnel to conduct medical research. NIH undertakes support for graduate training and career development in order to nurture new, highly trained investigators and to hone and expand the skills of those already performing research to benefit the nation's health. Our ability to maintain the momentum of recent scientific progress and our international leadership in medical research depends upon the continued development of such talent.

The ***Research Facilities Program***, which corresponds with the fourth objective, focuses on ensuring that NIH-supported scientists have adequate facilities in which to conduct their work. In fact, many of the advances in medical research that are leading to more effective treatments for illnesses reflect innovations in sophisticated, but often costly, research technologies that are far beyond the capacity of all but a handful of institutions to purchase, construct, or maintain. NIH recognizes that ensuring broad access to these research facilities creates efficiencies that make the research dollar go farther, while providing critical resources to many scientists.

1.3 Organizational Overview

The core NIH programs are implemented by an array of individual Institutes and Centers that work individually and collectively in partnership with an extensive extramural research community.

The Institutes and Centers. NIH is composed of 27 Institutes and Centers (ICs), whose research activities extend from basic research that explores the fundamental workings of biological systems and behavior, to studies that examine disease and treatments in clinical settings, to prevention, and to population-based analyses of health status and needs. (A brief mission statement for each of the ICs appears in Appendix 6.) The Office of the Director, NIH, provides leadership, oversight, and coordination for the enterprise.

The ICs are the NIH "visible" to most Americans. Some of the ICs focus on diseases (e.g., cancer, diabetes); others concentrate on organ systems (e.g., heart, eye, kidney), while others focus on a stage of life (e.g., children, the aging). Yet, no less essential to the nation's health are NIH ICs that address overarching scientific needs and opportunities. Included here are such efforts as deciphering the human genome, understanding cellular and tissue biology and physiology, and developing the array of technologies dictated by the needs of cutting-edge

research. All are scientific innovations that lead to discovery, move research findings into clinical practice, and/or lead to improvements in the quality of routine medical treatment.

NIH supports research, research training and career development, and research facilities through both extramural and intramural activities.

The Extramural Community. The extramural community is composed of non-federal scientists at universities, medical centers, hospitals, and research institutions throughout the country and abroad. With NIH support, these investigators and their institutions conduct the lion's share of the research that uncovers new knowledge that leads to improvements in the prevention, detection, diagnosis, and treatment of disease and disability. In tandem with the conduct of research, the extramural community contributes to training the next generation of researchers, enhancing the skills and abilities of established investigators, and renewing the infrastructure for NIH-sponsored research.

More than $8 out of every $10 dollars appropriated to NIH flows out to the scientific community at large. This "extramural" system is premised on *independence*, embodied in "investigator-initiated" research; on *self-governance*, embodied in peer review of scientists by scientists as the primary basis for judging the merits of research proposals and awarding funds; and on the powerful incentive of *competition* among the most highly trained scientists in the world. The extramural research community numbers more than 50,000 scientists affiliated with approximately 1,700 universities, hospitals, and other research facilities located in all 50 states, the District of Columbia, Puerto Rico, Guam, the Virgin Islands, and points abroad.

Research at NIH's Intramural Laboratories. A much smaller fraction of NIH funds, approximately 10 percent of the budget, supports a core program of basic and clinical research activities administered and staffed by NIH's own physicians and scientists. Approximately 1,200 scientists head intramural research projects. This in-house, or intramural, research program includes the NIH Clinical Center and other resources that provide scientific, clinical, and educational benefits to the citizens of the U.S. and the world.

1.4 Operational Strategies

Sustained and diligent Federal stewardship is the touchstone for the planning and management activities carried out by each IC as they implement the core programs that embody the NIH mission. The fundamental principles that underpin this stewardship, and thus guide the utilization of resources for program purposes, are described below. These principles ensure the relevance, quality, and performance of NIH programs.

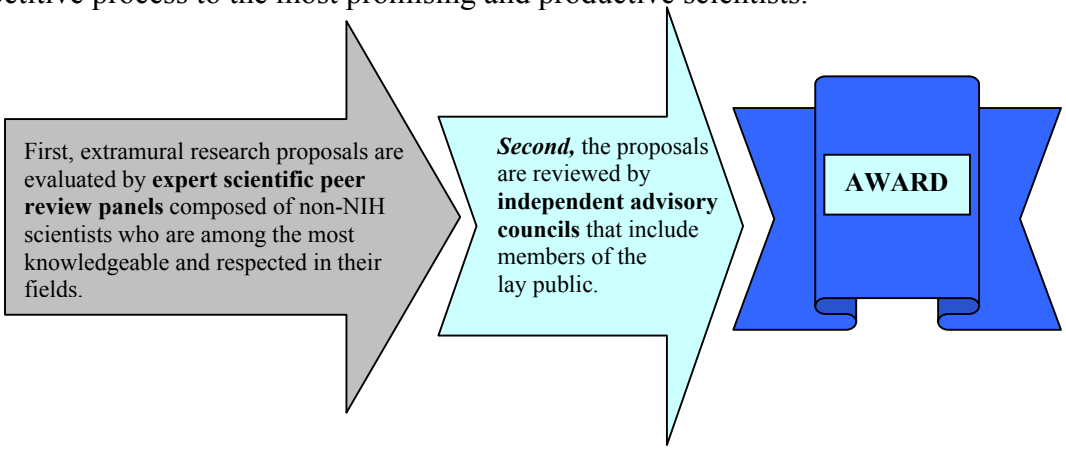
Provide scientific leadership and establish research priorities. Establishing research priorities is essential to ensure that science meets national public health needs and efficiently uses limited resources. In general, NIH sponsors research that addresses burden of illness – ways to prevent, treat, or cure disease and to minimize pain and suffering. But addressing burden alone is not enough; there must also be some real opportunity for success.

How do we identify areas of increased scientific opportunity? New knowledge comes from the pursuit of answers to gaps in knowledge. The rate-limiting step in the generation of new knowledge is not the number of experiments conducted, but rather the number of new hypotheses or questions. When an arena of research is enjoying an exponential increase in the number of new questions, it is, indeed, an area of scientific opportunity. New questions emerge as a result of several converging factors, including the creativity of individual investigators, the emergence of new methods and tools that allow previously unanswered questions to be addressed, and what is already known about a problem. It is imperative that NIH capitalizes on such areas of scientific opportunity.

NIH uses a multi-level system to establish and review research priorities. The NIH Director, in collaboration with IC Directors and their respective advisory councils and boards and the biomedical research community, guides the priority-setting process. Additional input is sought from the Administration, including the Department of Health and Human Services (DHHS), Congress, and the public. The NIH considers the research priorities identified through this process and makes resource allocation decisions that are intended to ensure that NIH commits federal resources to projects and programs that are relevant and are most likely to achieve the greatest yield from the nation's medical research investment.

In short, understanding burden of illness, identifying knowledge gaps, and deciding how to best capitalize on scientific opportunities are the primary drivers in the allocation of resources.

Fund the best extramural research and training. NIH funds are awarded through a highly competitive process to the most promising and productive scientists.



This two-tiered independent review system is critical to ensuring that the best proposals are funded from the approximately 44,000 research and training applications NIH receives each year.

Conduct leading-edge research in NIH laboratories. NIH also ensures that the research conducted in its own (intramural) laboratories is of the highest caliber. Each Institute maintains a Board of Scientific Counselors, composed of external experts, that reviews the intramural programs and makes recommendations to the Institute Director. The intramural program enables scientists to apply the results of laboratory research to patient care and to seek answers in the laboratory to questions that arise in the clinical setting. This national resource permits NIH to

respond rapidly to critical health problems and emergencies and to take advantage of emerging opportunities.

Collaborate and coordinate with others. NIH collaborates and coordinates on an ongoing basis with other Federal agencies and research organizations where research interests intersect and when joint efforts will enhance the individual activities of each entity. Medical research benefits from multiple perspectives being brought to bear on a particular problem. Collaborative efforts bring diverse domains of expertise together and can facilitate a more rapid response to emerging opportunities. In addition, collaborative efforts work to produce the best possible science while making the most economical use of the resources available.

These collaborative endeavors frequently involve NIH's sister agencies in DHHS, including the Food and Drug Administration (FDA), Centers for Disease Control and Prevention (CDC), and the Agency for Healthcare Research and Quality (AHRQ). Nonetheless, the full scope of NIH's collaborative activities, both in the past and those contemplated for the future, is far wider. NIH partners include many other federal agencies, government bodies, non-governmental organizations, and industry. More details on NIH partnership and collaboration are provided in Appendix 3.

1.5 Budget Linkage to GPRA Programs

The FY 2004 HHS budget submission provides funding to support each of the core NIH programs. The following table provides a six-year summary of funding by NIH GPRA program.

GPRA Program	**FY 2004 HHS Budget Submission** (dollars in thousands)					
	FY 1999 Actual	**FY 2000** Actual	**FY 2001** Actual	**FY 2002** Actual	**FY 2003** Amended President's Budget	**FY 2004** Request
Research	$14,580,705	$16,692,719	$19,214,206	$21,883,989	$24,734,244	$26,426,569
Research Training and Career Development	811,120	912,241	1,023,475	1,156,532	1,259,660	1,304,214
Research Facilities	239,343	251,747	248,386	429,553	1,273,739	83,208
All Programs	**$15,631,168**	**$17,856,707**	**$20,486,067**	**$23,470,074**	**$27,267,643**	**$27,813,991**

Budget and Performance Integration. In NIH's budget and cost accounting system, dollars are not directly associated with GPRA goals (such association is not required under GPRA). Rather, Congress appropriates funds to each NIH institute and center and funds are distributed through specific budget mechanisms that, in aggregate, support NIH's three core programs – Research, Research Training and Career Development, and Research Facilities. The following "crosswalk"

table provides an overview of the budget mechanisms that correspond to each core program and the amount of FY 2002 funds that were used to support each core program. The dollar amounts cited in the "Budget Mechanism" column correspond to amounts provided in Volume I of this Congressional Justification. (See the Budget Mechanism table in the Overview section; but as indicated in the footnotes to the crosswalk, some budget mechanisms are prorated across GPRA programs e.g., the Research Management and Support activity.) As indicated in the table, Research Project Grants (RPGs) are the primary source of support for extramural research. As such, RPGs allow NIH to sustain the momentum of investigator-initiated research while providing new research opportunities.

Crosswalk Between GPRA Programs and NIH Budget Mechanisms			
Budget Mechanism	**FY 2002 Actual (000's)**	**NIH GPRA Program**	**Program Resources (000's)**
Research Project Grants	$13,015,894	**Research** • Research • Communication of Results • Technology Transfer • Grants Administration and Peer Review • Agency Management and Administrative Support	**$21,883,989**
Intramural Research	$2,234,015		
Research Centers	$2,116,928		
Research and Development Contracts	$1,790,125		
Cancer Prevention and Control	$486,622		
Library of Medicine	$274,284		
Other Research[1]	$986,078		
Research Management and Support[2]	$732,819		
Office of the Director[3]	$247,224		
Research Training	$653,257	**Research Training and Career Development`** • Training Support and Outreach	**$1,156,532**
Other Research[1]	$459,998		
Research Management and Support[2]	$38,728		
Office of the Director[3]	$4,549		
Buildings and Facilities	$295,879	**Research Facilities** • Intramural Modernization and Maintenance • Extramural Assistance	**$429,553**
Construction	$117,600		
Research Management and Support[2]	$14,384		
Office of the Director[3]	$1,690		
All Mechanisms	**$23,470,074**	**All Programs**	**$23,470,074**

[1] The Other Research budget mechanism supports both the Research Program and the Research Training and Career Development Program
[2] The Research Management and Support budget mechanism supports all programs
[3] The Office of the Director budget mechanism supports all programs

Part II.
Goal-by-Goal Performance Measurement

Consistent with the Department of Human Health and Service (DHHS) approach to GPRA, this document (Volume II of the FY 2004 Congressional Justification for the National Institutes of Health) integrates NIH's Annual Plan and Annual Report into a single document to eliminate redundancy and to present the progress toward agency program goals and across multiple years.

- The planning elements of this document describe NIH's mission, programs, goals, and the annual performance expectations (targets) that mark the path toward achieving these goals. The goals and targets described for FY 2004 are "final " and for FY 2003 are "revised final".

- The reporting elements of this document describe NIH's accomplishments and progress toward goal achievement during FY 1999 - FY 2002. A few FY 2003 targets already have been met and those accomplishments are noted as well.

Part II of the Plan presents all of the NIH goals and targets organized according to the three Core Programs that NIH identifies for GPRA purposes – Research, Research Training and Career Development, and Research Facilities. The following information is provided for each program:

- The ***Program Description and Context*** provides an overview of the program, including the functional areas within the program.

- The ***Summary of Performance*** provides a snapshot, by year, of performance on the targets under each goal, within each functional area, of the program. The summary also provides linkage to the budget.

- The ***Program Performance Analysis*** provides detailed information on progress toward each goal. Goals are presented by functional area within each program. The details include information about the significance of each goal, summary charts displaying performance targets and results, and summary discussions of performance results. To allow the reader to easily perceive the status of each target, the following codes are used in the summary charts:

  - ◆ **Target Met** – "Target Met" indicates that NIH's actual performance met or surpassed the stated target for quantitative/objective goals.

  - ◇ **Target Active** – Indicates when NIH plans to meet the target.

  - → **Target Not Met and Extended** – Indicates that actual performance fell short of the target and that NIH extended the timeframe for meeting the target.

× **Not Met** – Indicates that actual performance fell short of the target and that the target was specific to a particular fiscal year. No further action can be taken to achieve the target.

TBD **To Be Determined** – Indicates that the data needed to determine whether the goal was met are not yet available.

2.1 Research Program

2.1.1 Program Description and Context

NIH's research activities range widely across the medical research continuum, and include basic and disease-oriented research, observational and population-based research, behavioral research, health services research, and clinical research. Clinical research includes research to understand both normal health and disease states, to move laboratory findings into medical applications, and to assess new treatments or compare different treatment approaches.

While the specific research activities undertaken by the agency's numerous Institutes and Centers—through the intramural labs or grants to the extramural medical research community—are many, the universal objectives are 1) to increase understanding of normal and abnormal biological functions and 2) to utilize this new knowledge in developing improved prevention, diagnosis, and treatment options for diseases, disabilities, and other adverse human conditions.

Scientific research probes and seeks to understand the unknown. The scientific insights that provide a basis for solutions usually accumulate over many years, and often are derived from the efforts of diverse investigators working on and communicating about differing facets of the problem. Medical discovery is marked by stops and starts, and a vital interplay between theory, experimental evidence, and clinical observations. It is very hard if not impossible to predict what discoveries will arise or to anticipate the opportunities that such new knowledge will provide. Accordingly, NIH must support research along a broad, and, of necessity, expanding front. NIH's medical research program is a diverse and continually evolving portfolio that reflects the agency's obligation to respond to public health needs, a commitment to support research of the highest scientific caliber, and judgment as to the scientific opportunities that offer the best prospects for new knowledge and better health.

Successful outcomes from the research program mean a continuing flow of high quality research, discoveries of new fundamental knowledge, applications in new therapies, diagnostics, prevention, and new research tools originating from extramural grantees and the intramural laboratories. They also mean timely dissemination of scientific results and research-based health information and expeditious transfer of the results of medical research for further development and commercialization of products of immediate benefit to improved health as an important mandate.

As the strategic principles that broadly guide NIH's research program indicate, success in mission achievement also involves effective implementation in several key management/process areas. Notably, these include effective mechanisms for grants management, high-quality projects for the portfolio, and effective management/administrative support.

NIH Research Program Activities

Research Outcomes–NIH's ongoing scientific enterprise. This includes research conducted through grant awards and contracts to individual investigators and organizations in the Extramural Research community. It also includes research conducted at NIH's Intramural labs. The intended long-run outcomes of all these activities are increased understanding of normal and abnormal biological functions and behavior and improved prevention, diagnosis, and treatment of diseases and disabilities.

Communication of Results–Communicate scientific results and health information to the medical research community, health care providers, patients, and the general public.

Technology Transfer–Promote the efficient transfer of the new technology forthcoming from NIH research to the private sector to facilitate the development of new drugs and other products of benefit to human health.

Grants Administration and Peer Review–Maintain effective and efficient grants administration and a high quality of peer review to ensure the most meritorious research projects are considered for funding.

Agency Management and Administrative Support–Ensure that management and administrative functions necessary to support the agency's mission are carried out effectively and efficiently.

2.1.2 Summary of Performance – Research Program

Comprehensive summary tables covering all the goals and targets in NIH's Research Program follow. These tables provide updated information on the status of all of the program's performance targets. More extensive information on each goal, including a chart summarizing the performance results for each target, can be found at the referenced page number for details "D - #."

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
a) Add to the body of knowledge about normal and abnormal biological functions and behavior.	**FY 2002** Annual milestones that may include descriptions of science advances and stories of discovery; and initiatives such as Requests for Announcements (RFAs), Program Announcements (PAs), conferences and workshops.	FY 2002 performance was not subjected to an independent assessment. The performance report is available at http://www1.od.nih.gov/osp/ospp/gpra/gpra_nih.htm	SP - 1, 4 D - 65
	FY 1999 – FY 2001 Progress in advancing scientific understanding in key fields bearing on our knowledge of biological functions and behavior in their normal and abnormal states.	An independent Research Assessment Working Group reviewed annual progress and made the following assessments: **FY 2001** – Target substantially exceeded. **FY 2000** – Target substantially exceeded. **FY 1999 –** Target substantially exceeded.	
b) Develop new or improved instruments and technologies for use in research and medicine.	**FY 2002** Annual milestones that may include descriptions of science advances and stories of discovery; and initiatives such as Requests for Announcements (RFAs), Program Announcements (PAs), conferences and workshops.	FY 2002 performance was not subjected to an independent assessment. The performance report is available at http://www1.od.nih.gov/osp/ospp/gpra/gpra_nih.htm	SP - 4 D - 67

[1] —Indicates that the goal is part of the President's Management Agenda
SP-#—Indicates the DHHS Strategic Plan (October 2002 draft) goal to which each GPRA goal pertains
HP-#—Indicates the Chapter of "Healthy People 2010" to which each goal pertains
CDC-#—Indicates a related goal in the CDC's GPPA plan. See Appendix 1.
FDA-#—Indicates a related goal in the FDA's GPRA plan. See Appendix 1.
D-#—Indicates the page in this report at which details on the goal can be found

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	FY 1999 – FY 2001 Progress in developing new instrumentation or technologies that enhance capabilities for investigating biological functions and diagnosing and treating diseases and disorders.	An independent Research Assessment Working Group reviewed annual progress and made the following assessments: **FY 2001** – Target substantially exceeded. **FY 2000** – Target substantially exceeded. **FY 1999 –** Target substantially exceeded.	
c) Develop new or improved approaches for preventing or delaying the onset or progression of disease and disability.	**FY 2002** Annual milestones that may include descriptions of science advances and stories of discovery; and initiatives such as Requests for Announcements (RFAs), Program Announcements (PAs), conferences and workshops.	FY 2002 performance was not subjected to an independent assessment. The performance report is available at http://www1.od.nih.gov/osp/ospp/gpra/gpra_nih.htm	SP – 1, 4 D – 69
	FY 1999 – FY 2001 Progress in developing (or facilitating the private sector's development of) new or improved approaches for preventing or delaying the onset of diseases and disabilities – and which reflect NIH responsiveness to emerging health needs, scientific opportunities, and new technologies.	An independent Research Assessment Working Group reviewed annual progress and made the following assessments: **FY 2001** – Target substantially exceeded. **FY 2000** – Target substantially exceeded. **FY 1999 –** Target successfully met.	
d) Develop new or improved methods for diagnosing disease and disability.	**FY 2002** Annual milestones that may include descriptions of science advances and stories of discovery; and initiatives such as Requests for Announcements (RFAs), Program Announcements (PAs), conferences and workshops.	FY 2002 performance was not subjected to an independent assessment. The performance report is available at http://www1.od.nih.gov/osp/ospp/gpra/gpra_nih.htm	SP – 1, 4 D – 71
	FY 1999 – FY 2001 Progress in developing (or facilitating the private sector's development of) new or improved diagnostic methods that are more accurate, less invasive, and/or more cost-effective -- and which reflect NIH responsiveness to emerging health needs, scientific opportunities, and new technologies.	An independent Research Assessment Working Group reviewed annual progress and made the following assessments: **FY 2001** – Target substantially exceeded. **FY 2000** – Target substantially exceeded. **FY 1999 –** Target substantially exceeded.	

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
e) Develop new or improved methods for treating disease and disability.	**FY 2002** Annual milestones which may include descriptions of science advances and stories of discovery; and initiatives such as Requests for Announcements (RFAs), Program Announcements (PAs), conferences and workshops.	FY 2002 performance was not subjected to an independent assessment. The performance report is available at http://www1.od.nih.gov/osp/ospp/gpra/gpra_nih.htm	SP – 1, 4 D – 72
	FY 1999 – FY 2001 Progress in developing (or facilitating the private sector's development of) new or improved treatments that expand therapy options; improve the length and quality of life; and/or are more cost effective -- and which reflect NIH responsiveness to emerging health needs, scientific opportunities, and new technologies.	An independent Research Assessment Working Group reviewed annual progress and made the following assessments: **FY 2001** – Target substantially exceeded. **FY 2000** – Target substantially exceeded. **FY 1999 –** Target substantially exceeded.	
f) Develop critical genomic resources, including the DNA sequences of the human genome and the genomes of important model organisms and disease-causing microorganisms. FY 2002 will be the last year on which NIH reports performance on this goal. Goals 7c and 8a under the new NIH approach will supersede the present goal.	**FY 2002** 1. Finish two-thirds of the human genome (accuracy of at least 99.99%). NIH grantees will be responsible for half of this target, i.e., one-third of the human genome.	1. The International Human Genome Sequencing Consortium surpassed this goal, by finishing over 88% of the human genome. NIH grantees have completed roughly half of this amount, i.e., over 42% of the human genome (considerably more than their goal of 33%). The essentially complete sequence of the human genome is expected to be achieved in FY 2003.	SP – 4 ---------- HP – 2,3,4,5,8, 12,13,14, 16,18,19, 21,24,25, 26,27,28 ---------- FDA– 3,4 ---------- D - 73
	2. Obtain full-length clones and sequence data for 20,000 mammalian cDNAs.	2. The goal for FY 2002, to obtain sequence data for 20,000 putative full-open reading frame mammalian cDNAs, was exceeded, with the centers participating in the project sequencing 23,000 clones. Over a two-year period, and accounting for redundancy, a total of 19,600 unique sequences with complete open reading frames have been submitted to public databases. A manuscript outlining the progress of the project has been submitted for publication.	

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	3. Complete full shotgun coverage of the sequence of the mouse genome; finish 10% of the mouse genome.	3. By the end of FY 2002, 10.9% of the mouse genome was finished. The mouse genome-sequencing project completed the whole genome shotgun phase of the project and achieved approximately 7X shotgun coverage, containing 96% of the mouse genome in an assembled sequence. The Mouse Genome Sequencing Consortium is about to publish a landmark paper that describes the first analysis of this data.	
	4. Complete 3X sequence coverage of the rat genome.	4. By the end of FY 2002, the rat genome sequencing project generated approximately 5.5X coverage through adding random whole genome shotgun reads to light shotgun coverage of a set of genomic clones.	
	5. Initiate pathogen genome sequencing projects for additional NIH priority areas based upon Blue Ribbon Panel Report.	5. Two important sequencing projects identified by the 1999 Blue Ribbon Panel as among the twenty top sequencing priorities received initial funding in FY 2002─*Trichomonas vaginalis* and *Aedes aegypti*. Genetic comparison of isolates of *Bacillus anthracis* was also performed in FY 2002 based on goals of the 2002 Blue Ribbon Panel on Bioterrorism.	
	6. Establish a mechanism to facilitate access to resources, services, and technologies (bioinformatics, scanning, micro arrays, genome chips) needed to investigate microbial gene function.	6. In FY 2001, NIH awarded a contract for a Pathogen Functional Genomics Resource Center (PFGRC) for the functional analysis of pathogen genome sequences. Expansion of this resource in FY 2003 and FY 2004 is likely. Additional initiatives are being developed to support genomic sequencing, microbial genome databases and bioinformatics, and proteomics development research.	

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	7. Develop technologies that assess, display, and/or query human genome sequence data to facilitate investigation of how the immune system responds during different disease conditions (i.e., infection, transplantation, autoimmune disease, and other diseases).	7. Progress toward the development of new technologies is being made through the International Histocompatibility Working Group (IHWG), a Proteomics Facility as a Core project within a Center for Translational Research in Human Immunology, an innate immune system Request for Proposals (RFP), the Multiple Autoimmune Disease Genetics Consortium (MADGC), and the North American Rheumatoid Arthritis Consortium (NARAC).	
	FY 2001 1. Worldwide effort completes "full shotgun" of human genome sequence (95% complete, 99.9% accurate).	1. The Human Genome Project public consortium succeeded in meeting its target to complete a "full shotgun" of human genome sequence.	
	2. Finish one-third of human genome (accuracy of at least 99.99%).	2. As of September 30, 2001, 54 percent of the genome was in the completely finished form that has no remaining, closable gaps and an accuracy of 99.99 percent.	
	3. Identify 2,500,000 human single nucleotide polymorphisms (SNPs).	3. As of September 30, 2001, the public database that serves as a central repository for SNPs, dbSNP, had received submissions for 3,845,467 SNPs for the human genome, 2,421,261 of which are non-redundant.	
	4. Complete 2X depth of coverage toward the working draft of the mouse genome (90% coverage, 99% accurate).	4. 3X depth of coverage of the mouse DNA sequence has been achieved.	
	5. Complete 1X depth of coverage toward the working draft of the rat genome (90% coverage, 99% accurate).	5. The rat sequencing consortium has completed nearly 2X coverage of the rat genome in whole genome sequence reads.	
	6. Complete worldwide sequencing effort of the entire genome of *Plasmodium falciparum*.	6 NIH supported the sequencing of three of the fourteen chromosomes of *P. falciparum*, specifically chromosomes 2, 10, and 11. The complete genome sequence of *P. falciparum* was published in October 2002.	

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	7. Complete sequencing of five additional bacterial pathogens and five chromosomes of protozoan parasites.	7. The genome sequences of five bacterial pathogens were published in FY 2001. Also in FY 2001, manuscripts were in preparation for an additional five bacterial pathogens. Sequencing of the five chromosomes of protozoan parasites advanced in FY 2002 with publication of the sequences for two more *P. falciparum* chromosomes. Sequencing of four other chromosomes of protozoan parasites (*Giardia lamblia*, *Leishmania major*, *Trypanosoma brucei*, and *Trypanosoma cruzi*) is ongoing, and is expected to be completed in FY 2004.	
	8. Augment existing knowledge of pathogen genomes by initiating sequencing projects for at least six additional genomes (bacterial, fungal, parasitic).	8. NIH initiated genome-sequencing projects in FY 2001 for nine pathogens.	
	FY 2000 1. Worldwide effort completes "working draft" of human genome sequence (90% complete, 99% accurate). U.S. contributes two-thirds of that amount, and NIH contributes 85% of U.S. total.	1. The Human Genome Project public consortium completed a "working draft" of the sequence of the human genome.	
	2. Finish the sequence of at least one human chromosome.	2. The Human Genome Project public consortium completed the "finished" sequence of two human chromosomes.	
	3. Complete sequence of the genome of *Drosophila melanogaster* (excluding heterochromatin).	3. A consortium of publicly funded scientists, in collaboration with a private company, published the genome sequence of the fruit fly (*Drosophila melanogaster*).	
	FY 1999 1. Reach U.S. annual production rate of human genomic sequence of 90 million base-pairs.	1. An annual U.S. production rate of 173 million base-pairs was achieved.	
	2. Reach worldwide annual production rate of human genomic sequence of 220 million base-pairs.	2. An annual worldwide production rate of 265 million base-pairs was achieved.	
	3. Total human genomic sequence completed worldwide at the end of FY 1999 at 400 million base-pairs.	3. The worldwide completed sequence achieved was 442 million base-pairs.	

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	4. Complete the sequence of the *Caenorhabditis elegans* genome (97 million base-pairs).	4. The complete sequence of the *Caenorhabditis elegans* genome was published.	
g) Develop an HIV/AIDS vaccine by 2007. *Because this goal is converting to the format of the new NIH research outcome goals, no future targets are indicated here*.	**FY 2002** 1. Progress in the design and development of new or improved vaccine strategies and delivery/production technologies. 2. Progress in characterization, standardization, and utilization of animal models for evaluating candidate vaccines. 3. Progress in completion of ongoing trials. 4. Progress in initiation of additional domestic and/or international trials of new or improved concepts and designs, including the progression of promising candidates to larger trials testing vaccine candidates.	1. Vaccine candidates and concepts evaluated preclinically in FY 2002 include improved DNA vaccines, a stabilized HIV envelope protein, novel viral vectors, and HIV regulatory proteins. There are now over 20 candidate preventive vaccines advancing toward clinical studies by NIH-funded researchers in academia and the private sector. 2. In FY 2002, a subcontract was awarded to support the creation and implementation of a non-human primate vaccine database in order to better track the growing numbers of animals under study through the Simian Vaccine Evaluation Units. Progress in the development of small animal models (the rat and the mouse) of HIV infection continued. 3. In FY 2002, NIH completed six trials. 4. In FY 2002, NIH initiated three new phase I trials.	SP – 1, 2, 4 ---------- HP – 13,14 ---------- D – 85

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	5. Progress in collaborating with scientists in developing countries and with industry to further promote the development of vaccines for worldwide use.	5. In FY 2002, an additional HIV Vaccine Design and Development Team (HVDDT) contract was awarded to Wyeth-Lederle Vaccines to study HIV vaccines delivered using vesicular stomatitis virus vectors. The HIV Vaccine Trial Network (HTVN) added additional sites in Puerto Rico, Brazil, Peru and South Africa. And in the Comprehensive International Program of Research on AIDS (CIPRA) program to develop research infrastructure in developing countries, awards were made to support multi-disciplinary research projects in China and South Africa. Smaller, planning and organizational grants were also awarded to institutions in Brazil, Vietnam, Dominican Republic, Cambodia, Thailand, India, Zambia, Mexico, Tanzania, Congo, and Zimbabwe.	
	FY 2001 1. Progress in the design and development of new or improved vaccine strategies and delivery/production technologies.	1. Advances in the design and development of vaccine strategies during FY 2001 continued to fuel the pipeline of promising HIV/AIDS vaccine candidates. Vaccine candidates and concepts evaluated preclinically in FY 2001 include improved DNA vaccines, a stabilized HIV envelope protein, novel viral vectors, and HIV regulatory proteins.	
	2. Progress in characterization, standardization, and utilization of animal models for evaluating candidate vaccines.	2. Animal models, especially the use on non-human primates, continue to provide valuable information in advancing HIV vaccine research and in testing candidate vaccines. Advances in FY 2001 utilized non-human primates in their evaluation of candidate vaccines. In addition, progress was made in development of small animal models (the rat and the mouse) of HIV infection.	
	3. Progress in completion of ongoing trials.	3. In FY 2001, NIH completed seven clinical trials.	

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	4. Progress in initiation of additional domestic and/or international trials of new or improved concepts and designs, including the progression of promising candidates to larger trials testing vaccine candidates. 5. Progress in collaborating with scientists in developing countries and with industry to further promote the development of vaccines for worldwide use.	4. NIH initiated two new phase II vaccine trials in FY 2001, one domestic, and the other in the Caribbean and Brazil. These trials are being conducted under the direction of the HIV Vaccine Trials Network. 5. In FY 2001, NIH initiated an international phase II clinical trial through the HIV Vaccine Trials Network. In addition, activities were completed to increase collaboration with industry that included: a solicitation for additional new HIV Vaccine Design and Development Teams, awarding of five new Vaccine Developmental Resources Contracts to private companies, and signing of a Cooperative Research and Development Agreement with Merck.	
	FY 2000 1. Progress in the design and development of new or improved vaccine strategies and delivery/production technologies. 2. Progress in characterization, standardization, and utilization of animal models for evaluating candidate vaccines. 3. Progress in completion of ongoing trials. 4. Progress in initiation of additional domestic and/or international trials of new or improved concepts and designs, including the progression of promising candidates to larger trials testing vaccine candidates. 5. Progress in collaborating with industry to enhance opportunities for vaccine development.	1. Notable progress was made both scientifically and programmatically. 2. A variety of animal models were utilized to make important advances. 3. Three ongoing trials were completed. 4. One new trial was initiated. 5. HIV Vaccine Design and Development Team awards were made that substantially promote university-industry collaboration.	
	FY 1999 1. Increases in the research portfolio supporting innovative vaccine discovery. 2. Progress in completion of ongoing trials	1. The number and dollar value of awards made for vaccine discovery increased. 2. Of the seven trials started in prior fiscal years, two were completed.	

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	3. Progress in initiation of additional domestic and/or international trials of new or improved concepts and designs, including the progression of promising candidates to larger trials testing vaccine candidates. 4. Increased interactions between academic investigators and industry to enhance opportunities for vaccine discovery and product development.	3. Four new trials were begun. 4. Actions were taken to increase the interaction of academic investigators and industry.	
Research Outcome Goals for FY03 and beyond (See page 92 for presentation of the research outcome goals in the matrix format.)	*Milestones/targets will be defined as plans for annual performance reporting are developed.*		
1a Conduct medications development with use of animal models, and begin to conduct Phase I and II trials of two potential treatments for alcoholism: cannabinoid antagonist Rimonabant and corticotropin-releasing hormone antagonist Antalarmin.			SP – 1.4, 4.1 --- HP – 26 --- D – 93
1b By 2006, develop one or more prototypes for a low-power, highly directional hearing aid microphone to help hearing-impaired persons better understand speech in a noisy background.			SP – 4.1, 6.2 --- HP – 28 --- D – 94
2a By 2007, demonstrate the feasibility of islet transplantation in combination with immune tolerance induction for the treatment of type 1 diabetes in human clinical studies.			SP – 4.1 --- HP – 5 --- D – 95
2b By 2009, evaluate the efficacy of two novel approaches to prevent weight gain and/or treat obesity in clinical trials in humans.			SP – 4.1 --- HP – 19 --- D – 96
2c Develop methods that can classify at least 75% of proteins from sequenced genomes according to evolutionary origin and biological structure.			SP – 4.1 --- D – 97

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
2d By 2007, develop an HIV/AIDS vaccine.			SP – 1.2, 4.1 --- HP – 13 --- CDC – 5 --- D – 98
3a Identify at least one clinical intervention that will delay the progression, delay the onset, or prevent Alzheimer's disease.			SP – 4.1, 6.2 --- HP – 18 --- D – 99
3b By 2010, develop one universal antibiotic effective against multiple classes of biological pathogens.			SP – 2.1 4.1 --- HP – 14, 24 --- CDC – 6, 7, 9 --- FDA – 1 --- D – 100
3c Determine the efficacy of using salivary diagnostics to monitor health and diagnose at least one systemic disease by 2013.			SP – 4.1 --- D – 101
4a By 2005, develop two new animal models to use in research on at least one agent of bioterror.			SP – 2.1, 4.1 --- HP – 14 --- CDC – 7, 9 --- FDA – 1, 2 --- D – 102

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
4b By 2005, develop improved animal models that best recapitulate Parkinson's Disease (PD), based emerging scientific findings of genetic or environmental influences, or interactions of genes and the environment on the development of PD.			SP – 4.1 --- HP – 8 --- D – 103
4c By FY 2007, identify 20 small molecules that are active in models of nervous system function or disease and show promise as drugs, diagnostic agents, or research tools.			SP – 4.1 --- HP – 26, 18 --- D – 105
5a By 2007, evaluate the efficacy of three new treatments strategies for HIV infection in phase II/III human clinical trials in an effort to identify drugs that are more effective, less toxic, and/or simpler to use than the current recommended HIV treatment regimen.			SP – 4.1 --- HP – 13 --- D – 106
5b Establishing the efficacy of statins[1] in preventing progression of atherosclerosis in children with Systemic Lupus Erythematosus (SLE or lupus).			SP – 4.1 --- HP – 12 --- D – 107
5c Expand the range of available methods used to create, analyze, and utilize chemical libraries, which can be used to discover new medicines. Specifically, use these chemical libraries to discover 10 new and unique chemical structures that could serve as the starting point for new drugs.			SP – 4.1 --- FDA – 1 --- D – 108

[1] Statins are drugs that lower cholesterol in blood and decrease the risk for atherosclerosis and cardiovascular disease.

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
6a Identify the genes that control the risk for the development of age-related macular degeneration and glaucoma in humans.			SP – 4.1, 6.2 --- HP – 28 --- FDA – 5 --- D – 109
6b By 2011, assess the efficacy of at least three new treatment strategies for reducing cardiovascular morbidity/ mortality in patients with type 2 diabetes and/or chronic kidney disease.			SP – 4.1 --- HP – 4, 5, 12 --- D – 110
6c By 2012, develop a knowledge base on Chemical Effects in Biological Systems using a "systems toxicology" or toxicogenomics approach.			SP – 1.1, 4.1 --- HP – 8 --- CDC – 3 --- FDA – 3, 5 --- D – 112
7a By 2005, evaluate 10 commonly used botanicals for inhibition/induction of enzymes that metabolize drugs as a method of identifying potential botanical/drug interactions.			SP – 4.1, 5.1 --- D – 113
7b By 2006, integrate nanotechnology-based components into a system capable of detecting specific biomarker(s) (molecular signatures) to establish proof of concept for a new approach to the early detection of cancer and, ultimately, cancer preemption.			SP – 4.1 --- HP – 3 --- D – 114

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
7c By 2005, create the next generation map of the human genome, a so called "haplotype map" (HapMap), by identifying the patterns of genetic variation across all human chromosomes.			SP – 4.1 --- FDA – 5 --- D – 115
8a By 2007, determine the genome sequence of an additional 45 human pathogens and three invertebrate vectors of infectious diseases.			SP – 1.2, 2.1, 4.1 --- HP – 14, 24, 25 --- CDC – 7, 9 --- FDA – 1, 5 --- D – 116
8b Identify and characterize two molecular interactions of potential clinical significance between bone-forming cells and components of bone. Such interactions are defined as those having significant impact on the accrual of bone mass or the actual mechanical performance of bone (i.e., fracture resistance) in laboratory animals.			SP – 4.1, 6.2 --- HP – 2 --- D – 117

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
8c Build a publicly accessible Collection of Reference Sequences to serve as the basis for medical, functional, and diversity studies. A comprehensive Reference Sequence Collection will serve as a foundation for genomic research by providing a centralized, integrated, non-redundant set of sequences, including genomic DNA, transcript (RNA), and proteome (protein product) sequences, integrated with other vital information for all major research organisms.			SP – 4.1 ---------- D – 118
9a By 2009, assess the impact of two major Institutional Development Award (IdeA) programs on the development of competitive investigators and their capacities to compete for NIH research funding.			SP – 4.3 ---------- D – 119
9b By 2010, demonstrate through research a capacity to reduce the total years lost to disability (YLDs) in the U.S. by 10 percent by 1) developing treatment algorithms to improve the management of treatment-resistant and recurrent depression and 2) elucidating the mechanisms by which depression influences at least two comorbid physical illnesses (e.g., heart disease, cancer, Parkinson's disease, or diabetes). Major depression is now the leading cause of YLDs in the nation.			SP – 4.1, 6.2 ---------- HP – 18 ---------- D – 120

RESEARCH OUTCOMES			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
9c By FY 2010, identify culturally appropriate, effective stroke prevention programs for nationwide implementation in minority communities.			SP – 1.1, 3.4, 4.1, 4.4 --- HP – 7, 12 --- CDC – 1, 2, 8 --- D – 122

COMMUNICATION OF RESULTS			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
a) Increase awareness of NIH-sponsored research among health care providers to promote research application.	**FY 2004** Disseminate resources to assist hearing health professionals, such as nurses, audiologists, pediatricians, and family practice doctors, in all 50 states in following up on early identification of hearing loss.	Performance will be reported in February 2005.	SP – 1, 2, 3, 4, 5, 6 --- HP – 11 --- D – 126
	FY 2003 Collaborate with the National Committee for Quality Assurance to foster implementation of cholesterol clinical practice guidelines.	Performance will be reported in February 2004.	
	FY 2002 Develop a communications campaign to build support and enhance the recruitment for domestic and international HIV/AIDS vaccine trials.	**Target met.** The HIV vaccine trials campaign sponsored lectures, workshops and exhibits at seven key professional and scientific conferences in FY 2002, and conducted an advertising campaign targeting professionals in minority communities.	
	FY 2001 1. Use continuing medical education programs based on the web-based Asthma Management Model System to disseminate and encourage the use of clinical practice guidelines on asthma. 2. Complete web accessibility for viewing nursing education programs and establish a once-a-month chat room where program directors will be available to answer questions from nursing students and nurse researchers.	1. As of the end of FY 2001, about 800 physicians had accessed the module on NHLBI's website and completed the course for full Category I credit. Many more physicians had visited the CME site. 2. NINR established a chat room for participants in the NINR Summer Genetics Program to enhance information about the application of genetic advances to nursing. The chat room was averaging 70 messages per month at the end of FY 2001.	

[1] —Indicates that the goal is part of the President's Management Agenda
SP-#—Indicates the DHHS Strategic Plan goal to which each GPRA goal pertains
HP-#—Indicates the Chapter of "Healthy People 2010" to which each goal pertains
CDC-#--Indicates a related goal in the CDC GPRA plan. See Appendix I.
D-#—Indicates the page in this report at which details on the goal can be found

COMMUNICATION OF RESULTS			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	3. Increase awareness of NIDA-sponsored clinical treatment among health care providers.	3. Seven research-based drug abuse treatment protocols were tested in real-world treatment settings by the network's six pioneering regional research centers in collaboration with more than 35 community treatment programs.	
	4. Use a partnership with the American Academy of Family Physicians to increase the knowledge of primary care physicians about the diagnosis and treatment of mental disorders.	4. NIH engaged in partnership with the AAFP and helped them develop core elements of a training program to include video and online CME programs and case studies; lectures; a quality improvement module for improving care for major depressive disorders; an AAFP monograph "Diagnosis and Management of Depression," mailed to 88,232 AAFP members; a series of mental health patient education handouts on mental health topics; and articles and reports published throughout the year on mental health issues in the AAFP journal.	
	FY 2000 1. Disseminate and encourage the use of clinical practice guidelines for the treatment of high blood pressure, high blood cholesterol, and other conditions by physicians who provide care to African-American patients.	1. Model cases studies on 11 aspects of treatment for cardiovascular disease were developed. Physicians who treat African-American patients have reached more than 600,000 other health professionals, patients, and members of the public with guidelines-based messages.	
	2. Use telehealth technology and TV cable networks for education projects with nursing organizations and academic institutions: broadcast select conferences and workshops to nursing organizations and academic institutions and add website components that will allow users to interact on-line with live discussions, conferences, and other types of meetings.	2. Enhanced electronic outreach, on-line access to educational conferences and a National Institute of Nursing Research (NINR) listserve were all created by the end of FY 2001. NINR also collaborated with other organizations to develop a videotape series on End of Life Care.	

COMMUNICATION OF RESULTS			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	3. Expand the "Not Just Once, But for A Lifetime" mammography campaign to reach health professional organizations, physicians, nurses, and other health and medical practitioners to increase awareness of the importance of mammography screening and the Medicare mammography benefit, and referrals for women, particularly those aged 65 and older. 4. Fund a series of demonstration projects applying telemedicine and other technology to improve the speed of reaching heart attack victims with lifesaving treatment.	3. NCI expanded the Breast Cancer and Mammography Education program by the end of FY 2001 by conducting qualitative research to develop and refine health education materials for consumers, emphasizing women 65 years and older, and health professionals. Research conducted with physicians was incorporated into a mammography campaign to reach health professionals. 4. Ten project awards were made.	
	FY 1999 1. Evaluate the use of clinical practice guidelines on the treatment of high blood pressure and high blood cholesterol by physicians who provide care to African-American patients. 2. Evaluate use of clinical practice guidelines on high blood pressure and obesity.	1. The results of the evaluation are described in FY 2000 Target 1 above. 2. Focus groups to evaluate whether physicians were using the guidelines were completed in FY 2000.	
b) Increase awareness of NIH-sponsored research results among racial/ethnic minorities and high risk, underserved, and/or affected publics.	**FY 2004** 1. Evaluate effectiveness of summit meetings as a means of extending "Back To Sleep" campaign to African American populations. 2. In collaboration with American Indian and Alaska Native leaders, draft, pilot test, and publish culturally appropriate SIDS risk reduction materials to disseminate widely in American Indian and Alaska Native communities 3. Use WISE EARS! evaluation to conduct outreach activities for targeted audiences, including African American, Hispanic/Latino/Latina individuals and Native American youth and their parents.	Performance will be reported in February 2005.	SP – 1, 3, 4 --- HP – 7, 11, 16 --- CDC – 8 --- D - 129
	FY 2003 1. In collaboration with African American organizations, community health and other local officials, and faith-based organizations, conduct regional summit meetings to train and motivate individuals who will implement SIDS risk reduction activities in their communities. 2. Implement a campaign to provide information on noise-induced hearing loss.	Performance will be reported in February 2004.	

COMMUNICATION OF RESULTS			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	3. Use a variety of media approaches (TV and radio news inserts, etc.) to communicate the importance of eating 5 fruits and vegetables a day.		
	FY 2002 1. Develop and disseminate Asian language materials communicating the benefits of mammography.	1. **Target met early in FY03**. Breast screening brochures in English and three Asian languages were developed and distributed in October 2002.	
	2. Provide support and technical assistance to NHLBI's Enhanced Dissemination and Utilization Centers to conduct heart-health education projects in high-risk communities.	2. **Target met.** NHLBI provided teleconference and extranet support, advised on media strategies, produced videos, and provided numerous publications in support of a network of community-based centers that conduct heart-health education projects.	
	3. Develop messages and materials to communicate the health implications of obesity.	3. **Target met.** NIDDK produced three publications to help Hispanic Americans reduce their incidence of obesity and diabetes through healthy eating and physical activity practices.	
	4. Develop and implement an eye health awareness campaign. *Also see FY 2001 target #6, which has been extended to FY 2002.*	4. **Target extended to FY 2003**. NEI worked with Hispanic leaders and communities to plan an eye health awareness campaign. They developed campaign messages and strategies, tested them in three market sites, and are using the results to refine the campaign, scheduled for launch in FY 2003.	
	FY 2001 1. Increase awareness of autoimmune diseases (such as rheumatoid arthritis, lupus, and scleroderma) among minority populations who are disproportionately affected.	1. NIAMS's Community Health Center was opened to screen and educate patients from minority populations; distribution of materials in English and Spanish was begun for these populations; and NIH participated in national and local events that reached minority patients and the health professionals who serve them.	
	2. Increase understanding about the seriousness of diabetes and the importance of blood glucose control among African Americans, Asian/Pacific Islanders, and American Indians.	2. Increased awareness was accomplished through 5 major National Diabetes Education Program awareness campaigns.	

COMMUNICATION OF RESULTS			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	3. Increase awareness about how to prevent fully preventable noise-induced hearing loss (WISE EARS! campaign) among industrial workers, Hispanic/Latino/Latina individuals, and Native American youth and their parents.	3. Examples of activities for each of the groups included collaborations with the Indian Health Service, Administration for Children and Families, Bureau of Indian Affairs/Department of Interior, and the Department of Education, and participation in conferences, meetings, and exhibits sponsored by the National Hispanic Medical Association and the National Hispanic Heritage Association.	
	4. Increase knowledge among Hispanic parents of the effects of drugs on the brain and encourage them to talk with their children about drug abuse.	4. NIH distributed 140,000 booklets via Hispanic organizations, clinics and Spanish radio public service announcements. NIH also translated a series of fact sheets; four NIDA research reports; and NIDA's popular books on marijuana, preventing drug abuse among children, and treatment principles. Spots for NIDA's public awareness campaign, "Keep your brain healthy. Don't use drugs", were aired on Spanish radio and television stations.	
	5. Increase awareness of the effects of drug abuse among Native American Indians.	5. NIH distributed 150,000 calendars with drug messages to this population via Native American organizations and businesses.	
	6. Develop and implement an HIV/AIDS vaccine communication campaign to increase awareness of HIV/AIDS vaccines before the initiation of a large efficacy trial.	6. The HIV/AIDS vaccine communications campaign was launched in May 2002.	
	7. Increase awareness of sports injury prevention among parents.	7. NIAMS developed, distributed, and promoted a fact sheet about sports injury prevention entitled, *Childhood Sports Injuries and Their Prevention: A Guide for Parents with Ideas for Kids*, to 128 newspapers in 10 states and to more than 2,700 YMCA's and YWCA's.	

COMMUNICATION OF RESULTS			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	FY 2000 1. As an activity of the NIH Hispanic Communications Initiative (HCI), conduct a Spanish-language "media summit" that will detail strategies for developing continuous and sustainable working partnerships between NIH information offices, national Spanish-language media outlets, and national Hispanic intermediary organizations. 2. Develop and disseminate easy-to-read and Spanish language health education materials on health issues to targeted special populations. 3. Expand programs on anxiety disorders and depression to audiences for whom language or literacy are challenges. 4. Pursue new outreach and collaboration initiatives to disseminate information and resources on rheumatic diseases in minority populations 5. Develop and implement diabetes awareness campaigns that target minority populations and their health care providers. 6. Develop and disseminate motivational messages related to breast and cervical screening to African American, Hispanic, and Asian communities. 7. Increase the available information on the benefits of exercise to older people.	1. The media summit was held in the first quarter of FY 2001. 2. Several NIH Institutes developed and disseminated easy to read and Spanish language materials on various diseases and other health topics. 3. A series of five easy to understand booklets on anxiety disorder were developed. Similar materials for depression and bipolar disorder are under development. 4. An African American Community Partnership was launched. 5. Seven diabetes awareness campaigns targeting minority populations and seniors were developed and implemented. 6. Motivational messages on these topics were disseminated to these communities. 7. Information about the benefit of exercise was made available through the news media, health fairs, and professional meetings.	
	FY 1999 1. Establish a centralized site on the NIH home page for access to NIH materials in Spanish. 2. Evaluate several selected NIH outreach programs: cardiovascular health outreach activities for Latinos. 3. Extend the "Back to Sleep" campaign to minority populations.	1. A centralized site was established and launched. 2. An evaluation of the community based outreach initiative "Salud para su Corazon" was completed. 3. An educational video, outreach to day care facilities, and creation of a coalition of African American organizations to promote back sleeping by infants were completed.	

COMMUNICATION OF RESULTS			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	4. Develop and implement NIH information, education, and outreach programs on specific health issues: Breast Cancer and Mammography Education Program.	4. NCI continued to implement the Breast Cancer and Mammography Education program.	
c) Increase awareness of NIH-sponsored research results among the general public.	**FY 2004** 1. Extend the impact of the *Know Stroke: Know the Signs. Act in Time* campaign to increase awareness among the general public about the symptoms of stroke and the need to seek treatment rapidly by partnering with providers and volunteers in at least five communities to circulate materials and hold educational events. 2. Develop and implement a campaign for parents/guardians on the importance of early identification and treatment of hearing loss.	Performance will be reported in February 2005.	 SP – 1, 3, 4 --- HP- 11 --- CDC – 2 --- D - 133
	FY 2003 Launch three new services to enhance the online health information resource, MEDLINEplus.	Performance will be reported in February 2004.	
	FY 2002 1. Introduce an easily navigable site on the World Wide Web that can increase older adults' awareness of health information and, based on the National Institute on Aging-supported cognitive research findings, enhance the online learning experience for people age 60 and over. 2. Perform a process evaluation of the effectiveness of pap test information materials produced in FY 2001. 3. Develop campaign materials about the importance of calcium from milk and other sources for strong bones.	1. **Target was extended to FY 2003.** Information for seniors on three health topics were made available on NLM's website in FY 2002, but the full launch covering 10 topics will be accomplished in FY 2003. 2. **Target met**. *Cervical Cancer Evaluation Support: Final Report* - Process Evaluation of Health Professional Pap Test Packet, Press Release, and PSA; March 2002). 3. **Target met.** NICHD added information for children and parents to the Milk Matters website http://www.nichd.nih.gov/milk/ on the importance of calcium for bone health. (See FY01 target #5.)	

COMMUNICATION OF RESULTS			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	4. Implement a stroke awareness campaign. *Also see FY 2001 targets #3 and #5, which have been extended to FY 2002.*	4. **Target met.** In FY 2002 NIH continued to implement the public education campaign launched in May 2001, *Know Stroke. Know the Signs. Act in Time.* For Stroke Awareness Month in May 2002, NIH initiated a partnership with the American Stroke Association. Also, NIH placed television public service messages in markets reaching 2.1 million viewers and stroke experts conducted radio interviews. *(Also see FY01 target #6.)*	
	FY 2001 1. Strengthen relationships with constituency groups nationwide to increase awareness of the latest scientific information about drug abuse and addiction prevention and treatment and to provide a channel for feedback about emerging grassroots issues.	1. NIDA conducted continual outreach with its core constituents around the country. It held biannual meetings and sent out newsletters and other mailings to keep in touch and promote communication between the organizations and NIDA.	
	2. Improve the public's access to health information by expanding the NLM's consumer health information program to ensure that a medical library in every state is working with public libraries and community organizations.	2. By September 30, 2001 there was at least one NLM-supported medical library in every state and Puerto Rico working with public libraries and other community organizations, including faith-based organizations.	
	3. Increase awareness among university presidents, program planners, and policy makers about college drinking and related problems.	3. The National Institute on Alcohol Abuse and Alcoholism (NIAAA) Task Force on College Drinking released several publications on the effectiveness of current alcohol prevention strategies and recommendations on future research to improve college drinking prevention programs, including a report titled, *A Call to Action: Changing the Culture of Drinking at U.S.* Colleges. The report and accompanying materials have been sent to all college and university -presidents in the United States.	

COMMUNICATION OF RESULTS			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	4. Increase awareness among the general public about the achievements of publicly-funded vision research.	4. The National Eye Institute (NEI) completed a tour of VISION, a traveling exhibit that highlighted the sight-saving results of vision research. The exhibit was displayed in science museums in 26 metropolitan areas with more than 5.3 million people visiting the exhibit through July 2001.	
	5. Increase awareness among young people of the importance of calcium in their diet.	5. NICHD launched the new Milk Matters website for young children and teens on December 1, 2001.	
	6. Increase the number of people who know the symptoms of stroke and rapidly seek treatment.	6. In May 2001, NIH launched "Know Stroke. Know the Signs. Act in Time," a consumer education campaign about recognizing the signs of stroke and acting quickly to get treatment.	
	7. Increase the public's understanding of cancer research, advances, and opportunities.	7. In efforts to increase public understanding of cancer research, advances and opportunities, the National Cancer Institute initiated a number of activities including distributing almost 2 million publications about breast cancer prevention to women and health professionals; almost 300,000 informational materials for cervical cancer prevention; and 1.1 million 5 A Day related publications to consumers.	
	8. Increase awareness among the general public that drug addiction is a brain disease.	8. NIH developed and distributed materials to its target audiences. The campaign combined public awareness materials, educational materials, speakers, information online, and a wide array of outreach to the general public**.**	
	FY 2000 1. Increase the usage of NLM's existing catalog-based databases for books, serials, and audiovisuals by 15 percent.	1. The level of usage increased by 27% over the FY 1999 level.	
	2. Increase the number of "health topics" in the web-based MEDLINEplus to 300.	2. The number of health topics in MEDLINE plus increased to 414.	
	3. Ensure that no less than 85 percent of respondents to a customer feedback instrument rate NLM services at least satisfactory.	3. A survey showed that 98% of users rated NLM services as satisfactory or better.	

COMMUNICATION OF RESULTS			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	4. Increase collaboration with professional associations of journalists, science writers, and health communicators to increase their coverage of NIH-funded research results.	4. NIH collaborated with these organizations to increase the likelihood that medical research findings will be reported in the media or through health communication programs.	
	5. Implement "WISE EARS!" communications program by developing a coalition of more than 70 groups representing government, industry, the worker, children and older individuals as well as organizations directly committed to preventing noise-induced hearing loss and providing them with resources in order to reach children under 17, adults in mid-life, and older Americans as a means to provide information about how to prevent fully preventable noise-induced hearing loss with messages in at least 50% of states by 2001.	5. NIH developed a coalition of 78 organizations.	
	6. Expand the outreach of the "Milk Matters" campaign beyond parents and health professionals to focus directly on activities and products that help children and teens recognize the benefit of calcium in building strong bones.	6. New educational materials were developed, strategic alliances formed with professional organizations, and materials distributed to peer and opinion leaders.	
	7. Generate a minimum of 30 million media impressions through placements in newspapers and magazines nationwide and on national and local television and radio programs, to raise awareness among all Americans of the importance of eating at least 5 servings of fruit and vegetables a day.	7. Mass media activities to raise awareness generated more than 30 million media impressions.	
	FY 1999 1. Implement a system to track customer service interactions, measure response times, and record customer feedback on NLM products and services.	1. NLM installed software and a program that tracks inquiries, measures response times, and records customer feedback on its services.	
	2. Provide a single toll-free telephone number to reach NLM customer service staff.	2. The toll-free telephone line was established.	
	3. Complete the restructuring of NIMH's mental health education and information dissemination programs.	3. NIMH developed a new mission statement for the Institute's communications programs. A new Associate Director position was established and filled.	
	4. Increase the availability of consumer health information, publications, and reports under NIH's Centralized Consumer Health Information area by 20%.	4. The number of on-line publications increased approximately 76%.	

COMMUNICATION OF RESULTS			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	5. Strengthen relationships with universities, voluntary health associations, and other organizations that communicate health and scientific information--to expand the options for communicating NIH research results. 6. Develop and implement NIH information, education, and outreach programs on specific health issues: Low Vision.	5. NIDDK established relationships with 375 public and private organizations to support the National Diabetes Education Program. 6. NIH launched the Low Vision program.	
d) Increase awareness of clinical research and support participation in clinical trials.	**FY 2003** Develop messages and materials about participating in clinical studies.	Performance will be reported in February 2004.	SP – 3, 4, --- HP – 11 --- D - 138
	FY 2002 In partnership with community-based organizations, develop rheumatic disease health education materials and increase awareness of opportunities to participate in clinical studies.	**Target met.** Fourteen educational publications on rheumatic diseases, some with specific messages about clinical trials, were produced over FY01-02. A NIAMS-funded community health center in Washington, D.C. recruited more than 300 patients from minority communities to NIH clinical studies in its first 14 months of operation. NIAMS distributed rheumatic disease educational materials at 32 local community events during that time.	
	FY 2001 1. Develop web-based clinical trials tools that will improve the development, conduct, and ease of participation in NCI-sponsored clinical trials. 2. Improve NCI efforts to increase participation and retain minorities, underserved populations, and the elderly in clinical trials.	1. NCI developed a means to dynamically generate a list of trials that can appear on web pages about new drugs and treatments. NCI developed web-based informatics "products" to improve the reporting and exchange of clinical trials information. 2. NIH created the Special Populations Network (SPN), a new $60 million program to address the unequal burden of cancer within certain special population groups.	

COMMUNICATION OF RESULTS			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	3. Educate the public about the importance of NIMH-supported clinical research and interest individuals and their families in participating in clinical studies. 4. Increase the number of initial contacts about clinical trials with the Patient Recruitment and Public Liaison Office (PRPL).	3. NIMH developed a communications plan to increase awareness of and participation in NIMH intramural and extramural clinical trials. New materials (both web and print) and outreach activities were developed for select trials using target audience research (e.g., focus group testing), literature reviews, and consultation with investigators. NIMH sponsored a workshop and created a handbook to educate investigators on how to recruit locally using the media, the web, advertisements, and community groups. 4. The Patient Recruitment and Public Liaison Office at NIH received 31,251 contacts in FY00 and 42,863 in FY01, an increase of 37%.	
	FY 2000 1. Build and maintain networks of communication and support for clinical research between NIMH and consumer and advocacy organizations and professional groups nationally, regionally, and locally. 2. Develop methods and materials to improve communication with minorities and ethnic groups and to encourage them to participate in (NIMH-sponsored) clinical research, thus meeting a critical public health need. 3. Increase visitors to NCI's cancer trials website and the amount of information about cancer trials to patients, health professionals, the public and the media in all areas including prevention, detection, diagnosis, and treatment. 4. Develop simplified and easy-to-understand informed consent forms to help patients better understand a study's treatments and tests and their possible benefits and risks before deciding whether or not to participate.	1. NIH initiated the Constituency Outreach and Education Program (COEP). Networks were established with 51 partners in FY 2001. 2. Formative research, including focus group testing, was used to gather information on minority participation in NIMH clinical research in FY 2001. Media activities to increase enrollment included a focus on minority audiences. 3. The total number of users expanded by 33% and the range of information available on the website was considerably enlarged. 4. NCI developed a simplified and easy to understand informed consent form.	
	FY 1999 Initiate a broad-based communications and public outreach program to reach physicians, and eventually, community groups and the general public.	A broad-based communications and public outreach program was initiated.	

COMMUNICATION OF RESULTS			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
e) Establish a Clinical Trials Database, as required by the FDA Modernization Act. (Retained only for reporting purposes. This goal was met in FY 2002.)	**FY 2001** 1. Complete an implementation study to determine the optimal design and function of a toll-free telephone to facilitate access to the Clinical Trials Database. 2. Expand the number of industry-sponsored clinical trials in the database by 250. 3. Expand the number of clinical trials in the database sponsored by other Federal agencies by 100. 4. Promote the database as a resource for patients, physicians, researchers, community health groups and others.	1. NIH completed the implementation study in March 2001. 2. The number of industry sponsored clinical trials increased by 109 in FY 2001. NIH worked closely with the FDA to develop guidance for companies to submit their clinical trial information to ClinicalTrials.gov and that guidance was issued in March 2002. By the close of FY 2002, the number of industry sponsored clinical trials stood at over 1200. 3. The number sponsored by Federal agencies tripled from 104 in FY 2000 to 383 in FY 2001. 4. NIH promoted the ClinicalTrials.gov database as a resource for patients, physicians, researchers, community health groups and others. The site received about 2 million hits per month and hosted approximately 5,300 visitors daily in FY 2001.	D - 141
	FY 2000 1. Expand the Clinical Trials Database to include trials from other Federal agencies and the private sector. 2. Develop options for implementation of toll-free telephone access to information in the Clinical Trials Database.	1. At the end of FY 2000, 800 of the more than 5,000 trials in the database were supported by the private sector or other Federal agencies. 2. The final report was completed in 2001.	
	FY 1999 Develop and implement the Clinical Trials Database.	Data elements were developed for the database in FY 1999 based on the legislative requirements and discussions with collaborating NIH Institutes and other groups.	

TECHNOLOGY TRANSFER			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference**[1]
a) Increase the number of scientists who have received training in technology transfer.	**FY 2004** Seek to have the web-based training module incorporated as a standard requirement for all new scientists at NIH.	During FY 2002, NIH also met the FY 2004 target of making completion of the NIH On-Line Technology Transfer Training an ongoing requirement for employees. With completion of all targets, this goal has been met and will be dropped from future GPRA plans.	SP – 4 D - 144
	FY 2003 1,000 scientists complete the web-based training module.	During FY 2002, 1231 scientists were trained using the NIH On-Line Technology Transfer Training module.	
	FY 2002 200 scientists complete the web-based training module.	During FY 2002, 1231 scientists were trained using the NIH On-Line Technology Transfer Training module. As a result, NIH surpassed its training targets for both FY 2002 and FY 2003. Furthermore, NIH also met the FY 2004 target of making completion of the NIH On-Line Technology Transfer Training an ongoing requirement for employees. With completion of all targets, this goal has been met and will be dropped from future GPRA plans.	
	FY 2001 Seek to have 15% of scientists complete the training module, and/or attend technology transfer seminars.	Approximately 62% of the scientists attended technology transfer seminars and meetings.	
	FY 2000 1. Implement training module. 2. Contact 20% of NIH scientific staff.	1. The training module was activated on the web in the first quarter of FY 2001. 2. By the end of FY 2001, over 60% (2450 of the over 4000 members) of the scientific staff were contacted.	

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TECHNOLOGY TRANSFER			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	FY 1999 Contractor development of a web-based training module.	The training model was completed in FY 2000.	
b) Develop a system to identify and measure the health outcomes of technologies licensed by NIH.	**FY 2004** Review and modify the methodology as needed and continue to apply the metrics to at least 10% of all exclusively licensed technologies that are a part of commercially available products.	Performance will be reported in February 2005.	D - 146
	FY 2003 Finalize the approach and apply the methodology to 10% of all exclusively licensed technologies that are a part of commercially available products.	Performance will be reported in February 2004.	
	FY 2002 Develop two case studies to test the proposed methodology. *Also see FY 2001 target #1, which has been extended to FY 2004.*	The NIH Health Outcomes Working Group completed two pilot case studies analyzing the public health outcomes of 2 commercially available products incorporating exclusively licensed NIH technologies. The reports are located on the NIH Office of Technology Transfer website (ott.od.nih.gov).	
	FY 2001 Establish a working group in the Office of Technology Transfer and obtain recommendations on potential outcome measures and sources for obtaining reliable data for those measurements on therapeutic drugs, vaccines and devices.	The NIH Health Outcomes Working Group was established in FY 2001 to work on the development of health outcomes measures. During FY 2002 meetings, the group recommended potential outcome measures and suggested reliable sources of data. The group also selected two products to utilize as case studies.	
c) Maintain oversight and protection of the public investment in NIH research through increased monitoring of licensee activities.	**FY 2002** 1. Implement a data system that includes all license monitoring milestones and benchmarks for all exclusive licenses. 2. Reduce delinquencies over 180 days and bring that number to zero by the end of FY 2002, except for cases that are being actively negotiated due to the affect on public health.	1. NIH established a data system for monitoring license milestones and benchmarks. 2. NIH reduced the number of delinquencies over 180 days to zero.	D - 148

TECHNOLOGY TRANSFER			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
(Retained only for reporting purposes. This goal is part of the normal technology transfer process and will not be continued past FY 2002.)	3. Ensure that all delinquent payments associated with terminated licenses will be submitted to the NIH Debt Collection Officer within 120 days of termination. 4. Perform audits on up to 3 licensees during the year, if warranted. *Also see FY 2001 target #1, which has been extended to FY 2002.*	3. NIH reported all delinquencies for debt collection. 4. NIH conducted two audits found to be warranted during FY 2002.	
	FY 2001 1. Recruit and select personnel to establish a special license-monitoring unit to provide oversight of licensee progress in developing and commercializing technologies licensed from NIH.	1. Following a long recruitment effort that was initially unsuccessful, two Technology License Monitoring and Enforcement Specialists were selected to oversee the progress of licensees towards commercializing technologies licensed from NIH.	
	2. When indicators show that sales and royalty information may be incorrect, conduct audits of up to 3 licensees during the year.	2. An outside group, hired by NIH, audited five licenses.	
	3. Reduce the number of delinquent payments over 180 days by 50% from the number in place at the end of FY 2000.	3. The number of delinquent payments over 180 days was reduced by 55% from the number in place at the end of FY 2000.	
	4. Reduce the number of terminated licensees with outstanding royalty amounts owed by 10% from the number at the end of FY 2000. 5. Develop and implement a process to refer infringers of NIH intellectual property rights to the Department of Justice with recommendations for bringing a Federal law suit against such entities.	4. NIH exceeded its target by reducing the number of terminated licenses with outstanding balances by 43%. 5. A process for referring infringers of NIH intellectual property rights to the Department of Justice was developed and implemented. One of three cases initiated in FY 2001 was settled.	

GRANTS ADMINISTRATION AND PEER REVIEW			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference[1]**
a) Improve electronic Research Administration (eRA) technology by developing capability for end-to-end electronic research administration by 2004.	**FY 2004** Pilot-test electronic receipt of simple (non-clinical, non-human) competing R01 applications.	Performance will be reported in February 2005.	 SP – 8 D - 153
	FY 2003 Expand availability of electronic progress reporting to all grantee institutions.	Performance will be reported in February 2004.	
	FY 2002 1. Release NIH Commons modules in the new architecture. 2. Scan all incoming competing R01 applications in preparation for pilot testing receipt of R01 applications in 2003. *Also see FY 2000 targets #2 and #3,which have been extended to FY 2002.*	1. NIH Commons modules were deployed in the new architecture in August 2002. 2. All incoming applications are being scanned and made available electronically to NIH staff, as of January 2002.	
	FY 2000 1. Full deployment of key eRA business process modules. 2. Implement electronic progress reporting with all 65 newly on-line institutions participating in the Federal Demonstration Partnership. 3. Begin pilot testing of progress reporting for multi-project mechanisms.	1. Key business process models in NIH Commons were made widely available. 2. Pilot testing of the beta version of the e-SNAP module was completed. Recommendations from the pilot are being incorporated into the release of the new version of e-SNAP. NIH expects the electronic progress reporting system to open to the 65 FDP institutions in early 2003. 3. Electronic research administration projects have been reprioritized to align the NIH focus more closely with the Federal e-Grant emphasis on the competitive application process. This redirection necessitated a reallocation of resources. Accordingly, development of a system to accommodate progress reporting for multi-project mechanisms has been postponed until 2005.	
	FY 1999 1. Design and test new systems.	1. The Electronic Notice of Grant Award (NGA) system was pilot tested in FY 1998 and fully deployed in FY 1999.	

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GRANTS ADMINISTRATION AND PEER REVIEW			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference[1]**
	2. Streamline post-award reporting while continuing to ensure appropriate oversight and enhancement of recipients' compliance with reporting and accountability requirements.	2. E-SNAP began receiving the first electronic applications in a limited pilot.	
b) Ensure proper stewardship of public funding for research.	**FY 2004** 1. Conduct 5 proactive compliance site visits. 2. Begin internal compliance reviews.	Performance will be reported in February 2005.	SP – 8 D - 156
	FY 2003 1. Conduct 5 proactive compliance site visits. 2. Perform a risk assessment and develop a plan for reviews of compliance with grant related policies. 3. Provide Internet-accessible resource information and/or tools for implementing institutional compliance programs.	Performance will be reported in February 2004.	
	FY 2002 1. Conduct 8 proactive compliance site visits to grant recipient research institutions. 2. Publish a compendium of observations and examples of compliance in action in the conduct and administration of sponsored programs at NIH's recipient institutions.	1. NIH completed 8 proactive compliance site visits in FY 2001. 2. The compendium was completed and posted on the grants Compliance and Oversight web pages at http://grants1.nih.gov/grants/compliance/compendium_2002.htm.	
	FY 2001 1. Create an organizational component within NIH with FTEs devoted expressly to compliance-related activities. 2. Perform a minimum of 10 compliance site visits.	1. In the last quarter of FY 2001 the Division of Grants Compliance and Oversight was established within the Office of Policy for Extramural Research Administration, Office of Extramural Research, NIH. 2. Eight proactive compliance site visits were completed.	
c) Ensure that the NIH peer review process keeps pace with current advances in research and that the expertise of peer reviewers is appropriate for the needs of modern science.	**FY 2004** Present the recommendations of at least three Integrated Review Group Study Section Boundary (IRG SSB) Teams to the CSR Advisory Committee each year, and subsequently develop implementation plans for reorganizing each of those IRGs.	Performance will be reported in February 2005.	SP – 4 D - 158

GRANTS ADMINISTRATION AND PEER REVIEW			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference[1]**
	FY 2003 1. Present the recommendations of at least three IRG SSB Teams to the CSR Advisory Committee each year, and subsequently develop implementation plans for reorganizing each of those IRGs. 2. Complete formation of all Steering Committees and SSB Teams.	Performance will be reported in February 2004.	
	FY 2002 1. Increase number of Steering Committees and SSB Teams to 10. 2. Complete the formation of all external IRG Advisory Groups.	1. NIH exceeded the target by increasing the number of Steering Committees and their respective SSB Teams to 12. 2. Enhancements of study section operations were completed ahead of schedule, with the last 5 IRG Advisory Groups being formed in FY 2002. Reports from all 19 Advisory Groups, which are working groups of the CSR Advisory Committee, were developed and a summary was presented to the CSR Advisory Committee in January 2002.	
	FY 2001 1. Create 4 Steering Committees and their respective SSB teams. 2. Increase the number of external IRG Advisory Groups to 14.	1. A total of 7 Steering Committees and their respective SSB teams were created. 2. A total of 19 external IRG advisory groups were created.	
	FY 2000 1. Complete Phase 1 of the PSBR and develop an implementation plan for Phase 2. 2. Double the number of external IRG Advisory Groups from 3 to 6.	1. Phase 1 final report was completed and Phase 2 implementation was initiated. 2. Ten external advisory groups were formed.	
	FY 1999 Conduct various assessments of the functions and organization of NIH study sections.	The assessments were completed.	
d) Develop innovative business practices to facilitate government/public interactions.	**FY 2004** Pilot test opportunities for reengineering and streamlining application requirements for the PHS 398.	Performance will be reported in February 2005.	SP – 8 D - 161
	FY 2003 1. Evaluate the results of the simplified Streamlined Noncompeting Award Process (SNAP) pilots and make recommendations.	Performance will be reported in February 2004.	

GRANTS ADMINISTRATION AND PEER REVIEW			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference[1]**
	2. Identify opportunities for reengineering and streamlining data requirements for NIH's competing application (PHS 398).		
	FY 2002 Pilot-test ways to further simplify NIH's SNAP.	Target extended to FY 2003. Simplification of the administrative processes required of grantees has been integrated into the new electronic progress reporting system. Deployment of this system is scheduled for early 2003.	
	FY 2001 Further facilitate expediting the processing of the most meritorious grant applications by reducing the receipt-to-award cycle from 9-10 months to 6-7 months.	En bloc Council concurrence was introduced in 13 Institutes. These Institutes made awards to the most meritorious Type 1 grant applications in approximately 6-8 months from application receipt.	
	FY 2000 Expedite the processing of the most meritorious grant applications by extending to all NIH Institutes the use of expedited en bloc Council review procedures.	A policy announcement encouraging adoption of, and providing guidance for, expedited procedures was prepared and disseminated.	
	FY 1999 Identify approaches to expedite the processing and award of grant applications.	A procedure called en bloc concurrence was established to significantly expedite the awards process.	
e) Improve grantee reporting of inventions developed with Federal funds.	**FY 2003** Deploy a redesigned Edison system to 350 grantee/contractor organizations.	Performance will be reported in February 2004.	SP – 8 D - 163
	FY 2002 Integrate Edison into the Federal Commons (a governmental electronic grants and contracts administration system).	The Federal Commons concept has evolved in the E-Grants initiative under OASAM. Invention reporting is not included in Phase I E-Grant activities. Integration will not occur for some years yet.	
	FY 2001 1. Identify ways to improve the quality of historical invention reporting data.	1. Additional full-time staff were added to allow for a more thorough analysis of historical records.	

GRANTS ADMINISTRATION AND PEER REVIEW			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference[1]**
	2. Further educate constituents of their invention reporting obligations.	2. NIH officials responsible for invention reporting participated in all proactive compliance site visits in FY 2001, and made formal presentations at numerous national meetings of academic technology transfer and grants administration professionals.	
	FY 2000 Fully establish the Edison system for use by all grantee institutions, and expand its use to other government agencies.	The Edison system was fully established and is capable of being used by all grantee institutions.	
	FY 1999 Enhance recipient compliance with reporting and accountability requirements.	189 grantee institutions were using Edison (40% increase over 1998 level). Memoranda of Understanding with several Federal agencies were in use.	

MANAGEMENT AND ADMINISTRATIVE SUPPORT			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
a) Improve the efficiency of the simplified acquisition process by continuing to expand the Purchase Card Program.	**FY 2004** 1. $215 million in orders. 2. $395,000 total orders/transactions.	Performance will be reported in February 2005.	 SP – 8 D - 167
	FY 2003 1. $212 million in orders. 2. 380,000 orders/transactions.	Performance will be reported in February 2004.	
	FY 2002 1. $210 million in orders. 2. 365,000 orders/transactions.	l. The dollar volume of purchase card orders achieved in FY 2002 was $225 million, which exceeds the targeted amount by $15 million. 2. The number of purchase card orders/transactions for FY 2002, was 385,000, which exceeds the targeted amount by 20,000.	
	FY 2001 $200 million in orders.	Dollar volume of purchase card orders was $196 million in FY 2001.	
	FY 2000 1. $160 million in orders.	1. $173 million in orders were made.	
	2. 3,600 people trained to use cards. 3. 2,000 card holders.	2. The total number of persons trained increased to 3,922 by the end of FY 2001. 3. Total number of purchase card holders reached 1,729 in FY 2000 and 1,866 card holders at the end of FY 2001.	
	FY 1999 1. $110 million in orders. 2. 3,000 people trained to use cards. 3. 1,600 card holders.	1. $130 million in orders were made. 2. The total number of persons trained increased to 2,860 and reached 3,391 in FY 2000. 3. The total number of card holders increased to 1,485 and reached 1,729 by FY 2000.	
b) Expand the use of Performance Based Contracting (PBC).	**FY 2004** Allocate $226.0 million of the available NIH contracting dollars to PBC-eligible contracts.	Performance will be reported in February 2005.	SP – 8 D – 170

[1] –Indicates that the goal is part of the President's Management Agenda
SP-#–Indicates the DHHS Strategic Plan goal to which each GPRA goal pertains
HP-#–Indicates the Chapter of "Healthy People 2010" to which each goal pertains
D-#–Indicates the page in this report at which details on the goal can be found

MANAGEMENT AND ADMINISTRATIVE SUPPORT			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	FY 2003 Allocate $226.0 million of the available NIH contracting dollars to PBC-eligible contracts.	Performance will be reported in February 2004.	
	FY 2002 Allocate $207.0 million of the available NIH contracting dollars to PBC-eligible contracts.	In FY 2002, NIH contracting offices obligated over $417 million toward performance-based contracts, an amount more than twice the FY 2002 target. The size of the increase reflects a major shift in the way NIH writes contracts and a commitment to the Administration's PBC goals.	
	FY 2001 Allocate $21.2 million of the available NIH contracting dollars to PBC-eligible contracts.	The dollars allocated to PBC contracts totaled $36.5 million.	
	FY 2000 Allocate $19.8 million of the available NIH contracting dollars to PBC-eligible contracts.	The dollars allocated to PBC contracts totaled $198.5 million.	
c) Identify and pilot new approaches to providing human resource services which increase manager satisfaction with personnel system flexibility and ease of use. (Retained only for reporting purposes. This goal will be discontinued after FY 2002.)	**FY 2002** Complete distribution of the final year management satisfaction survey, interviews, and collect and analyze data for the final report due in 2002.	Target met in FY 2002. Data was collected, analyzed, and reported.	D - 172
	FY 2000 A 10% increase in manager satisfaction with personnel system flexibility and ease of use as reflected in the 1999 survey outcome against the 1997 baseline.	The 1999 interim survey data showed a 6% increase in satisfaction. However, the final survey showed a 38% increase in satisfaction.	
	FY 1999 Complete the delegations of authority and related training.	Delegations completed and related training provided to managers.	
d) Recruit and retain highly qualified extramural investigators to biomedical/ behavioral research through the use of student loan repayment programs.	**FY 2004** 1. 25% of CR awardees are first time grant or other award recipients. 2. 50% of ECR applicants are in training or recently commenced their research careers. 3. 50% of HDR applicants are in training or recently commenced their research careers. 4. 25% of PR awardees are first time grant or other award recipients. 5. 75% of past participants conduct contraception and/or infertility research two years after completing the CIR.	Performance will be reported in February 2005.	SP – 8 D – 174

MANAGEMENT AND ADMINISTRATIVE SUPPORT			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
	FY 2003 1. 25% of CR awardees are first time grant or other award recipients. 2. 50% of ECR applicants are in training or recently commenced their research careers. 3. 50% of HDR applicants are in training or recently commenced their research careers. 4. 25% of PR awardees are first time grant or other award recipients. 5. 75% of past participants conduct contraception and/or infertility research two years after completing the CIR.	Performance will be reported in February 2004.	
	FY 2002 1. 50% of eligible sites use the CIR for recruitment. 2. 50% of applicants apply because of the opportunity to participate in the CIR. 3. 15% of CR awardees are first time grant or other award recipients. 4. 30% of ECR applicants are in training or have recently commenced their research careers. 5. 30% of HDR applicants are in training or recently commenced their research careers. 6. 15% of PR awardees are first time grant or other award recipients. 7. 50% of HDR awardees are from health disparities populations. 8. 50% of past participants conduct contraception and/or infertility research two years after completing the CIR.	All FY 2002 targets were met.	
e) Implement government-wide initiative on delayering management levels and streamlining organization.	**FY 2003** Complete delayering for each organizational unit identified.	Performance will be reported in February 2004.	SP – 8 D – 179
	FY 2002 1. Complete assessment of NIH organizational level structure and rationale for current patterns. 2. Identify organizational units for delayering. 3. Develop implementation plans to accomplish delayering for each organizational unit. 4. Develop specific numeric targets for the implementation plans.	All FY02 targets were met.	
f) Implement the NIH Business Research and	**FY 2004** 1. Deploy the Contracts / Acquisition / AP / Supply module.	Performance will be reported in February 2005.	

MANAGEMENT AND ADMINISTRATIVE SUPPORT			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
Support System.	2. Deploy the Service and Supply Fund Activities module.		SP – 8 D – 181
	FY 2003 1. Deploy the GL / Budgeting module. 2. Deploy the Property module. 3. Deploy the Travel module.	Performance will be reported in February 2004.	
	FY 2002 Implement EHRP.	In FY 2002, NIH implemented the Department's EHRP core software.	
g) Improve accountability for organizational performance results and support for the President's Management Agenda by linking the employee performance management plans/contracts to NIH's program and management priorities.	**FY 2004** *Additional targets to be determined*.	Performance will be reported in February 2005.	SP – 8.2 D – 183
	FY 2003 Incorporate outputs and outcome methodology in managers and supervisors' performance plans.	Performance will be reported in February 2004.	

MANAGEMENT AND ADMINISTRATIVE SUPPORT			
Performance Goal	**FY Targets**	**Actual Performance**	**Reference[1]**
h) Identify and develop potential successors for critical leadership positions by (1) developing and implementing a NIH-wide succession planning process that assesses the gaps between senior leadership needs and talent available; (2) identifying leadership competencies that will be critical to the mission of NIH now and into the future; and (3) providing developmental opportunities that will prepare our potential successors to meet the demands required of senior leadership positions.	**FY 2004** 1. Provide guidance and direction for NIH-wide succession planning efforts, including succession planning pilot (see targets below) 2. Develop automated tools to support succession-planning processes. 3. Incorporate competencies into Individual Development Plan (IDP) goals for target positions. 4. Identify potential successors for critical administrative positions and prepare IDPs to guide their development efforts,[1] as the first steps in preparing to conduct the succession planning pilot.	Performance will be reported in February 2005.	 SP – 8.2 D – 185
	FY 2003 1. Conduct study and report on average age, years of service, and retirement eligibility. Assess future potential impact. 2. Conduct study and report on current state. Assess strengths, weaknesses and needs for changes in current practices. 3. Establish steering/oversight committee. 4. Identify industry best practices. Develop a succession planning process to meet the needs of NIH. 5. Conduct study to identify competencies needed of NIH leaders that will drive future development efforts.	Performance will be reported in February 2004.	

[1] Once potential successors have been identified, an assessment of individual strengths will need to be made based on agreed upon leadership competencies. The NIH will work closely with DHHS and the new Corporate University to provide training and development opportunities to address areas for improvement (e.g. leadership courses, SES Candidate Development Program, etc.).

GPRA Research Program						
Budget (dollars in thousands)	**FY 1999 Actual**	**FY 2000 Actual**	**FY 2001 Actual**	**FY 2002 Actual**	**FY 2003 President's Budget**	**FY 2004 Request**
	$14,580,705	$16,692,719	$19,214,206	$21,883,989	$24,734,244	$26,426,569

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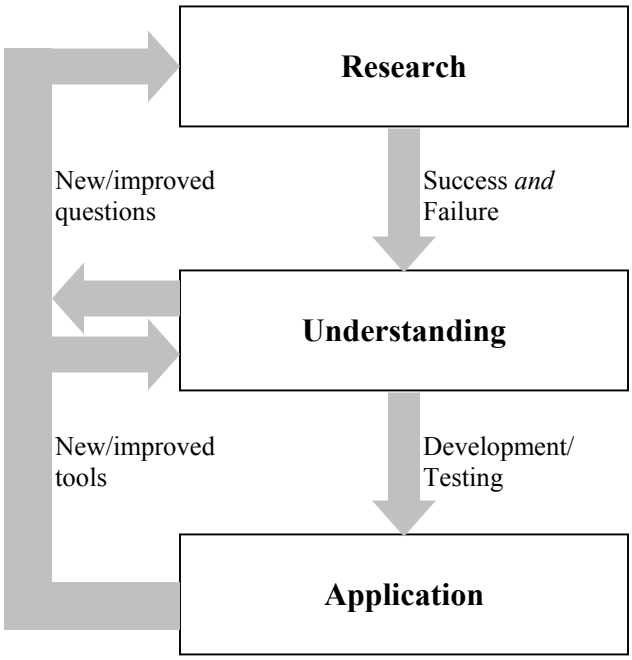
2.1.3 Program Performance Analysis

2.1.3.1 Research Outcomes

NIH's research Institutes and Centers (ICs) maintain extensive medical research programs on numerous topics in their areas of focus. In addition to providing grant support to the extramural research community through a competitive proposals process, most of the ICs also conduct their own research in NIH's intramural laboratories. Each year, NIH supports approximately 50,000 awards made to the most promising and productive scientists at universities and research centers throughout the country and, where special opportunities exist, from scientists abroad.[1]

The nation's investment in medical research has a long history of success. In recent years, NIH has been able to report annually on advances that represent outstanding achievements in science. Typically, these achievements are the result of past investments made with the belief that medical research will lead to improvements in the nation's health. The Federal effort devoted to medical research, combined with private sector efforts, can and does improve the length and quality of our lives.



The first set of research outcome goals that NIH presents and reports on in this document (Goals a – g) are being phased out. As indicated in earlier consolidated NIH GPRA plan/reports, NIH had intended to replace its comprehensive research outcome goals with subgoals. Now however, with this report, NIH is introducing 26 new goals that are far more specific and only sample NIH research aims in order to represent the agency's mission (see page 91). The original

[1] Includes all research, training, fellowship, R&D contracts, and SBIR/STTR.

comprehensive goals appear in this document for FY 2002 reporting purposes, but will be dropped in future NIH GPRA Plan/Reports.

Previous NIH GPRA plans also included two other research outcome goals -- the genomic resources goal and the HIV/AIDS vaccine goal. The genomic resources goal is being phased out after FY 2002 reporting because it overlaps with two of the new specific/representative goals. The HIV/AIDS vaccine goal is migrating into the same format as the new goals, giving NIH a total of 27 research outcome goals for FY 2003 and FY 2004.

Given the fact that the original comprehensive goals are being replaced, OMB excused NIH from FY 2002 reporting on the now obsolete goals. Accordingly, NIH did not arrange for conduct of an independent assessment of the FY 2002 performance it reported on under the research outcome goals. Nonetheless, as required under GPRA, NIH has assembled reporting materials that can be assessed (see http://www1.od.nih.gov/osp/ospp/gpra/gpra_nih.htm) and thus is in compliance with GPRA reporting requirements.

Performance Goal Details – Research Outcomes

Goal a) Add to the body of knowledge about normal and abnormal biological functions and behavior.

Clinical advances occur in the context of a body of knowledge developed by basic and applied biomedical researchers. A great deal of this body of scientific knowledge is accumulated in stepwise fashion. Investigators strive to understand broad processes of health and disease (for example, how genes are expressed or what causes cancer metastasis) by elucidating the molecular factors, chemical reactions, and biological mechanisms that contribute to the processes.

Progress in treating the complex diseases that affect our society, which include heart disease, diabetes, cancer, AIDS, hepatitis, mental illness, and many others, is abetted by an understanding of genetic and environmental mechanisms that are involved. Advances that seem to happen overnight, such as the emergence of a useful pharmacological drug or an understanding of a disease etiology, are usually based on a body of knowledge that was accumulated over many years. When a host of investigators use complementary approaches to address related questions, incremental advances can be translated into improved public health.

Both basic and clinical research constitute the science assessed under Goal a. The research considered within this goal is intended to expand the body of basic scientific knowledge, to increase understanding of health and disease, and to provide concrete information that can be applied to improving health and health care.

For fiscal years 1999-2001, NIH measured its success under this goal against the broad target of progress in advancing scientific understanding. For fiscal years 2002 and 2003, NIH had established two representative subgoals to better define NIH's research plans under this goal. However, NIH's original comprehensive research outcome goals (Goals a – e) and the planned subgoals (Goals a-h in earlier plans) are being replaced with a set of specific representative goals. Thus, for purposes of FY 2002 reporting, NIH is using the original goal.

Annual Performance	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Progress in advancing scientific understanding in key fields bearing on our knowledge of biological functions and behavior in their normal and abnormal states.	⩔	⩔	⩔	◆		

⩔ **Target Substantially Exceeded**	◆ **Target Successfully Met**	◇ **Target Active**	✕ **Target Not Met**

Summary of Performance Results

In FY 1999 - 2001, Assessment Working Groups unanimously concluded that NIH had substantially exceeded the goal of adding to the body of knowledge about normal and abnormal biological functions and behavior.

NIH reporting on FY 2002 performance can be found at http://www1.od.nih.gov/osp/ospp/gpra/gpra_nih.htm.

Goal b) Develop new or improved instruments and technologies for use in research and medicine.

The development of new or improved instruments and technologies has always played a complementary and critical role in the success of biomedical research. The dawn of the 21st century finds biomedical research increasingly more complex and multi-disciplinary. As the result, researchers face the constant challenge of finding new and more effective ways to probe these complex systems. Only then, can important information be translated to knowledge and ultimately, converted into effective prevention, diagnosis, and treatment of diseases for mankind. Unlike a century ago, this effort requires collaborations between and among scientists from a wide range of disciplines, including chemistry, biology, genetics, mathematics, computer science, engineering, and physics. Nowhere is this more evident than the ever expanding field of genomics–the collection and analysis of genetic information.

In many cases, advances in instrumentation continue to be built on previous research. One good example is found in the area of imaging. Imaging techniques have continued to be fine-tuned and become more sophisticated, greatly aiding our ability to understand complex systems such as the visual and central nervous systems. Traditional tools such as electron microscopy have also been improved to give images with much greater resolution–allowing live detailed observation, in some cases, of complex cellular processes.

In other cases, instrumental techniques, which traditionally have not been widely used in biomedical research, have found new audiences. For example, mass spectrometry–widely used in chemistry and physics–is now being modified to find drug targets, analyze cocaine exposure in newborns, and separate complex proteins. Other spectroscopic techniques such as X-ray crystallography now give better 3-dimensional molecular details of large, complex proteins.

Microarray techniques have continued play a key role in the area of instrumentation. They have revolutionized the speed and efficiency with which scientists can analyze genomic data. New and improved databases have been developed for storing, compiling, and analyzing the vast amount of microarray data. The ability to analyze many genes simultaneously allows scientists to compare and characterize genes involved not only in a variety of genetic disorders, but also those important to the developmental and aging processes.

In the area of information technology, the refinement of databases has allowed researchers to better classify and predict 3-dimensional protein structures based on their amino acid sequences. A firm grasp of the structures of proteins is the key to understanding root causes of diseases. Innovative algorithm development has also played a key role in producing effective models for monitoring specific disease stages. It helps monitor DNA changes in tumors, improves colon cancer detection, and predicts survival rate of end-stage liver disease, to name just a few examples. In another related area, improved statistical analysis of various clinical studies has led to better data interpretation, evaluation, and identification of fundamental causes of serious epidemics.

For fiscal years 1999-2001, NIH measured its success under this goal against the broad target of progress in developing new instrumentation or technologies that enhance capabilities for investigating biological functions and diagnosing and treating diseases and disorders. For fiscal years 2002 and 2003, NIH had put in place two representative subgoals to better define NIH's research plans under this goal. However, NIH's original comprehensive research outcome goals (Goals a – e) and planned subgoals (Goals a-h in earlier plans) are being replaced with a set of specific representative goals. Thus, for purposes of FY 2002 reporting, NIH is using the original goal.

Annual Performance	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Progress in developing new instrumentation or technologies that enhance capabilities for investigating biological functions and diagnosing and treating diseases and disorders.	⩔	⩔	⩔	◆		

⩔ **Target Substantially Exceeded**	◆ **Target Successfully Met**	◇ **Target Active**	✕ **Target Not Met**

Summary of Performance Results

In FY 1999 - 2001, Assessment Working Groups unanimously concluded that NIH had substantially exceeded the goal of developing new or improved instruments and technologies for use in research and medicine.

NIH reporting on FY 2002 performance can be found at http://www1.od.nih.gov/osp/ospp/gpra/gpra_nih.htm.

Goal c) Develop new or improved approaches for preventing or delaying the onset or progression of disease and disability.

Disease and disability exact enormous tolls on our society, both economic and personal. Rising health care costs highlight the importance of research that seeks to prevent disease and disability, or to delay and/or minimize its impact. Basic research is the first stage in the development of preventive measures. With a solid foundation of basic research, NIH-funded scientists have developed a broad range of preventive measures. These measures include interventions that change behaviors, screening tools that identify susceptible individuals, vaccines that block transmission of infectious diseases, and drugs that prevent the development of serious disease or disability in individuals who have already acquired a disease or who are genetically at risk.

Seemingly simple behavioral interventions can be very effective with respect to many types of diseases. For example, behavioral measures may be important in preventing the transmission of infectious diseases, in stopping or controlling alcohol and drug abuse or other abusive behavior, and in preventing obesity, diabetes, and cardiovascular disease. Behavioral measures such as the adoption of exercise programs or other changes in lifestyle can be very effective in preventing many diseases or impairments, and may eliminate the need for riskier or more expensive preventive measures, such as drug therapy.

In the best of both worlds, behavioral interventions, vaccines, and other interventions targeted at individuals with a genetic susceptibility would altogether prevent the development of disease or disability. However, it is inevitable that preventive measures aimed at limiting or controlling disease after its onset will be necessary. These may be microbicidal or physical measures that prevent the transmission of infectious disease, or drugs that reduce the transmission by lowering the concentration or viability when taken by individuals with low-level or pre-symptomatic indications of disease.

Targeting preventive and disease- or disability-delaying health interventions to at-risk individuals, as opposed to the general population, not only improves the effectiveness of these measures but also permits efficient use of health care dollars. To eliminate health disparities, targeting measures should include the identification of at-risk ethnic, gender, and socioeconomic groups, as well as identification of individual genetic susceptibilities. And studies that evaluate the relative effectiveness of expensive and inexpensive preventive measures contribute to the effectiveness of prevention efforts by enhancing their adoption by broad segments of the population.

For fiscal years 1999-2001, NIH measured its success under this goal against the broad target of progress in developing new or improved approaches for preventing or delaying the onset of diseases or disabilities. For fiscal years 2002 and 2003, NIH had put in place two representative subgoals to better define NIH's research plans under this goal. However, NIH's original comprehensive research outcome goals (Goals a – e) and planned subgoals (Goals a – h in earlier plans) are being replaced with a set of specific representative goals. Thus, for purposes of FY 2002 reporting, NIH is using the original goal.

Annual Performance	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Progress in developing (or facilitating the private sector's development of) new or improved approaches for preventing or delaying the onset of diseases and disabilities -- and which reflect NIH responsiveness to emerging health needs, scientific opportunities, and new technologies.	⩒	⩒	◆	◆		

⩒ **Target Substantially Exceeded**	◆ **Target Successfully Met**	◇ **Target Active**	✕ **Target Not Met**

Summary of Performance Results

In FY 1999 - 2000, Assessment Working Groups unanimously concluded that NIH had substantially exceeded the goal of developing new or improved approaches for preventing or delaying the onset or progression of disease and disability. In FY 2001 the Assessment Group concluded that the goal had been successfully met.

NIH reporting on FY 2002 performance can be found at http://www1.od.nih.gov/osp/ospp/gpra/gpra_nih.htm.

Goal d) Develop new or improved methods for diagnosing disease and disability.

Disease diagnosis is a practical area that links the growing body of basic biomedical knowledge to the health care system and the health care consumer. An understanding of genetics and the molecular basis of disease are particularly applicable to improvements in diagnosis and development of new diagnostic tests. Frequently, basic molecular research identifies a molecule or metabolic byproduct that has tremendous value in diagnosis. In addition, other areas of technological innovation provide information for diagnosis, and practical application of research in imaging and spectroscopy is a potential source of valuable new instrumentation.

Early and accurate diagnosis of disease often has an impact on treatment. There are many cancers for which early diagnosis may be the key to survival. For some conditions, disability can be controlled or limited if treatment begins early. Possible features of an advance in diagnosis include increased accuracy, the replacement of an invasive technique with an approach that is non-invasive, and the ability to identify a condition that puts a patient at risk before disease symptoms are apparent.

For fiscal years 1999-2001, NIH measured its success under this goal against the broad target of progress in developing new or improved diagnostic methods that are more accurate, less invasive, and/or more cost effective. For fiscal years 2002 and 2003, NIH had combined this goal with goal e and put in place two representative subgoals to better define NIH's research plans under the new, combined goal. However, NIH's original comprehensive research outcome goals (Goals a – e) and the planned subgoals (Goals a-h in earlier plans) are being replaced with a set of specific representative goals. Thus, for purposes of FY 2002 reporting, NIH is using the original goal.

Annual Performance	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Progress in developing (or facilitating the private sector's development of) new or improved diagnostic methods that are more accurate, less invasive, and/or more cost-effective -- and which reflect NIH responsiveness to emerging health needs, scientific opportunities, and new technologies.	⩒	⩒	⩒	◆		

⩒ Target Substantially Exceeded	◆ Target Successfully Met	◇ Target Active	✕ Target Not Met

Summary of Performance Results

In FY 1999 - 2001, Assessment Working Groups unanimously concluded that NIH had substantially exceeded the goal of developing new or improved methods for diagnosing disease and disability.

NIH reporting on FY 2002 performance can be found at http://www1.od.nih.gov/osp/ospp/gpra/gpra_nih.htm.

Goal e) Develop new or improved methods for treating disease and disability.

The development of new treatments for disease is often viewed as the culmination of many years of collaborative effort on the part of the research community. However, the search for treatments actually forms part of a larger knowledge loop. In addition to enhancing quality of life for patients, the search for new treatments often illuminates understanding of fundamental biochemical and molecular processes. Conversely, researchers unraveling the basic mechanisms of complex disease states discover new approaches to treatment. This year, researchers have developed many new and improved strategies for treating disease states, and in the process, they have made significant contributions to the realms of basic science.

From standard-of-care issues for individual patients to world health concerns, NIH-sponsored research has forged many new paths for the treatment and control of numerous diseases. In addition to providing novel approaches to and a refined understanding of treatment paradigms, this research lays the groundwork for further investigations designed to improve the quality of life for patients worldwide.

As indicated in earlier NIH GPRA plans, NIH had planned to combine this goal with goal d and report on subgoals for the combined goal. However, NIH's original comprehensive research outcome goals (Goals a – e) and the planned subgoals (Goals a-h in earlier plans) are being replaced with a set of specific representative goals. Thus, for purposes of FY 2002 reporting, NIH is using the original goal.

Annual Performance	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Progress in developing (or facilitating the private sector's development of) new or improved treatments that expand therapy options; improve the length and quality of life; and/or are more cost effective -- and which reflect NIH responsiveness to emerging health needs, scientific opportunities, and new technologies.	⩔	⩔	⩔	◆		

⩔ **Target Substantially Exceeded**	◆ **Target Successfully Met**	◇ **Target Active**	✕ **Target Not Met**

Summary of Performance Results

In FY 1999 - 2001, Assessment Working Groups unanimously concluded that NIH had substantially exceeded the goal of developing new or improved methods for treating disease and disability.

NIH reporting on FY 2002 performance can be found at http://www1.od.nih.gov/osp/ospp/gpra/gpra_nih.htm.

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

Human Genome Project

In April 2003, the DNA sequence of the human genome will be completed. The NIH, along with the International Human Genome Sequencing Consortium, announced the availability of a working draft of the human genome sequence in June 2000, and published the initial analysis in February 2001. Since then, the consortium has been working on "finishing" the human genome sequence, a process that involves closing all of the gaps in the draft version and ensuring that all of the regions of the genome that are amenable to sequencing meet the consortium's high standards for quality and completion. The Human Genome Project expects to finish the analysis in time for the 50th anniversary of Watson and Crick's discovery of the double helix structure of DNA, a landmark achievement in the annals of scientific research.

The availability of the genome sequence of humankind will mark the starting point of the genome era in biology and medicine. There is now much important work to do to deliver on the promise that these advances in genomics offer for human health.

The Human Genome Project is already producing results that will have an effect on human health. As of April 2002, the amount of highly accurate finished sequence was 76 percent. The average accuracy of all of the DNA sequence in this assembly is more than 99.99 percent. The sequence and analysis was a remarkable achievement, representing the work of thousands of scientists working in 20 associated genome centers around the globe. The "Book of Life," as some have termed the human genome, is actually three books. It is a history book that tells the narrative of the human species' journey through time. It is a shop manual that provides the parts list, and an incredibly detailed blueprint for building every human cell. And finally, it is a transformative textbook of medicine that provides insights, giving health-care providers immense new power to treat, prevent, and cure disease.

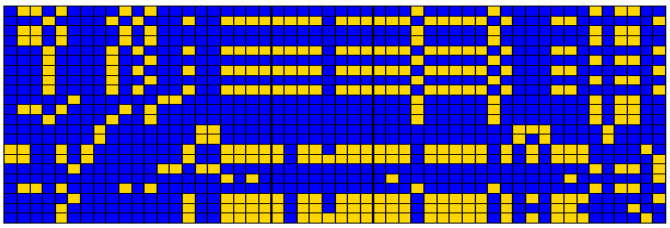
Because of the enormous value of sequence information to researchers around the world, Human Genome Project scientists have placed all DNA sequence data in public databases where they are immediately and freely available with no restrictions on their use or redistribution. The information is scanned daily by tens of thousands of scientists in academia and industry, as well as by commercial database companies providing information services to biotechnologists.

Haplotype Map

In addition to sequencing activity, the Human Genome Project and its partners also have been creating a catalogue of the places in the genome where the DNA sequence differs among individuals. The basic building block of DNA is the nucleotide, and DNA consists of a string of the four nucleotides: adenine, cytosine, guanine, and thymine (A, C, G, T). Human genes may exist in many different forms, some of them differing only by a single A, C, G, or T. The most common variations are single nucleotide polymorphisms (SNPs), or places where the DNA

sequence varies by a single base pair or DNA letter. Between two unrelated individuals, these occur approximately once every 1000 bases. Through these efforts, nearly 3 million SNPs have been identified and are available in public databases. A high-density map of SNPs is expected to be a valuable research tool that will help scientists pinpoint genetic differences that predispose some, but not others, to disease and underlie variability in individual response to treatment.

While whole genome association studies are potentially extremely valuable, they remain dauntingly expensive, requiring additional technical improvements. The next key step of the Human Genome Project is the generation of a new map of the patterns of genetic variations across populations, a so-called "haplotype map." The SNP variants do not occur at random, but are correlated in important ways with their neighbors. By finding the pattern of variation along chromosomes, scientists can select a much smaller "gold standard" set of SNPs distributed along the chromosomes that will represent nearly all of the underlying pattern of variation. Thus, testing for one or two SNPs can reveal the information about variation for a large region surrounding the indicator SNPs. Although still in its formative stage, the development of a haplotype map will provide a critical resource for researchers seeking to identify and understand the genetic basis of common human diseases. As currently envisioned, the haplotype map will be developed through an international collaboration, and should have covered 80% of the genome by 2005.



Haplotypes (rows) for 22 chromosomes in the region of the ACE gene. Each column is for a SNP; the more common SNP variant is in black and the rarer form of SNP is in white.
Rieder *et al.*, Nature Genetics, May 1999, vol. 22, page 61.

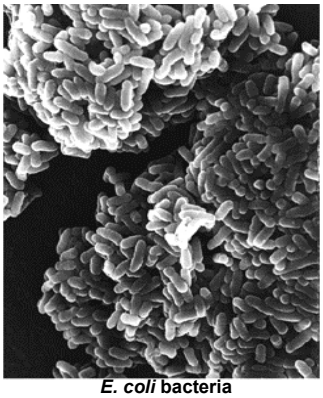
Comparative Genomics

The study of model organism genomes, including microorganisms and mammals such as the mouse and the rat, will also lead to greater understanding of the functions of human genes and their role in health and disease. 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.

The sequencing capacity developed by the international sequencing consortium now makes it possible to pursue large-scale sequencing of the genomes of other organisms. Interest in sequencing the genomes of additional organisms has grown enormously. Accordingly, the National Human Genome Research Institute (NHGRI) has developed a competitive process in which the selection of genomes to sequence using NHGRI-supported sequencing capacity is based on investigator–or community-initiated proposals and scientific merit as determined by peer review. This systematic process was implemented to encourage focus on the scientific questions that can be addressed by new sequence data. This will allow all investigators, the sequencers, and the NIH to participate in a process for selecting new organisms for genomic sequencing on the basis of specific, well-defined scientific goals, taking advantage of the rigor and robustness provided by scientific discussion and peer evaluation.

The first deadline for submitting proposals to the NHGRI for consideration was in February 2002. A working group of the National Advisory Council for Human Genome Research Council reviewed the proposals and concluded that several organisms, including the chimpanzee, chicken, honeybee, sea urchin, Tetrahymena (a protozoan), and seven fungi all have high priority and should be considered for sequencing upon completion of the three current projects (human, mouse, and rat). The Council adopted the working group's recommendations. Because some of these organisms are of interest to other Federal agencies (such as the U.S. Department of Agriculture in the case of the chicken, the honeybee, and certain fungi of agricultural importance; other institutes at NIH in the case of fungi of medical importance; an international consortium interested in the chimpanzee; the National Science Foundation), the NHGRI will be working with these agencies to ensure that appropriate coordination occurs and in other ways to speed the sequencing of these and other high priority genomes.

Genomes of Pathogenic Microbes



***E. coli* bacteria**

NIH also is working to sequence the entire genomes of pathogenic microbes and invertebrate vectors of infectious diseases. Worldwide, infectious diseases are the leading cause of death for children and young adults, and the second leading cause of death overall. In the United States, infectious diseases are the third ranking killer. Pathogen gene sequencing efforts are enabling scientists to locate genes that may lead to potential new vaccine candidates and drug targets so that infectious diseases can be prevented and treated. In addition, knowing a pathogen's genetic sequence will help researchers better understand how mechanisms of pathogenesis and pathogen mutation contribute to drug resistance. Many medically important pathogens have been sequenced, including the bacteria that cause tuberculosis, gonorrhea, chlamydia, and cholera and the microbes that cause malaria, pneumonia, meningitis, sepsis, dysentery, anthrax, and plague.

To accelerate use of this research output, NIH's data release policy for microbial genome sequencing projects requires that grantees obtain approval of their data release plans and deposit sequence data as they are acquired in specialized, Internet-publicly-accessible databases. Access to the sequence data, prior to its publication in peer-reviewed journals, enables the broader research community to identify genes of interest and to jump-start relevant experimental studies. For some pathogen genome sequencing projects, for example the malaria parasite, NIH coordinated its support with funding provided by other national and international agencies, including the U.S. Department of Defense, The Burroughs Wellcome Fund, the World Health Organization, and the United Kingdom's Wellcome Trust. In FY 2000, the NIH entered into an agreement with the Defense Advanced Research Project Agency for sequencing the genomes of pathogens that have the potential for use as bioterrorism agents. In 2001, as part of an international consortium, NIH supported a project to sequence the genome of the mosquito that transmits malaria, *Anopheles gambiae*, and very recently, the assembled genome sequence was

made freely available to the research community. In addition to coordination of funding, these collaborative efforts help to establish quality control, data release, and other standards by which genome projects are evaluated and funded. Moreover, these interactions are promoting the optimal use of the accrued data by relevant microbiologists and infectious disease specialists. With NIH support, 32 bacterial pathogens, 1 parasitic protozoan, 1 chromosome of another parasitic protozoan, and 1 infectious disease vector already are sequenced. That is, every nucleotide has been identified and properly ordered within the genome (see table below). Once completed, the sequences have been annotated and analyzed using sophisticated computer programs to predict gene structure and function. This information serves as the basis for further experimental studies that help to identify the features of the genome that determine the microbe's ability to infect humans and cause disease.

Completed Pathogen, Parasitic Protozoa Chromosome, and Vector Sequencing Projects

Pathogen	Disease
Actinobacillus actinomycetemcomitans	Periodontal diseases
Anopheles gambiae	Malaria vector (mosquito)
Bacillus anthracis (Kruger B1 strain)	Anthrax
Bacillus anthracis (Ames strain)	Anthrax
Bacillus anthracis (Western North American strain)	Anthrax
Bacillus cereus	Food poisoning
Brucella suis	Brucellosis
Chlamydia pneumoniae	Respiratory disease
Chlamydia trachomatis (human strain)	Chlamydial infection – ocular or genital
Chlamydia trachomatis (mouse strain)	Chlamydial infection – pneumonitis
Coxiella burnetii	Q Fever
Enterococcus faecalis (strain V583)	Nosocomial infections
Escherichia coli 0157:H7	Gastritis, hemolytic uremic syndrome
Escherichia coli CFT073	Gastritis, hemolytic uremic syndrome
Haemophilus ducreyi	Chancroid
Klebsiella pneumoniae	Pneumonia
Leishmania major Chromosome 1	Cutaneous leishmaniasis
Mycobacterium avium	Pulmonary disease; opportunistic disease
Mycobacterium tuberculosis	Tuberculosis
Neisseria gonorrhoeae	Gonorrhea
Plasmodium falciparum Chromosomes 2, 10, and 11	Malaria
Porphyromonas gingivalis	Periodontal diseases
Salmonella paratyphi	Gastritis; Enteric fever
Salmonella typhimurium	Food-borne diseases; gastritis
Shigella flexneri	Shigellosis
Staphylococcus aureus (strain COL)	Bacteremia; endocarditis
Staphylococcus aureus (strain 8325)	Bacteremia; endocarditis
Staphylococcus epidermis	Nosocomial infections
Streptococcus pneumoniae	Respiratory disease
Streptococcus pyogenes	Group A strep
Streptococcus mutans	Dental caries
Treponema pallidum	Syphilis
Ureaplasma urealyticum	Nongonococcal urethritis
Vibrio cholerae	Cholera
Yersinia pestis	Plague

As is true for the Human Genome Project, new technologies employing advances in miniaturization, robotics, and fabrication are providing new tools for research on microbial genomes. Using pathogen genome sequencing information, high-density arrays of microbial DNA segments (gene "chips" and microarrays) are being used to determine how gene expression varies under different conditions, at different stages of infection, and in different isolates of the organism, as well as to discover new drug and vaccine targets. In parallel, advances in protein detection and characterization are enabling scientists to define a pathogen's proteome (the total complement of proteins within a cell) and its metabolome (the entire set of metabolic pathways accomplished by a cell). More powerful computerized databases are being developed to handle all of the sequence, structure, expression, and function information and to disseminate this information to the research community.

Overall, NIH supported approximately 40 large-scale genome-sequencing projects for microbial pathogens and invertebrate vectors of disease in FY 2002. In addition, NIH continues to support a contract, awarded at the end of FY 2001, for a pathogen functional genomics resource center, which is providing the research community with resources for functional analysis of microbial pathogens and invertebrate vectors of infectious diseases.

NIH/NIAID is in the process of reviewing its ongoing priorities and policies for the support of large-scale genome projects as well as other genomic related activities. Advice from outside experts on sequencing priorities has been gained from two Blue Ribbon Panels, one in 1999 and another in February 2002 on Bioterrorism and its Implications for Biomedical Research. It is anticipated that an update of NIH/NIAID priorities and policies for large-scale genome projects will be announced on the NIH/NIAID's Pathogen Genomics Web site.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003[1]**	**FY 2004[1]**
Complete the sequencing of the human genome:						
Reach U.S. annual production rate of human genomic sequence of 90 million base-pairs.	◆					
Reach worldwide annual production rate of human genomic sequence of 220 million base-pairs.	◆					
Total human genomic sequence completed worldwide at the end of FY 1999 at 400 million base-pairs.	◆					
Finish the sequence of at least one human chromosome.		◆				
Worldwide effort completes "working draft" of human genome sequence (90% complete, 99% accurate). U.S. contributes two-thirds of that amount, and NIH contributes 85% of U.S. total.		◆				
Worldwide effort completes "full shotgun" of human genome sequence (95% complete, 99.9% accurate).			◆			
Identify 2,500,000 human single nucleotide polymorphisms (SNPs).			◆			
Finish one-third of human genome (accuracy of at least 99.99%).			◆			
Finish two-thirds of human genome (accuracy of at least 99.99%). NIH grantees will be responsible for half of this target, i.e., one-third of the human genome.				◆		
Obtain full-length clones and sequence data for 20,000 mammalian cDNAs.				◆		
Sequence the mouse genome:						
Complete 2X depth of coverage toward the working draft of the mouse genome (90% coverage, 99% accurate).			◆			
Complete full shotgun coverage of the sequence of the mouse genome; finish 10% of the mouse genome.				◆		
Sequence the rat genome:						
Complete 1X depth of coverage toward the working draft of the rat genome (90% coverage, 99% accurate).			◆			
Complete 3X sequence coverage of the rat genome.				◆		
Sequence the genomes of other model organisms:						
Complete the sequence of the *C. elegans* genome (97 million base-pairs).	◆					
Complete sequence of the genome of *Drosophila melanogaster* (excluding heterochromatin).		◆				

[1] The FY 2003 an FY 2004 targets for this goal have been deleted because the goal is being superceded by goals 7c and 8a under the new NIH approach to research outcome goals.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003** [1]	**FY 2004** [1]
Sequence the genomes of pathogens:						
Complete worldwide sequencing effort of the entire genome of *Plasmodium falciparum*.			→	◆		
Complete sequencing effort of five additional bacterial pathogens and five chromosomes of protozoan parasites.			→	→		
Augment existing knowledge of pathogen genomes by initiating sequencing projects for at least six additional genomes (bacterial, fungal, parasitic).			◆			
Initiate pathogen genome sequencing projects for additional NIH priority areas based upon Blue Ribbon Panel Report.				◆		
Develop and facilitate access to new technologies for investigating pathogen gene functions and pathogen/host interaction: [1]						
Establish a mechanism to facilitate access to resources, services, and technologies (bioinformatics, scanning, microarrays, genome chips) needed to investigate microbial gene function.				◆		
Develop technologies that assess, display, and/or query human genome sequence data to facilitate investigation of how the immune system responds during different disease conditions (i.e., infection, transplantation, autoimmune disease, and other diseases).				◆		

◆ **Target Met**	◇ **Target Active**	→ **Target Not Met and Extended**	✕ **Target Not Met**

Summary of Performance Results

- **The sequencing of the human genome remains ahead of schedule.** All targets in FYs 1999 through 2002 have been met or exceeded. Sequencing the human genome has always been the ultimate goal of the Human Genome Project. Between March 1999 and June 2000, production of human genome sequence skyrocketed. During this time, scientists sequenced 1,000 DNA letters a second−7 days a week, 24 hours a day. The resulting working draft version of the sequence covered more than 94 percent of the human genome with over one-third in highly accurate finished form. The average accuracy of all of the DNA sequence in this assembly is greater than or equal to 99.9 percent. The International Human Genome Sequencing Consortium published in *Nature* the sequence and initial analysis of the human genome on February 15, 2001. The sequence and analysis was a remarkable achievement, representing the work of thousands of scientists working in 20 HGP-associated genome centers around the globe.

[1] The FY 2003 an FY 2004 targets for this goal have been deleted because the goal is being superceded by goals 7c and 8a under the new NIH approach to research outcome goals.

By the end of FY 2002, The International Human Genome Sequencing Consortium surpassed the goal to finish more than two-thirds of the human genome (accuracy of at least 99.99%), by finishing over 88% of the human genome. NIH grantees have completed roughly half of this amount, i.e., over 42% of the human genome (considerably more than their goal of 33%). The essentially complete sequence of the human genome is expected to be achieved in FY 2003.

One aspect critical to deciphering the information contained within genomic sequence data is the ability to identify and study those fragments within the sequence that contain the code for individual genes. Therefore, the goal of the Mammalian Gene Collection project is to provide a complete set of full-length (open reading frame) sequences and cDNA clones of expressed genes for human and mouse. The goal for FY 2002, to obtain sequence data for 20,000 putative full-open reading frame mammalian cDNAs, was exceeded, with the centers participating in the project sequencing 23,000 clones. Over a two-year period, and accounting for redundancy, a total of 19,600 unique sequences with complete open reading frames have been submitted to public databases. A manuscript outlining the progress of the project has been submitted for publication. This growing collection of tools and information will facilitate and expedite research efforts to increase the understanding of the relationship between genetic information and disease processes.

Because, under the new NIH approach to research outcome goals, the current NIH genomic resource goal is being superceded by two new goals, future GPRA reports will not cover performance on the outstanding targets regarding cDNAs. (FY 2003: Obtain full-length clones and sequence data for 20,000 mammalian cDNAs; FY 2004: Obtain full-length clones and sequence data for 22,000 human and non-human cDNAs.) Nonetheless, this research will continue and information on performance can be obtained at http://www.genome.gov/page.cfm?pageID=10001047.

The rationale for the Human Genome Project has been the promise of improving human health. We are already beginning to see the fruits of that investment. More than 50 genes involved in human disease have been discovered, based on access to the public human genome sequence data. These, some of which were cited in the *Nature* publication, represent research that could not have been accomplished in nearly the same way or would not have been as profound were it not for the draft sequence of the human genome.

Because of the enormous value of sequence information to researchers around the world, HGP scientists have placed all DNA sequence data in public databases where it is immediately and freely available with no restrictions on its use or redistribution. The information is scanned daily by tens of thousands of scientists in academia and industry, as well as by commercial database companies providing information services to bio-technologists. More information about the sequencing and analysis of the human genome is available at: http://www.nhgri.nih.gov/genome_sequence.html.

- **Full shotgun coverage of the sequence of the mouse genome completed.** The Mouse Genome Sequencing Consortium (MGSC) became fully operational in FY 2001 and has made rapid progress. In a landmark advance in genomics, the Consortium announced in May 2002 that it has assembled an advanced draft sequence of the mouse genome—the

genetic blueprint for the most important animal model in biomedical research. The Consortium is on target to generate a more detailed version of the sequence by 2003, and a finished sequence by 2005. By the end of FY 2002, 10.9% of the mouse genome was finished. The mouse genome-sequencing project completed the whole genome shotgun phase of the project and achieved approximately 7X shotgun coverage, containing 96% of the mouse genome in an assembled sequence. On December 5, 2002, the Mouse Genome Sequencing Consortium published a landmark paper that describes the first analysis of this data. As with the human sequence, the data from the mouse project is rapidly released to the public databases and the World Wide Web with no restrictions. The scientific community is using the data set extensively. NHGRI and the MGSC publish the *Mouse Genome Monthly* newsletter http://mouse.ensembl.org/newsletter/, one of many methods developed to keep the community of mouse researchers abreast of the progress in sequencing the mouse genome.

Because, under the new NIH approach to research outcome goals, the current NIH genomic resource goal is being superceded by two new goals, future GPRA reports will not cover performance on the outstanding targets regarding sequencing of the mouse genome. (FY 2003: Complete full shotgun coverage of the sequence of the mouse genome; finish 40% of the mouse genome, i.e., greater than 99.99% accuracy; FY 2004: Finish 65% of the mouse genome, i.e., greater than 99.99% accuracy.) Nonetheless, this research will continue and information on performance can be obtained at http://www.genome.gov/page.cfm?pageID=10001859.

- **Greater than 3X sequence coverage of the rat genome completed.** NIH is on target to meet its objective to produce a draft quality version of the rat sequence by 2003. The effort became fully operational in FY 2001 and has made rapid progress. By the end of FY 2002, the rat genome sequencing project generated approximately 5.5X coverage through adding random whole genome shotgun reads to light shotgun coverage of a set of genomic clones. As with the human sequence, the data from the rat project is rapidly released to the public databases and the World Wide Web with no restrictions. The scientific community is using the data set extensively.

  Because, under the new NIH approach to research outcome goals, the current NIH genomic resource goal is being superceded by two new goals, future GPRA reports will not cover performance on the outstanding targets regarding sequencing of the rat genome. (FY 2003: Complete 5-6X sequence coverage of the rat genome.) Nonetheless, this research will continue and information on performance can be obtained at http://www.genome.gov/page.cfm?pageID=10001855.

- **The genome sequences of other model organisms were completed.** The FY 1999 target and FY 2000 target were both met. In the first quarter of FY 1999, the complete sequence of the *Caenorhabditis elegans* (roundworm) genome was published in *Science*, and in FY 2000, a consortium of publicly funded scientists, in collaboration with a private company (Celera Genomics), published the complete sequence of the *Drosophila melanogaster* (fruit fly) genome. In FY 2002, NIH completed the sequence of *Wolbachia* sp., an endosymbiont (an organism that lives within the body of another host organism) of *Drosophila* and *Brugia malayi* (tissue nematode or roundworm).

- **Scientific milestone in malaria parasite and mosquito vector genome sequencing was achieved.** Progress continues to be made in sequencing of other protozoan parasites and bacteria. The complete genome sequence of *Plasmodium falciparum* and *Anopheles gambiae*, the most lethal malaria-causing parasite and its mosquito vector, respectively, were published in 2002. The detailed map of the parasite's 5,300 genes, included in its 14 chromosomes, and their predicted functions is a milestone in malaria research. NIH contributed to the extraordinary scientific achievement and worldwide sequencing effort. Due to the size and complexity of the *P. falciparum* genome, sequencing of this genome was divided among different institutions under the coordination of the International Malaria Genome Sequencing Consortium. NIH funding was used to support the sequencing of 3 of the 14 chromosomes of *P. falciparum*, specifically chromosomes 2, 10, and 11. This accomplishment will speed efforts to investigate and develop control strategies for this devastating disease. Researchers overcame significant technical challenges on the way to accomplishing this—a task that has required more time than planned. The genome of *P. falciparum* is very rich in adenine and thymine (82%), chemicals that make up the nucleotide bases of DNA, making sequence reads more difficult than other projects. In addition, assemblies of the random sequences were more difficult because the adenine and thymine content of large insert libraries was not stable.

  In 1999, NIH joined the Anopheles Gambiae Genome Consortium (AGGC) to accelerate sequencing of the 14,000 genes of *Anopheles,* the insect vector responsible for malaria transmission. In August 2001, NIH expanded its support for sequencing the *Anopheles* genome, and sequencing was completed in 2002. Information obtained from the *Anopheles* genome is giving researchers new insights into mosquito physiology and behavior and may help determine how *Anopheles* reacts, at a molecular level, to infection with *Plasmodium* parasites. A more detailed understanding of this interaction could lead to improved mosquito control efforts.

  Sequencing of four protozoan parasites (*Giardia lamblia*, *Leishmania major*, *Trypanosoma brucei,* and *Trypanosoma cruzi*) is ongoing and is expected to be completed in FY 2004. NIH completed the sequencing of 10 bacterial pathogens in FY 2001. Of the 10 bacterial pathogens sequenced: the genome sequences of five bacterial pathogens were published in FY 2001 (*Escherichia coli* O157:H7, *Salmonella typhimurium*, *Streptococcus pneumoniae, Streptococcus pyogenes,* and *Ureaplasma urealyticum*); the genome sequence of *Mycobacterium tuberculosis* was published in FY 2002; and manuscripts are in preparation regarding the sequences of an additional four bacteria (*Enterococcus faecalis*, *Haemophilus ducreyi, Neisseria gonorrhoeae*, and Staphylococcus *aureus*). Also, NIH initiated more pathogen genome sequencing activities in FY 2001 than originally planned (nine rather than six); these include *Brugia malayi*, *Clostridium perfringens*, *Coccidioides immitis, Cryptococcus neoformans*, Group B streptococcus, *Histoplasma capsulatum*, *Rickettsia rickettsii, Schistosoma mansoni*, and *Toxoplasma gondii.*

  In FY 2002, fourteen bacterial pathogen sequences were completed, including *Bacillus anthracis* (3 strains), *Bacillus cereus*, *Brucella suis, Coxiella burnetii, Escherichia coli* CFT073, *Klebsiella pneumoniae*, *Mycobacterium avium, Salmonella paratyhi A, Shigella flexneri, Staphylococcus aureus-8325, Staphylococcus epidermidis* and *Yersinia pestis).* Three (Ames strain of *Bacillus anthracis*, *Brucella suis*, and *Yersinia pestis*) of the fourteen

bacterial pathogen sequences were published in FY 2002. Two important sequencing projects received initial funding in FY 2002-*Trichomonas vaginalis* and *Aedes aegypti*. Both are pathogens identified by the 1999 Blue Ribbon Panel as among twenty top sequencing priorities. *T. vaginalis* is a protozoan parasite that causes a significant sexually transmitted disease. *A. aegypti* is the vector for the dengue virus.

- **NIH is developing and facilitating access to new technologies for investigating pathogen functions and pathogen/host interactions.** NIH achieved its target of developing and facilitating access to new technologies for investigating pathogen functions early. In FY 2001, a contract was awarded to support a Pathogen Functional Genomics Resource Center (PFGRC) that, by offering a variety of services, technology, and resources to the research community, is significantly augmenting the functional analysis of pathogen genome sequences. PFGRC distributed high quality pathogen-specific microarrays to the broad scientific community. Expansion of this resource in FY 2003 and 2004 is highly likely**.**

  Additional initiatives are being developed to support genomic sequencing, microbial genome databases and bioinformatics, and proteomics technology development research. For example, NIH issued a program announcement to facilitate the application of innovative/emerging technologies to currently funded NIH research projects related to the study of infectious diseases, diseases caused by category A agents of bioterrorism, HIV/AIDS, basic immunology, and immune-mediated conditions. That same fiscal year, NIH funded 28 of 84 grant applications in response to the program announcement.

  NIH is also making progress towards developing technologies that assess, display, or query human genome sequence data to facilitate investigation of how the immune system responds during different disease conditions. In FY 2002, NIAID with several other NIH Institutes and Centers, and the Juvenile Diabetes Research Foundation International, continued to support the International Histocompatibility Working Group (IHWG), an international network of more than 200 laboratories, established in FY 2000, that are collecting and sharing data on the genes of the human leukocyte antigen (HLA) gene complex. The collaborative research projects have identified genetic markers associated with several autoimmune diseases, developed a preliminary map of the killer immunoglobulin-like receptor (KIR) gene complex, and initiated a database to link specific HLA genotypes with the clinical outcomes of hematopoietic stem cell transplantation. New technologies that will be developed or used include sequencing technologies polymerase chain reaction (PCR) and hybridization for the characterization of human HLA genes and their products. Also, bioinformatics tools, such as algorithms for translating traditional serological data into sequence identification, and other tools to store, analyze, and distribute data will be developed.

  In FY 2002, NIAID supported a Proteomics Facility as a Core project within a Center for Translational Research in Human Immunology at Duke University. The project studies the expression of the human proteins encoded by the genes, although not specifically genome sequencing. This Proteomics Facility will allow the application of cutting-edge technologies to define the gene products that control effective immune responses to invading pathogens or to vaccines, and thereby identify new targets for immunoregulation or the development of more effective antibiotics, anti-viral drugs and vaccines. It is anticipated that new

technologies will be developed and/or used including advanced proteomics technologies such as surface plasmon resonance, protein chip capture and differential display, and protein sequencing/ mass spectrometry.

Taking advantage of enormous scientific strides in understanding how the innate immune system rapidly detects and responds to infection, plans were developed in FY 2002 for the practical application of this information in a new program to discover and characterize new adjuvant candidates that will enhance the potency, longevity, and safety of specific vaccines for biodefense, and will also be able to enhance nonspecific immunity for immediate protection against acute infectious threats. Possible technologies that will be developed or used include surface plasmon resonance, protein chip capture and differential display, and protein sequencing/ mass spectrometry, and may also include additional technologies such as flow cytometry and adaptations of eluspot assays.

The Multiple Autoimmune Disease Genetics Consortium, established by NIAID in FY 1999, collects clinical data and genetic material from families in which two or more individuals are affected by two or more distinct autoimmune diseases, and is intended to promote research aimed at discovering the human immune response genes involved in autoimmunity. To date, 102 families have been fully enrolled and 123 families are in the process, working toward the goal of 400 families in 2004. New technologies that will be developed or used include bioinformatics tools and technologies for advanced databasing, data analysis, and data/information distribution.

In FY 2002, the NIAID, the Arthritis Foundation, and the National Institute of Arthritis and Musculoskeletal and Skin Diseases, supported the North American Rheumatoid Arthritis Consortium (NARAC), to collect medical information and genetic material (DNA) from 1,000 families nationwide in which two or more siblings developed rheumatoid arthritis between 18 and 60 years of age and have at least one surviving parent. Identifying these genes will provide insight into the pathogenesis of this disease and could lead to the development of new prevention approaches and treatment strategies. The NARAC database contains information on 902 families, encompassing 1,522 patient visits. Of the 902 families, data for more than half have been validated, including 600 affected sibling pairs. New technologies that will be developed or used include bioinformatics tools and technologies for advanced databasing, data analysis, and data/information distribution.

Goal g) Develop an HIV/AIDS vaccine by 2007.

As the HIV/AIDS pandemic continues to rage around the world, the development of a safe and effective HIV vaccine is a global public health imperative. In 2001, there were 3 million deaths due to AIDS making it the fourth leading cause of mortality, and its impact is going to increase. Worldwide, roughly 5 million people became newly infected with HIV during 2001 and 40 million were living with HIV/AIDS. Over 95 percent of new cases occur in the developing world, mostly among young adults and increasingly in women. About 68 percent and 16 percent of the infections occur in sub-Saharan Africa and South-East Asia, respectively. In seven countries in southern Africa, at least one in five adults are living with HIV. Moreover, HIV/AIDS is poised to devastate other regions as well. HIV infections are rising faster in Eastern Europe than anywhere else in the world. In the Russian Federation, in 2001, there were 250,000 new HIV infections bringing the total number of people living with HIV to 1 million. Moreover, HIV also continues to have a profound effect on children, with approximately 2.7 million below the age of fifteen living with the disease at the end of 2001.

HIV/AIDS is having a worldwide impact as the number of sick and dying people depletes the skilled workforce in the developing world; as commercial, governmental, educational, and military sectors lose capability and productivity; as families lose wage earners; and as a multitude of children become impoverished orphans. In response to this crisis, the United Nations Security Council declared AIDS an issue of national security, representing a new kind of threat to political stability. A safe and effective vaccine suitable for worldwide use offers the best hope for halting the HIV/AIDS pandemic.

In the United States, the number of deaths due to HIV/AIDS has declined substantially in the last decade due primarily to the introduction of antiretroviral drugs. When used in combinations termed highly active antiretroviral therapy, or HAART, these drugs can decrease the amount of virus in the body to virtually undetectable levels. But the prolongation of the lives of infected individuals has resulted in more individuals living with the disease. According to the Centers for Disease Control and Prevention (CDC), an estimated 950,000 residents in the United States are living with HIV/AIDS, with about one-third unaware of their HIV positive status. New infections continue at roughly 40,000 per year, with more than half occurring in individuals younger than 25 years of age. HIV/AIDS also continues to disproportionately affect minorities, notably African Americans.

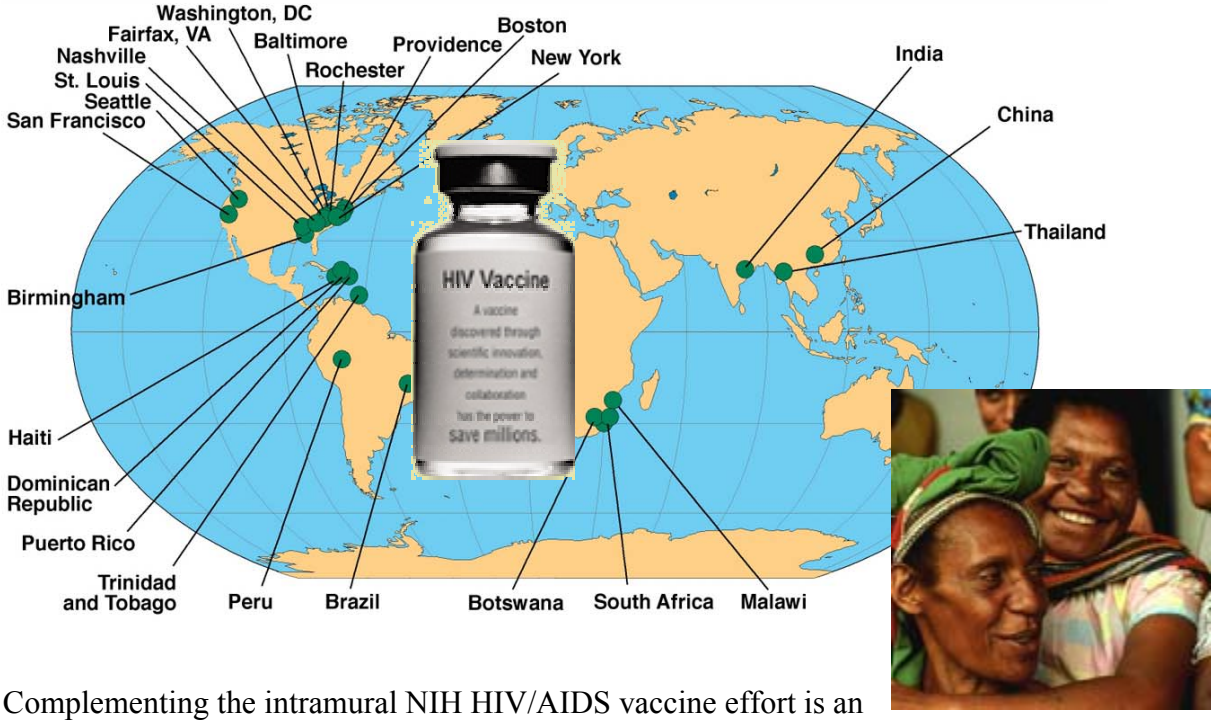
African Americans and HIV/AIDS

- African Americans make up almost 38 percent of all AIDS cases reported in this country even though they represent an estimated 12 percent of the total US population.
- For a decade, HIV/AIDS has been the leading cause of death for African American males aged 25-44.
- In children, African Americans represent almost two-thirds of pediatric cases.

Since the beginning of the epidemic, NIH's comprehensive research program has made significant progress in elucidating the structure of HIV and how HIV attacks the immune system, understanding the role of the immune system in controlling HIV, developing new and improved models for testing candidate vaccines, and in sponsoring and conducting clinical trials. Construction of the Dale and Betty Bumpers Vaccine Research Center (VRC) was completed in 2000. The VRC was created to conduct a comprehensive program of vaccine research on the NIH intramural campus. The primary focus

of the VRC in its first years is developing a vaccine for HIV. In October 2001, just a year after construction of the building, the VRC launched clinical testing of their first vaccine. The role of the VRC is to stimulate multi-disciplinary research, from basic and clinical immunology and virology to vaccine design and production. In addition to the investigations carried out on the NIH campus, the VRC works with scientists in academic, clinical, and industrial laboratories through a program of national and international collaborations. The VRC facilitates the movement of ideas from the broader NIH community into clinical trials by filling the gap between new basic concepts in immunology and initiation of clinical trials through the application of state-of-the-art methods to rational vaccine design. The VRC conceives, designs, and prepares vaccine candidates; performs laboratory analysis and animal testing; and conducts Phase I trials to evaluate candidate HIV vaccines. This involves community education on HIV prevention, recruitment of healthy adults into clinical trials, study design and analysis, and the maintenance of regulatory standards. The VRC also conducts studies of the natural history of HIV infection and evaluates basic aspects of pathogenesis, antigen presentation, and immune response.

NIH HIV Vaccine Trials Networks (HVTN) Domestic and International Sites



Complementing the intramural NIH HIV/AIDS vaccine effort is an extensive extramural program. In all, ten NIH units have activities to advance progress toward an HIV vaccine. These activities include a comprehensive Vaccine and Prevention Research Program sponsored by the National Institute of Allergy and Infectious Diseases (NIAID), the investments of the Fogarty International Center (FIC) in training of foreign scientists, work on primate models sponsored by the National Center for Research Resources (NCRR), and considerable basic research relevant to vaccines on the part of the National Cancer Institute. In the basic research arena, NIAID's extramural HIV vaccine research program supports research on novel vaccine concepts, genetic and immunologic variation, mucosal immunity, delivery methods, adjuvants, and correlates of immune protection. At the preclinical level, NIAID's programs include support for the development of new candidate vaccine designs, evaluation in non-human primate models of HIV/AIDS, pilot-lot production of new candidate

vaccines, and additional preclinical work required for advancement into human trials. At the clinical level, the program coordinates all phases of clinical trials of candidate HIV vaccines and supports work to characterize potentially protective immune responses in vaccinated volunteers. The NIH Office of AIDS Research has the important role of coordinating efforts across Institutes.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003[1]**	**FY 2004[1]**
Design and develop new or improved vaccine strategies and delivery/production technologies:						
Increases in the research portfolio supporting innovative vaccine discovery.	◆					
Progress in the design and development of new or improved vaccine strategies and delivery/production technologies.		◆	◆	◆		
Develop and utilize animal models for testing candidate vaccines:						
Progress in characterization, standardization, and utilization of animal models for evaluating candidate vaccines.		◆	◆	◆		
Advance clinical research by completing ongoing vaccine trials and initiating new trials:						
Progress in completion of ongoing trials.	◆	◆	◆	◆		
Progress in initiation of additional domestic and/or international trials of new or improved concepts and designs, including the progression of promising candidates to larger trials testing vaccine candidates.	◆	◆	◆	◆		
Strengthen collaboration:						
Increased interactions between academic investigators and industry to enhance opportunities for vaccine discovery and product development.	◆					
Progress in collaborating with industry to enhance opportunities for vaccine development.		◆				
Progress in collaborating with scientists in developing countries and with industry to further promote the development of vaccines for worldwide use.			◆	◆		

◆ **Target Met**	◇ **Target Active**	→ **Target Not Met and Extended**	✕ **Target Not Met**

[1] This goal will be superseded by a goal consistent in format with the new specific/representative goals NIH has adopted for FY 2003 and beyond. Because FY 2002 will be the last year this goal is reported on in this form the FY 2003 and FY 2004 targets have been deleted. Future reporting of annual performance will be consistent with reporting on the new goals.

Summary of Performance Results

- **Advances in the design and development of vaccine strategies continue to fuel the pipeline of promising HIV/AIDS vaccine candidates**. In FY 1999, the target to increase the number and dollar value of awards made for vaccine discovery was met. In FYs 2000, 2001, and 2002, notable scientific progress was made, as evidenced by the emergence of new vaccine concepts, the advancement of vaccine concepts into preclinical testing, and promising results in preclinical testing (animal models). Vaccine candidates and concepts evaluated preclinically in FY 2002 include improved DNA vaccines, a stabilized HIV envelope protein, novel viral vectors, and HIV regulatory proteins. There are now over 20 candidate preventive vaccines advancing toward clinical studies by NIH-funded researchers in academia and the private sector. Some recent findings are highlighted below.

  NIH-supported scientists recently developed a vaccine that used a live-attenuated animal virus called vesicular stomatitis virus (VSV) as a "vector," or carrier for HIV genes. The vaccine effectively prevented AIDS from developing in rhesus macaque monkeys that were exposed to a highly virulent strain of simian (monkey)-human immunodeficiency virus (SHIV), a genetically engineered hybrid virus that mimics HIV infection and causes serious disease in macaque monkeys. These results may have potential application in developing a vaccine for humans.

  In previous studies, a DNA vaccine, which included the genes for several components of HIV, was shown to protect monkeys from disease when combined with a second novel viral vector vaccine. More recent studies of this vaccine suggest that specific HIV genes are important in protecting monkeys from disease and are needed in an effective AIDS vaccine. These studies will help guide which genes should be included in a human version of this vaccine.

  NIH has been successful in utilizing animal models for testing candidate vaccines and in making progress toward the development of new models. Animal models, especially the use of non-human primates, continue to provide valuable information in advancing HIV vaccine research and in testing candidate vaccines.

  In one vaccine study, for example, NIH scientists discovered important limitations to developing effective AIDS vaccines using rhesus monkeys. They found that one of eight monkeys given an experimental vaccine against SHIV, and subsequently infected with the virus, showed an increase in virus replication starting at 24 weeks after infection. The monkey was unable to control HIV infection because the virus had mutated in a way that prevented the monkey's "killer" cells, known as cytotoxic T lymphocytes (CTLs), from killing the virus. As a result, the mutant virus replicated and caused disease. These new findings mean that effective AIDS vaccines must elicit a broad range of immune responses, including those mediated by CTL killer cells, so that mutant viruses will not be able to "escape" immune surveillance and cause disease.

  In another vaccine-related study performed in rhesus monkeys, NIAID investigators were able to identify which CTL immune responses against simian immunodeficiency virus (SIV) were responsible for causing the virus to mutate and "escape" detection during the early stage

or acute phase of infection. These CTLs were elicited in response to low protein concentrations of the infecting SIV. It is thought that the CTLs that are sensitized by low protein concentrations are more effective than those requiring larger concentrations of protein. These results have important implications in the design of vaccines to prevent HIV in humans.

NIH's Simian Vaccine Evaluation Units (SVEU) continue to provide a valuable contract resource for evaluating HIV vaccines in non-human primates. In FY 2002, a subcontract was awarded to support the creation and implementation of a non-human primate vaccine database in order to better track the growing numbers of animals under study through the SVEUs. The database will compile all information on study protocols and laboratory data generated following vaccination and virus challenge. In addition, NIH investigators are also continuing to develop, study and evaluate the usefulness of rat and mouse small animal models to evaluate HIV vaccines.

- **NIH has advanced clinical research by completing ongoing vaccine trials and initiating new trials**.

Trials Completed, Ongoing, or Initiated during FY 2002 (10/1/01 – 9/30/02)

Study	Phase	Vaccines Evaluated	Accrual Initiated	Accrual Completed
Completed				
AVEG 032	1	ALVAC vCP205 1 +/- rgp120 SF-2 [4], +/- p24 [4]	8/99	9/99
AVEG 033	1	ALVAC vCP205 + GM-CS F Amended to add Gag-Pol DNA	1/98	6/98
AVEG 034A	1	ALVAC vCP205 or ALVAC vCP1452	9/99	12/99
AVEG 202/ HIVNET 014	2	ALVAC vCP205 +/- rgp120 SF-2[4]	5/97	1/98
HIVNET 007	1	ALVAC vCP205	2/99	8/99
HVTN 203	2	ALVAC vCP1452 +/- rgp120 MN or rgp120 MN/GNE8 (B/B')	12/00	8/01
Ongoing				
VRC-001	1	VRC4302	5/01	--
HIVNET 026	2	ALVAC vCP1452 +/- r rgp120 MN	3/01	--
Initiated				
HVTN 039	1	ALVAC vCP1452 (high dose)	12/01	8/02
HVTN 041	1	Nef-tat +/- rgp120	2/02	5/02
HVTN 803	1	Booster immunization/Passive antibody/Macaques	4/02	4/02

In FY 1999, four new clinical trials were initiated, and of the seven trials started in prior fiscal years, two more completed accrual of patients. In FY 2000, one new trial began and four ongoing trials were completed. In FY 2001, NIH completed seven clinical trials and initiated two new phase II vaccine trials, one domestic and the other in the Caribbean and Brazil. These trials are being conducted under the direction of the HIV Vaccine Trials Network (HVTN). In FY 2002, NIH completed six trials and initiated three new phase I

trials. Enrollment was still ongoing in Trinidad, Brazil, Haiti and Peru for HIVNET 026, a phase II trial initiated in FY 2001. In addition, NIAID and the U.S. Army Medical Research and Materiel Command (USAMRMC) of the Department of Defense (DoD) Program will continue to work closely to ensure the effective integration and coordination of HIV vaccine research efforts. NIAID will increase its support of USAMRMC HIV vaccine development activities, including support of its planned prime-boost vaccine Phase III efficacy trial in Thailand, which is scheduled to commence in 2003.

- **NIH has strengthened collaborations with scientists in developing countries and with industry.** In FY 1999, actions were taken to increase the interaction between academic investigators and industry including a workshop and resource sharing. In FY 2000, four HIV Vaccine Design and Development Team (HVDDT) awards were made to promote university-industry collaboration in the development of AIDS vaccines and the HIV Vaccine Trials Network (HVTN), an international network of sites for the conduct of clinical trials of candidate HIV/AIDS vaccines, was established. In FY 2001, a solicitation for additional HVDDTs was issued, additional contracts with industry for Vaccine Development Resources were awarded, a Cooperative Research and Development Agreement (CRADA) with Merck was established, vaccine related awards were issued under the Comprehensive International Program of Research on AIDS (CIPRA), and a phase II clinical trial started under the auspices of the HVTN.

  In FY 2002, NIH continued to foster national and international collaborations, including collaborations with industry. In FY 2002, an additional HVDDT contract was awarded to Wyeth-Lederle Vaccines to study HIV vaccines delivered using vesicular stomatitis virus vectors. Additional HVDDT awards will be made in FY 2003. In addition, the HVTN continued to expand the capabilities and capacity at international sites in FY 2002. The HVTN added additional sites in Puerto Rico, Brazil, Peru and South Africa and potential sites have been identified in the Dominican Republic, Honduras and Malawi. The CIPRA program continues to expand to help develop research infrastructure in developing countries. In FY 2002, CIPRA awards were made to support multi-disciplinary research projects in China (1) and South Africa (2). Smaller, planning and organizational grants were also awarded to institutions in Brazil, Vietnam, Dominican Republic, Cambodia, Thailand, India, Zambia, Mexico, Tanzania, Congo, and Zimbabwe.

  Annual performance reporting for FYs 2003 through 2007 will conform with the means of accounting for progress that is established for the new NIH research outcome goals.

Goals 1a – 9c) NIH GPRA Research Outcome Goals

The research outcome goals that NIH established for fiscal years 1999-2002 under the Government Performance and Results Act (GPRA) were intended to be comprehensive, that is, to encompass the totality of the NIH research portfolio. NIH's approach primarily used qualitative goals and thus relied on the "alternative form" provided under GPRA. Progress toward the qualitative goals was assessed via review of descriptions of science advances, stories of scientific discovery, and significant independent recognition of NIH supported investigators.

Last year, working with OMB and HHS, NIH began revising the comprehensive goals to add greater specificity in terms of desired outcome. Over the course of several meetings among OMB, HHS, and NIH staff, various approaches for articulating NIH research goals and reporting progress were considered. In Summer 2002, NIH developed a new approach to the NIH GPRA research outcome goals. Central to this new approach is a framework that characterizes goals on the basis of risk (likelihood of attaining the goal) and time, fiscal year 2003 and beyond. One way of visualizing this framework is to use a three-by-three matrix (see next page).

In this consolidated GPRA Plan/Report, NIH presents the goals that were developed within the new framework. The goals, a set of specific, representative research aims, were developed based on the following criteria, and are arrayed on the next page in the time/risk matrix.

- **Representative** – The goals will be a sampling of NIH aims that, as a set, represent the NIH mission. NIH has abandoned the previous approach of goals that, collectively, embody the NIH mission comprehensively.
- **Meaningful** - The goals must be credible to the research community, as well as the public and NIH stakeholders.
- **Specific** - Goals should be as specific to a disease or definable problem as possible, with reference to a metric and/or a date for progress/completion, as appropriate.
- **Objective or qualitative** - Objective goals are self-measuring, i.e., they permit a comparison between the actual achievement level and that targeted by the performance goal. If a goal is not self-measuring, i.e., it is qualitative, an independent assessment will have to be conducted in 3-5 years by outside experts.
- **Reportable** - Goals must lend themselves to annual reporting, regardless of whether the goal is objective or qualitative. Reports of incremental progress are fine.
- **Not obviously attainable** - The goal must be recognized as an outcome that *could* be achieved in the future, but may not be reachable for any number of reasons.

To the new goals, NIH is adding the current HIV/AIDS vaccine goal, reformatted to be consistent with the new specific/representative goals. Following presentation of the goals in the matrix format, background statements are provided for each of the goals. Next, NIH will develop plans for annual reporting and assessing performance. Those plans will involve the definition of milestones.

NIH GPRA Research Outcome Goals

RISK	1 – 3 years	4 – 6 years	7 –10 years
High	**1a** Conduct medications development with use of animal models, and begin to conduct Phase I and II trials of two potential treatments for alcoholism: cannabinoid antagonist Rimonabant and corticotropin-releasing hormone antagonist Antalarmin. **1b** By 2006, develop one or more prototypes for a low-power, highly directional hearing aid microphone to help hearing-impaired persons better understand speech in a noisy background.	**2a** By 2007, demonstrate the feasibility of islet transplantation in combination with immune tolerance induction for the treatment of type 1 diabetes in human clinical studies. **2b** By 2009, evaluate the efficacy of two novel approaches to prevent weight gain and/or treat obesity in clinical trials in humans. **2c** Develop methods that can classify at least 75% of proteins from sequenced genomes according to evolutionary origin and biological structure. **2d** By 2007, develop an HIV/AIDS vaccine.	**3a** Identify at least one clinical intervention that will delay the progression, delay the onset, or prevent Alzheimer's disease. **3b** By 2010, develop one universal antibiotic effective against multiple classes of biological pathogens. **3c** Determine the efficacy of using salivary diagnostics to monitor health and diagnose at least one systemic disease by 2013.
	4a By 2005, develop two new animal models to use in research on at least one agent of bioterror. **4b** By 2005, develop improved animal models that best recapitulate Parkinson's Disease (PD), based emerging scientific findings of genetic or environmental influences, or interactions of genes and the environment on the development of PD. **4c** By FY 2007, identify 20 small molecules that are active in models of nervous system function or disease and show promise as drugs, diagnostic agents, or research tools.	**5a** By 2007, evaluate the efficacy of three new treatments strategies for HIV infection in phase II/III human clinical trials in an effort to identify drugs that are more effective, less toxic, and/or simpler to use than the current recommended HIV treatment regimen. **5b** Establishing the efficacy of statins in preventing progression of atherosclerosis in children with Systemic Lupus Erythematosus (SLE or lupus). **5c** Expand the range of available methods used to create, analyze, and utilize chemical libraries, which can be used to discover new medicines. Specifically, use these chemical libraries to discover 10 new and unique chemical structures that could serve as the starting point for new drugs.	**6a**. Identify the genes that control the risk for the development of age-related macular degeneration and glaucoma in humans. **6b** By 2011, assess the efficacy of at least three new treatment strategies for reducing cardio-vascular morbidity/ mortality in patients with type 2 diabetes and/or chronic kidney disease. **6c** By 2012, develop a knowledge base on Chemical Effects in Biological Systems using a "systems toxicology" or toxicogenomics approach.
Low	**7a** By 2005, evaluate 10 commonly used botanicals for inhibition/induction of enzymes that metabolize drugs as a method of identifying potential botanical/drug interactions. **7b** By 2006, integrate nanotechnology-based components into a system capable of detecting specific biomarker(s) (molecular signatures) to establish proof of concept for a new approach to the early detection of cancer and, ultimately, cancer preemption. **7c** By 2005, create the next generation map of the human genome, a so called "haplotype map" (HapMap), by identifying the patterns of genetic variation across all human chromosomes.	**8a** By 2007, determine the genome sequence of an additional 45 human pathogens and three invertebrate vectors of infectious diseases. **8b** Identify and characterize two molecular interactions of potential clinical significance between bone-forming cells and components of bone. Such interactions are defined as those having significant impact on the accrual of bone mass or the actual mechanical performance of bone (i.e., fracture resistance) in laboratory animals. **8c** Build a publicly accessible Collection of Reference Sequences to serve as the basis for medical, functional, and diversity studies. A comprehensive Reference Sequence Collection will serve as a foundation for genomic research by providing a centralized, integrated, non-redundant set of sequences, including genomic DNA, transcript (RNA), and proteome (protein product) sequences, integrated with other vital information for all major research organisms.	**9a** By 2009, assess the impact of two major Institutional Development Award (IDeA) programs on the development of competitive investigators and their capacities to compete for NIH research funding. **9b** By 2010, demonstrate through research a capacity to reduce the total years lost to disability (YLDs) in the U.S. by 10 percent by 1) developing treatment algorithms to improve the management of treatment-resistant and recurrent depression and 2) elucidating the mechanisms by which depression influences at least two comorbid physical illnesses (e.g., heart disease, cancer, Parkinson's disease, or diabetes). Major depression is now the leading cause of YLDs in the nation. **9c** By FY 2010, identify culturally appropriate, effective stroke prevention programs for nationwide implementation in minority communities.

1a - GOAL STATEMENT: Conduct medications development with use of animal models and begin to conduct Phase I and II human trials of two potential treatments for alcoholism: the cannabinoid antagonist Rimonabant and the corticotropin-releasing-hormone antagonist Antalarmin.

***Prevalence/Incidence**:* The 2002 World Health Organization report lists alcohol as the third leading risk factor for preventable, premature death in developed countries, after tobacco and hypertension.[1] In the U.S., alcohol is the third leading root cause of death not attributable strictly to genetic factors, after tobacco and diet/activity patterns.[2] Almost 14 million American adults are alcoholic (physically dependent on alcohol) or abuse alcohol (dysfunctional, but not dependent).[3] Children also are at risk. Almost 30 percent of 9th - 12th graders report having five or more drinks, in a row, at least one day of the previous month.[4]

Disease Burden: Alcohol-use disorders cost U.S. society almost $185 billion each year, through injury, lost wages, property damage, death, and other factors.[5] Unlike other drugs of abuse, alcohol can have toxic effects on any organ in the body. Heavy alcohol use can cause brain damage, contributes to cardiovascular disease, and is a leading cause of liver cirrhosis and pancreatitis.[6] Alcohol is linked to some kinds of cancer.

Rationale: Alcoholism is a chronic disease subject to relapse; sustaining abstinence is the goal of treatment. However, current medications work for some people, but not others. Different factors contribute to abusive drinking and to subtypes of alcoholism. Some alcoholics have a genetic predisposition that affects specific brain systems, such as those regulating stress or rewarding sensations, resulting in molecular and cellular variations. Others are vulnerable to environmental stimuli. Developing more widely effective medications requires (1) understanding the different biological and environmental variations that underlie alcoholism, and targeting them, and (2) a wide array of candidate medications for testing. Animal models enabling us to test compounds in different biological and environmental scenarios are making this goal possible.

Two recently identified classes of compounds with treatment potential are Antalarmin and Rimonabant). By blocking a brain-cell receptor (CRH1) for a hormone that elicits anxiety in response to stress, Antalarmin reduced drinking in monkeys going through alcohol withdrawal. Rimonabant blocks another receptor (CB1) that otherwise would stimulate biological pathways in specific areas of the brain that result in rewarding sensations. In mice, this medication reduced drinking by young animals. We need to continue to cast a wide net to identify compounds with therapeutic potential for the different subtypes of alcoholism. This involves identifying molecular targets and new and existing compounds that act on them, conducting screenings that predict the utility of these compounds, and confirming their utility with animal and human studies.

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[1] World Health Organization. *World Health Report 2002: Reducing Risks, Promoting Healthy Life*. http://www.who.int/whr/en/
[2] McGinnis JM and Foege WH. *Actual Causes of Death in the United States*, JAMA, Nov. 10, 1993, Vol 270, No. 18, p. 2208.
[3] BF Grant et al. *Prevalence of DSM-IV alcohol abuse and dependence -- United States, 1992*. NIAAA=s Epidemiologic Bulletin No. 35 18:243-248, 1994.
[4] Centers for Disease Control and Prevention. *Youth 2001*, http://www.cdc.gov/nccdphp/dash/yrbs/2001/youth01online.htm; *Youth Risk Behavior Survey*, CD-ROM Youth '99, and *Youth Risk Behavior Survey*, CD-ROM Youth '97.
[5] Harwood et al. Update of *The economic costs of alcohol and drug abuse in the United States,1992*. NIH Publication No. 98-4327 1-9, 1998. Updated October 1999.
[6] Smart RG and Mann RE. *Alcohol and the epidemiology of liver cirrhosis*. Alcohol Health & Research World 16(3):217-222, 1991.

1b - GOAL STATEMENT: By 2006, develop one or more prototypes for a low-power, highly directional hearing aid microphone to help hearing-impaired persons better understand speech in a noisy background.

***Prevalence/Incidence*:** Approximately 20 million Americans are estimated to have sensorineural hearing loss, making this one of the most prevalent disabling conditions in the United States. Hearing loss can be hereditary, or it can result from disease, trauma, or long-term exposure to damaging noise or medications. The condition can vary from a mild but important loss of sensitivity, to a total loss of hearing.

Disease Burden: Sensorineural hearing loss affects people of all ages, in all segments of the population, and across all socioeconomic levels. It can harm an individual's physical, cognitive, behavioral, and social function and is caused by a problem in the cochlea or the auditory nerve; parts of the ear that help sound impulses reach the brain. Hearing aids are the main form of treatment for this condition, however only 20 percent of those who could benefit from hearing aids actually use them.[1]

Rationale: A hearing aid is a battery-operated device that amplifies and changes sound to allow for improved communication. Hearing aids receive sound through a microphone, which then converts the sound waves to electrical signals. The amplifier increases the loudness of the signals and then sends the sound to the ear through a speaker. A vast array of hearing aid technology is available, ranging from simple and relatively inexpensive analog circuits to complex and expensive digital devices that require sophisticated fitting procedures.

Although hearing aid technology has advanced rapidly over the last few decades with the development of microelectronic components, the various hearing aids currently available still do not function well when sound from more than one source is present. Most hearing aids are designed for compensating for high-frequency hearing loss and for suppressing static noise in a room. However, hearing aids are not particularly effective in restoring the listener's ability to sort a single speech sound among competing sources (as in meetings, banquets and sporting events).

NIH-supported scientists have been studying a tiny fly, *Ormia ochracea*, with such acute directional hearing that it has inspired ideas for a new generation of hearing aids. The biological lessons provided by this fly's abilities in hyper acute time coding and localization of sound provide strategies for improved nano/micro-scale directional microphones in hearing aids that would focus sound amplification on speech. Applications of these new principles may improve the quality of life for individuals with hearing loss who depend upon hearing aids to understand spoken language.

[1] Larson V, Williams D, et al: Efficacy of 3 Commonly Used Hearing Aid Circuits. JAMA 248: 1806-1813, 2000.

2a - GOAL STATEMENT: By 2007, demonstrate the feasibility of islet transplantation in combination with immune tolerance induction for the treatment of type 1 diabetes in human clinical studies.

***Prevalence/Incidence*:** Type 1 diabetes is an autoimmune disease in which the immune system attacks and destroys the insulin-producing islet cells of the pancreas.

- ~300,000-500,000 Americans have type 1 diabetes; ~120,000 are <20 years of age, making this one of the most common chronic diseases of childhood.[1]
- ~30,000 new cases occur each year, the majority with onset in early childhood and teens; ~1 in 300 cases of diabetes with onset in adulthood is autoimmune in origin.[2]

Disease Burden: Type 1 diabetes is a chronic, life-long disease characterized by elevations in blood sugar that, over time, lead to severe and life-threatening complications, including: heart disease; blindness; peripheral neuropathy; foot ulcers; and kidney failure. Treatment requires insulin administration through multiple daily insulin injections or use of an insulin pump and careful attention to diet and activity; blood sugar levels must be measured several times a day by finger pricks. However, even with careful attention to insulin dosing, even the most medically compliant patients are rarely able to maintain "tight" or physiologic control of their blood sugar. As a result, existing treatments can delay and diminish, but not prevent, many of the complications of diabetes. Even with careful attention to control of blood sugar, type 1 diabetes results in a reduction in quality of life and shortens life-span by ~15 years.

Rationale: Whole pancreas and pancreatic islet transplants offers type 1 diabetics the potential for physiologic control of blood sugar as an alternative to insulin therapy. Whole pancreas transplantation is a technically difficult procedure, while pancreatic islet cell transplantation is a minimally invasive procedure. In islet transplantation, cells from the pancreas called "islets" are isolated from a donor pancreas and injected into a large blood vessel that supplies the liver. The transplanted islets lodge in the liver where they produce insulin. Until recently, the intermediate and long-term success of this procedure has been disappointing: of the more than 300 islet transplants performed over a decade, fewer than 10% remained insulin-independent one year after the procedure. However, recent advances in pancreatic islet cell preparation and improvements in immunosuppressive regimens that are required to prevent transplant rejection have dramatically improved the outcome of islet transplantation. As a result, approximately 70-80% of type 1 diabetics can be expected to remain insulin-independent two years following islet transplantation. Despite these advances, patients must remain on potent immunosuppressive drugs to prevent immune-mediated rejection of the transplanted islet cells. Immunosuppressive agents may increase the risk of serious infection and other complications, such as hypertension and cardiovascular disease.

The successful induction of immune tolerance is a major therapeutic goal for the treatment of many immune-mediated diseases, including autoimmune disorders such as type 1 diabetes. Research is underway to develop selective, short-term and durable therapies that will eliminate the pathogenic immune responses, such as graft rejection and autoimmune injury, while preserving protective immunity. Tolerance induction holds great potential for improving the quality of life for those individuals afflicted by type 1 diabetes and other immune-mediated diseases. If successful, tolerance induction would: 1) enable life-long rejection-free maintenance of islet cells; and 2) eliminate ongoing autoimmune injury to transplanted islets without the many adverse effects of broadly immunosuppressive drugs.

[1] Diabetes in America, 2nd edition, 1995; NIH publication No. 95-1468, page 1.
[2] Diabetes in America, 2nd edition, 1995; NIH publication No. 95-1468, page 40.

2b - GOAL STATEMENT: By 2009, evaluate the efficacy of two novel approaches to prevent weight gain and/or treat obesity in clinical trials in humans.

Prevalence: The number of overweight and obese Americans has risen dramatically in the past two decades and is now at epidemic levels.

- Over 60 percent of adults are overweight; of these, approximately 31 percent are obese.[1]
- About 15 percent of children and teens ages 6-19 are overweight,[2] with ominous implications for our Nation's future health.
- Racial and ethnic minority populations are disproportionately affected by obesity, particularly African American, Hispanic, and Native American women and children.

Disease Burden: Obesity is associated with numerous serious diseases, including type 2 diabetes, heart disease, stroke, osteoarthritis, gallstones, breathing problems, and cancer. Type 2 diabetes, formerly viewed as a disease of adults, has been increasingly reported in children. This alarming trend is thought to be a consequence of increased obesity along with decreased physical activity. In addition to the negative impact on quality of life and the increased risk of premature death, overweight and obesity exact enormous economic costs. In 2000, costs associated with obesity were estimated to be $117 billion.[3]

Rationale: Overweight and obesity develop when energy intake (food calories) exceeds energy expenditure. While genetic factors may contribute substantially to the predisposition towards obesity, the recent dramatic increase in obesity prevalence is clearly fueled by environmental and behavioral changes interacting with genetic susceptibility. Results from the NIH-funded Diabetes Prevention Program clinical trial demonstrated a substantially reduced incidence of type 2 diabetes in a high risk population using an intervention that combined moderate weight loss and exercise; however, these modest lifestyle changes required intensive individual behavioral intervention. Thus, the goal of obesity prevention may benefit greatly from new approaches to modify factors pervasive in the environment that promote over-consumption of food and sedentary lifestyles, complemented by further research on strategies to help individuals achieve and maintain behavior changes. For people who are extremely obese, expected weight loss from behavior change alone may not be sufficient to have a major impact on health. Bariatric surgical procedures that restrict stomach size are being increasingly performed to treat severe obesity and can have dramatic benefits, but also carry substantial risks. Accelerated clinical research on this surgery will enhance patient evaluation, selection, and follow-up care. Finally, the continued elucidation of the molecular factors and pathways responsible for appetite regulation, metabolism, and energy storage offers rich prospects for the development of new drugs that will promote safe and effective long-term weight loss. A major goal of NIH-funded research is to develop and evaluate strategies to prevent obesity and to promote sustained weight loss in individuals who are overweight or obese. In addition to mechanisms falling within the three broad approaches to weight regulation just described, evaluation of other as yet unknown strategies may also be necessary in order to achieve success in meeting the goal. If successful, the approaches would decrease risk for the life-threatening diseases that accompany excess weight and would also reduce the social and economic costs of obesity.

[1] Flegal et al., JAMA 288: 1723-7, 2002.
[2] Ogden et al., JAMA 288: 1728-32, 2002.
[3] The Surgeon General's Call to Action to Prevent and Decrease Overweight and Obesity, 2001, p. 10.

2c - GOAL STATEMENT: Develop methods that can classify at least 75% of proteins from sequenced genomes according to evolutionary origin and biological structure.

Background: Classification of domains by computational sequence analysis is a powerful means to deduce the function of newly discovered proteins. In the context of proteins associated with human disease, this analysis can generate hypotheses concerning the metabolic pathways in which the proteins act and greatly accelerate research into the molecular basis of disease and therapy. Domain analysis identifies regions of high sequence similarity with respect to other proteins from a variety of organisms. Conserved domains, as these regions are called, have been shown to be fundamental units of biological function; they adopt similar 3-dimensional structures and interact with other molecular components of living cells in similar ways. A comprehensive domain database, searchable over the internet, will therefore be a powerful research tool for academic and industrial scientists with diverse interests.

Rationale: A comprehensive database is achievable because proteins contain only a few thousand domain families. Maintaining a collection up to date with respect to current knowledge nonetheless represents a challenge that can be met only by development of new methods for large-scale comparative analysis of molecular data, which allow curators to focus on functional annotation. The continuing investment of Federal agencies and other organizations in genome sequencing and structural genomics will yield the greatest return when combined with efforts to organize this data in useful ways. The conserved domain database anticipated here represents an advance over previous efforts because it will apply structure-based alignment and molecular evolutionary classification in a systematic and ongoing manner.

This resource will be particularly valuable to researchers such as medicinal chemists who require a synthesis of information on protein biological function, 3-dimensional structure, and sequence conservation. Effective anti-viral drugs have been designed by targeting the conserved regions of viral proteins, for example; the virus is unable to develop resistance to these drugs because sequence changes that block drug binding also block the normal function of the protein. By describing conserved regions in detail, the resource proposed here provides information that is directly useful to the medicinal chemist undertaking this research.

2d - GOAL STATEMENT: By 2007, develop a HIV/AIDS vaccine.

Incidence/Prevalence: Globally, over 40 million people (37.1 million adults and 3 million children) were living with AIDS at the end of 2001. In 2001, 3 million people died from AIDS, and 5 million people were newly infected with HIV. Of the 5 million new infections, 800,000 were in children. More than 95 percent of new HIV infections occur in the developing world, with 70 percent occurring in sub-Saharan Africa and 20 percent in Asia and the Pacific. Most of these new infections are in young adults, with an increasing number among women. In the United States, close to 950,000 people are living with HIV/AIDS, and each year 40,000 new infections occur, of which more than one-half are in individuals younger than 25 years of age.[1,2]

Disease Burden: AIDS is caused by the human immunodeficiency virus. Infection with the virus leads to destruction of a person's immune system, making the victim highly susceptible to multiple infections and certain cancers. AIDS is a fatal disorder.

Rationale: Significant progress has been made in HIV/AIDS research since 1981 when AIDS first emerged as a global infectious disease. Research has led to a better understanding of the structure of HIV and how HIV attacks the immune system, and the role of the immune system in controlling HIV infection. Potent therapeutic regimens commonly referred to as HAART, have been successful in suppressing HIV to virtually undetectable levels in the blood and in decreasing the incidence of opportunistic infections. HAART has greatly improved the quality of life of many HIV-infected individuals and has led to a dramatic decline in HIV-infected people in the United States. Educational and counseling efforts have had some success and remain essential, however, it has become evident that these prevention activities alone are not sufficient to contain the spread of the disease. Despite these advances, the HIV pandemic continues to rage around the world.

The development of a safe and effective vaccine against HIV is critical to worldwide efforts to control HIV/AIDS, and offers the best hope for halting the HIV/AIDS pandemic. An HIV vaccine that is simple to administer, inexpensive, and induces long-lasting immunity against most HIV sub-types is the ideal prevention strategy.

Since the beginning of the epidemic, NIH's comprehensive research program has made significant progress in elucidating the structure of HIV and how HIV attacks the immune system, understanding the role of the immune system in controlling HIV, developing new and improved models for testing candidate vaccines, and in sponsoring and conducting clinical trials. NIH's HIV vaccine program supports research on novel vaccine concepts, genetic and immunologic variation, mucosal immunity, delivery methods, adjuvants, and correlates of immune protection. At the preclinical level, NIH's programs include support for the development of new candidate vaccine designs, evaluation in non-human primate models of HIV/AIDS, pilot-lot production of new candidate vaccines, and additional preclinical work required for the advancement into human trials. At the clinical level, the program coordinates all phases of clinical trials of candidate HIV vaccines and supports work to characterize potentially protective immune responses in vaccinated volunteers.

[1] CDC, HIV/AIDS Surveillance Report, 13 (No. 2):1-44, 2001
[2] UNAIDS Report on the Global HIV/AIDS Epidemic, December 2001

3a - GOAL STATEMENT: Identify at least one clinical intervention that will delay the progression, delay the onset, or prevent Alzheimer's disease.

Prevalence/Incidence: Alzheimer's disease (AD) is a progressive, at present irreversible, brain disease that slowly destroys memory and thinking skills, eventually even the ability to carry out the simplest tasks of daily living.

- A consensus statement developed by the American Association for Geriatric Psychiatry, the Alzheimer's Association, and the American Geriatrics Society estimates the number of AD cases at 4 million nationally, and still concludes that Alzheimer's disease and related dementias are under diagnosed.[1]
- The prevalence of the disease doubles each 5 years over the age of 65.
- It is estimated that the prevalence of AD will nearly quadruple in the next 50 years.[2]

Disease Burden: The cost of AD care appears to vary by the stage of the disease. In 1996, annual costs of caring for patients with mild, moderate, and severe Alzheimer's disease were $18,408, $30,096, and $36,132, respectively.[2] The national cost of caring for people with AD is now thought to be about $100 billion every year.[3]

Rationale: In 1999, at the direction of Congress, the National Institute on Aging (NIA), in conjunction with the National Institute of Neurological Disorders and Stroke (NINDS), the National Institute of Mental Health (NIMH), and the National Institute of Nursing Research (NINR) embarked on the Alzheimer's Disease Prevention Initiative. A major focus of this initiative is on accelerating movement of promising new treatments and prevention strategies into clinical trials.

Advances in genetic, molecular and epidemiological research have increased our understanding of the biologic processes involved in the onset and progression of AD and provided important opportunities to test promising new interventions. Clinical research is rapidly developing ways of identifying persons early in the course of AD and better ways of predicting and following the progression of the disease. Ongoing clinical trials and those in the planning stages are focusing on specific biologic processes including inflammation, free radical accumulation, amyloid deposition, and cell death that scientists believe are among the first changes to appear in the brains of patients with AD. Completing these long-term human trials will identify ways that specific drugs can be used to most safely and effectively intervene in order to delay the progression, delay the onset, or prevent Alzheimer's disease.

[1] Small, GW et al. *JAMA* 16: 1363-1371, 1997.
[2] Brookmeyer, R, et al. *American Journal of Public Health* 88: 1337-1342,1998.
[2] Leon J, et al. *Health Affairs* 17(6): 206-216, 1998.
[3] Ernst, RL, Hay, JW, Fenn, C, Tinklenberg, J, and Yesavage, J, *Arch Neurol* 54: 687-693, 1997.

3b - GOAL STATEMENT: By 2010, develop one universal antibiotic effective against multiple classes of biological pathogens.

Background: In the 1940's, the widespread availability of newly discovered antibiotics led to a dramatic reduction in illness and death from infectious diseases. However, bacteria and other disease-causing organisms are remarkably resilient and have developed mechanisms of resistance that thwart or block the action of antimicrobial drugs. Microbes that were once easily controlled by antimicrobial drugs are causing infections that no longer respond to treatment with these drugs. In addition, new, frightening, and unforeseen infectious disease threats have emerged, including threats posed by agents of bioterrorism. The threat of these infectious agents coupled with the emergence of antimicrobial resistance is a grim and foreboding reminder of the power, destructiveness, and endless adaptability of infectious microbes and the global importance of research to treat these infections. A "universal antibiotic," a drug effective against a wide spectrum of infectious diseases, would help address these challenges.

Rationale: The U.S. government's ability to detect and counter a biological attack requires basic research aimed at understanding both the organisms that might be used as agents of bioterrorism and how the human immune system responds to those organisms. The National Institute of Allergy and Infectious Diseases (NIAID) has developed a comprehensive strategic plan and detailed research agendas (http://www.niaid.nih.gov/dmid/bioterrorism/) for the Category A, B, and C biological pathogens considered by the CDC to be the most serious bioterror threats.

Several of these pathogens are bacteria that can persist and grow within host cells and present unique challenges to biodefense researchers. The recent NIAID Blue Ribbon Panel on Bioterrorism and Its Implications for Biomedical Research on the Category B/C Agents stressed the need for more research on intracellular bacterial infections (infections within the cell) and approaches to their treatment and prevention. The bacterial mechanisms required for intracellular survival and the cellular changes induced in response to infection remain poorly understood. Therefore, characterizing the genetic and biochemical requirements of intracellular infections could lead to new therapeutic targets such as one universal antibiotic effective against multiple classes of bacterial/biological pathogens.

NIH scientists are working to decipher the basic mechanisms of non-specific (or innate) host defense against microbes. Understanding the molecular events that constitute the generic immune response to microbes that are "new" to the immune system, that is, those previously unseen via vaccination or prior infection, is key to the development of novel therapeutics that will be effective against multiple pathogens.

3c - GOAL STATEMENT: Determine the efficacy of using salivary diagnostics to monitor health and diagnose at least one systemic disease by 2013.

***Background**:* For many serious health conditions, early detection offers the best hope for cure. However, many individuals obtain a correct diagnosis only after they experience symptoms--and then it may be too late. The composition of saliva and other oral fluids reflects serum levels of substances that may be useful for diagnostic applications—such as therapeutic and recreational drugs, hormones, immunoglobulins, and toxic molecules. Oral fluids can also be used as a source of host or pathogen DNA. Thus, oral fluids could potentially be used to assess and monitor systemic health and disease, as well as determine exposure to environmental and occupational hazards. Real-time monitoring of oral fluids may also have a role in biodefense by facilitating early detection of agents used in bioterrorism.

Rationale: Saliva is easy to collect, and poses none of the risk, fears, or "invasiveness" concerns occasioned by blood tests or urine sampling. Miniaturization of the "lab on a chip" may allow placement of the detection device directly in the mouth, making sample collection unnecessary. However, because oral levels of most analytes are lower than blood levels, sensitive analytical techniques are required. (Analytes are any substance or chemical constituent of a body fluid that is analyzed.) To overcome this challenge and demonstrate the feasibility of salivary diagnostic tools, the NIH is taking steps to accelerate the technology needed to analyze oral fluids. This effort will require highly sensitive and accurate methods for the rapid detection of informative analytes in saliva, thus indicating the early stages of emerging disease. Our goal is to determine the efficacy of using salivary diagnostics to monitor health and diagnose at least one systemic disease by 2013. However, if successful, this line of research could yield improved detection for a number of diseases, as well as dramatically reducing the cost and risk associated with blood test based diagnostics. This would catalyze a shift of our current system of disease detection to one of health surveillance within the community or home.

4a - GOAL STATEMENT: By 2005, develop two new animal models to use in research on at least one agent of bioterror.

Background: Deliberate exposure of the civilian population of the United States to *Bacillus anthracis* (anthrax) spores revealed a gap in the nation's overall preparedness against bioterrorism. These attacks uncovered a need for tests to rapidly diagnose, vaccines and immunotherapies to prevent, and drugs and biologics to cure disease caused by agents of bioterrorism. The lack of routine clinical importance, and thus the absence of scientific and clinical expertise associated with a microbe, is a hallmark of a successful bioterrorist agent. The development of centralized sources of generalized as well as specific expertise in bioterrorism areas will be required to speed the development of new generation products. The NIAID Strategic Plan for Biodefense Research offers more detailed information on the types of biodefense research supported by NIH, including specific goals for each research category.

Rationale: New products and ideas must be thoroughly tested in the laboratory to ensure that they are safe and that they work. For example, scientists conduct tests in artificial environments (*in vitro*) and in animals when they develop and test vaccines, therapeutics, and diagnostics. In addition, safety testing in the lab is required to speed the development of new generation products. *In vitro* and animal models provide information that can be used to move the processes that occur in the laboratory to humans. In the field of biodefense research, animal models will be critical to FDA approval of therapies and vaccines, since, in most cases, clinical trials in humans to test efficacy are not possible or ethical. A number of promising candidate therapies and vaccines have been identified for bioterrorism organisms/diseases. However, development has been delayed because of the lack of standardized animal models in which to evaluate these candidates. New models need to be developed, particularly for non-human primates. The development of many infectious diseases and the response to therapy in non-human primates are similar to the human response (the rhesus macaque, a type of monkey, is particularly useful in medical research). However, the use of non-human primates is limited by their cost and difficulty in acquiring and maintaining them. The shortage of rhesus macaques is severely limiting development of new vaccines and therapies. Given the current level of interest in developing additional therapeutic and prevention strategies, particularly for organisms with potential use in bioterrorism, NIH must expand current resources, including animal models that are not based on non-human primates, for therapeutics and vaccine development.

In addition, NIH's biodefense and emerging infectious diseases research opportunities include support for developing animal models to: understand disease-causing mechanisms and pathogen/host interactions; define the body's natural and learned protective immune mechanisms, and study vaccines, diagnosis and treatment regimens for pathogens; define how these infections impact the immune system; determine the ways that these infections have adapted to avoid detection by immune cells; and study mechanisms of vaccination adverse events, including those in at-risk populations, methods for avoiding the introduction of adventitious agents during vaccine manufacture, and novel methods of vaccine production to enhance vaccine safety.

4b - GOAL STATEMENT: By 2005, develop improved animal models that best recapitulate Parkinson's disease (PD), based on emerging scientific findings of genetic or environmental influences, or interactions of genes and the environment on the development of PD.

Prevalence/Incidence: PD is a neurodegenerative disease for which there is no known cure.

- Incidence: 50,000 cases per year[1], increases dramatically after age 50
- Prevalence: estimates range from 500,000[1] to 1 million individuals in the U.S.[2]

Disease Burden: Parkinson's disease is a devastating, progressive motor disorder, characterized by rigidity, poor balance, and uncontrollable shaking or tremors; those affected by PD eventually lose their independence. PD is marked by a loss of neurons that produce the neurotransmitter dopamine, which are an essential part of the brain pathways controlling purposeful movement. The total economic cost per year was estimated to be $6 billion in 1992.[3] Most individuals with PD are treated with pharmacologic agents that mimic the actions of the lost dopamine. Although these drugs provide symptomatic relief, they do not cure or slow disease progression, are of limited benefit in later stages of the disease, and can produce undesirable side effects.

Rationale: In order to facilitate the understanding and treatment of any human disease, it is desirable to create animal models that precisely recapitulate the disease process, including pathways of disease causation and the impact of the disease on cellular processes, organ function, and ultimately, behavior. With such models in hand, researchers could track the earliest molecular events in the disease and develop intervention strategies to delay, or even prevent its progression. In the case of PD, researchers would *ideally* like to have access to an inexpensive, reproducible animal model that captures both the genetic and environmental roles in causation; reproduces the cellular changes that occur in PD over an appropriate period of time; and leads to behaviors in the animal that approximate the effects of the disease on humans.

Over the years, the research community has developed several animal models of PD that have been instrumental in accelerating our understanding of the disease process[4]. One such model is produced through the exposure of primates to MPTP (1-methyl-4-phenyl-1,2,5,6-tetrahydropyridine), a chemical substance with structural similarities to some pesticides. Although this model is likely to remain useful for predicting therapeutic efficacy, it is costly, and does not reproduce some key features of PD (e.g., the progressive nature of the disease, some cellular features of affected neurons, and the combined effects of the environment and genes on disease causation). By contrast, non-primate models have offered important practical benefits for dissecting gene-environment interactions in PD. For example, the creation of mice and fruit flies expressing mutant forms of a gene (alpha-synuclein) implicated in PD have provided an opportunity for studying the effects of environmental agents on key genes and proteins involved in the disease process. Further, the recent discovery that pesticide exposures (e.g., rotenone) can produce parkinson-like effects on neurons and behavior in rodents offers another possible strategy for understanding the effects of the environment on this disease.

Together, these models have enabled researchers to learn a great deal about the neural systems that are affected by PD, the molecules within cells that may play a role in the disease process, and the potential for various therapies to treat the disorder. However, each has its merits and limitations, and an optimal model is still not available to the PD research community[5]. For this reason, a collaborative effort will be

[1] NIH Cost of Illness Report, 2000, p. 98.
[2] Herndon, CM, et al. Parkinson's disease revisited. J. Neurosci. Nurs. 2000; 32(4): 216-221.
[3] NIH Cost of Illness Report, 2000, p. 98.
[4] Greenamyre JT, Animal models of Parkinson's disease. Bioessays 2002 Apr;24(4):308-18
[5] Beal, MF, Experimental models of Parkinson's disease. Nature Reviews Neuroscience. 2001; 2: 325-332.

needed in the future to capitalize on findings related to environmental and genetic influences on PD, and to develop this knowledge into inexpensive, reproducible animal models of PD that simulate the disease process even more accurately than do the models that are currently available, and improve our ability to test therapies.

4c - GOAL STATEMENT: By fiscal year 2007, identify 20 small molecules that are active in models of nervous system function or disease and show promise as drugs, diagnostic agents, or research tools.

Disease Burden: Diseases of the nervous system–stroke, trauma, drug addiction, alcoholism, autism, unipolar major depression, epilepsy, Parkinson's disease, schizophrenia, multiple sclerosis, chronic pain, and hundreds more–collectively constitute one of the largest disease burdens in terms of disability, costs, personal tragedy, and death.

Rationale*:* Identification of small molecules with promise as drugs, diagnostic agents, and research tools is critical for the development of new or improved treatments for diseases of the nervous system. Recent advances in understanding the nervous system and its disorders at the level of cells and molecules have revealed new targets for drug development, that is steps in the disease process at which a drug might act with a beneficial effect. In addition, the development of cell culture and animal models of human disease has greatly facilitated the testing of potential drugs. We can now evaluate large collections of molecules, which will provide an increasing number of promising candidates for further development as drugs, diagnostic agents, and research tools. Although other exciting approaches are being explored, for the immediate future small molecules will continue to constitute the vast majority of new therapies and tools for treating, diagnosing, and studying disorders of the nervous system.

5a - GOAL STATEMENT: By 2007, evaluate the efficacy of three new treatments strategies for HIV infection in phase II/III human clinical trials in an effort to identify drugs that are more effective, less toxic, and/or simpler to use than the current recommended HIV treatment regimen.

Prevalence/Incidence: Globally, over 40 million people (37.1 million adults and 3 million children) were living with AIDS at the end of 2001. In 2001, 3 million people died from AIDS, and 5 million people were newly infected with HIV. Of the 5 million new infections, 800,000 were in children. More than 95 percent of new HIV infections occur in the developing world, with 70 percent occurring in sub-Saharan Africa and 20 percent in Asia and the Pacific. Most of these new infections are in young adults, with an increasing number among women. In the United States, close to 950,000 people are living with HIV/AIDS, and each year 40,000 new infections occur, of which more than one-half are in individuals younger than 25 years of age.[1,2]

Disease Burden: AIDS is caused by the human immunodeficiency virus. Infection with the virus leads to destruction of a person's immune system, making the victim highly susceptible to multiple infections and certain cancers. AIDS is a fatal disorder.

Rationale: Over the past 13 years, efforts to develop drugs to treat HIV infection and/or AIDS have mainly concentrated on developing inhibitors of two important enzymes in the lifecycle of the virus, namely reverse transcriptase (RT) and protease (PR). When used in combination, RT and PR inhibitors (known as highly active antiretroviral therapy, or HAART) have been successful in decreasing the amount of HIV in the blood of many infected individuals to undetectable levels, decreasing the incidence of opportunistic infections and decreasing the number of AIDS-related deaths in the developed world. Nonetheless, complications have emerged with the use of these drugs, including the development of drug resistance, metabolic abnormalities and toxicities, and noncompliance due to the complexity of these regimens. Moreover, damage to the immune system is only partially repaired by currently available antiretroviral therapy. Thus, there remains an urgent need for the discovery and development of new classes of antiviral drugs for the treatment of HIV infection and AIDS that are not only less toxic and simple to use, but that are affordable and available for worldwide use.

Recently, research has identified several new classes of potential drugs to treat HIV infection which are in the early stages of development. Examples of these new classes of drugs include agents that interfere with other stages of the virus lifecycle, such as the initial attachment of the virus to the host cell and the entry of the virus into the cell. Another example includes drugs that interfere with other key steps in the virus' lifecycle, such as when HIV uses its integrase enzyme to insert its own genetic material into a host cell's DNA. Both entry inhibitors and inhibition of HIV integrase are attractive therapeutic strategies, since both would potentially protect healthy cells from infection, thereby helping to bolster the immune system. Further, there may be significant synergy when these agents are used in combination. NIH will continue to support the basic, preclinical and clinical development of novel therapeutics, including the identification of new host and viral targets, novel drugs and delivery systems, and immunological approaches to address the dual problems of drug resistance and toxicity. In addition to new therapeutic agents, there is a need to develop and test therapeutic vaccines for HIV/AIDS. The goal of a therapeutic vaccine is to enhance the ability of the immune system to fight HIV infection and prevent or delay the onset of AIDS (in contrast to a vaccine which prevents HIV infection). Since there are known and well defined immune defects caused by HIV, it might be possible to design vaccines, which in conjunction with HAART, can rebuild the immune system and establish a fully efficient host immune response that controls the virus. These types of strategies would have a profound impact domestically as well as in the developing world.

[1,2] CDC, HIV/AIDS Surveillance Report, 13 (no. 2):1-44, 2001 UNAIDS Report on the Global HIV/AIDS Epidemic, December 2001

5b - GOAL STATEMENT: Establishing the efficacy of statins in preventing progression of atherosclerosis in children with Systemic Lupus Erythematosus (SLE or lupus).

Prevalence/incidence: It is difficult to estimate how many children in the United States have lupus because its symptoms vary widely and its onset is often hard to pinpoint. Lupus is three times more common in African American women than in Caucasian women and is also more common in women of Hispanic, Asian, and Native American descent.[1]

***Disease Burden*:** Lupus is a disorder of the immune system known as an autoimmune disease. In autoimmune diseases, the body harms its own healthy cells and tissues, leading to inflammation and damage to various body tissues. Lupus can affect many parts of the body, including the joints, skin, kidneys, heart, lungs, blood vessels, and brain. Although people with the disease may have many different symptoms, some of the most common ones include extreme fatigue, painful or swollen joints (arthritis), unexplained fever, skin rashes, and kidney problems.

Lupus is a complex disease whose cause is unknown. It is likely that there is no single cause but rather a combination of genetic, environmental, and possibly hormonal factors that work together to cause the disease. Scientists are making progress in understanding the processes leading to lupus. Age at disease onset is a predictor of outcome, and children often have severe end organ disease. At present, there is no cure for lupus. Lupus is the focus of intense research as scientists try to determine what causes the disease and how it can be best treated.

***Rationale*:** Atherosclerosis is a thickening of the inside walls of arteries that is caused by the gradual buildup of fatty substances in arteries. This thickening narrows the space through which blood can flow, and can result in heart attacks or strokes. Atherosclerosis usually occurs when a person has high levels of cholesterol (a fat-like substance), which can build up on the walls of arteries. We know that women with lupus have a significantly increased risk for cardiovascular complications related to premature atherosclerosis, and children do as well. The data on cardiovascular and lipid abnormalities in children with lupus implicate atherosclerosis as an important potential source of long-term morbidity and mortality. Statins are drugs that lower cholesterol in blood and decrease the risk for atherosclerosis and cardiovascular disease. Not only do statins decrease mortality and morbidity from coronary artery disease in adults, but they also have intrinsic anti-inflammatory properties, which may be especially beneficial in lupus.

[1] Reva C. Lawrence, Charles G. Helmick, Frank C. Arnett, Richard A. Deyo, David T. Felson, Edward H. Giannini, Stephen P. Heyse, Rosemarie Hirsch, Marc C. Hochberg, Gene D. Hunder, Matthew H. Liang, Stanley R. Pillemer, Virginia D. Steen, and Frederick Wolfe; Arthritis & Rheumatism, Volume 41, Number 5, May 1998, pages 778 to 799.

5c - GOAL STATEMENT Expand the range of available methods used to create, analyze, and utilize chemical libraries, which can be used to discover new medicines. Specifically, use these chemical libraries to discover 10 new and unique chemical structures that could serve as the starting point for new drugs.

Background: Our nation is facing a pressing need for new drugs. Many existing medicines are becoming ineffective due to antibiotic resistance. In other cases, the side effects of existing drugs are as severe as the diseases they are designed to treat. Meanwhile, the number of new drug applications submitted in recent years has dropped precipitously, according to the Food and Drug Administration.[1] Most drugs are discovered by randomly screening thousands of chemical compounds for desired biological effects. To speed the discovery of new medicines, scientists need to have access to larger collections of chemicals to test. An especially promising approach to invigorating and strengthening the new drug pipeline is by using a new and powerful chemical strategy called diversity-oriented synthesis. This method can quickly generate a large number of potential drug compounds (a "chemical library"). Such a library could contain anywhere from a few chemical compounds to millions, and can be designed to include either related versions of a single molecule or a wide variety of completely new chemical structures. This new technique offers unprecedented opportunities for the discovery of molecules that may be developed into lifesaving drugs.

Rationale: Since diversity-oriented synthesis is such a new and intellectually challenging endeavor, the number of methods for designing, making, and analyzing chemical libraries is still limited. This restricts the variety of structures that chemists can make. Although the pharmaceutical industry has embraced chemical library screening as a useful drug discovery strategy, it has not invested in the long-term research needed to improve the technique. Similarly, few academic scientists have made a special effort to develop chemical library-related methods. The investment will likely enrich the field of diversity-oriented synthesis and give pharmaceutical scientists important tools for discovery of molecules that show promise as future medicines.

[1] U.S. Food and Drug Administration (2002). FY 2001 Performance Report to Congress for the Prescription Free Drug User Act of 1992 [Online]. Available: http://www.fda.gov/oc/pdufa/report2001/pdufareport.html

6a - GOAL STATEMENT: Identify the genes that control the risk for the development of age-related macular degeneration and glaucoma in humans.

Prevalence/Incidence: Age-related macular degeneration is a sight-threatening degenerative eye disease that affects the part of the retina known as the macula and leads to varying degrees of vision loss depending on the form and severity of the disease. Of the nearly 60 million people in the United States age 55 or older in the year 2000[1], approximately eight million are at risk of developing advanced, sight-threatening age-related macular degeneration in one or both eyes within five years.[2] Glaucoma is a group of eye disorders that shares a distinct type of optic nerve damage that can lead to blindness. Approximately 2.2 million Americans have glaucoma[3] and an estimated 2 million more are unaware that they have the disease. As many as 120,000 are blind from this disease.[4]

Disease Burden: Age-related macular degeneration (AMD) is the leading cause of irreversible vision loss in the United States among persons over 65 years of age, the fastest growing segment of the US population. AMD threatens the eyesight and independence of our growing population of older Americans. People over age 60 are at greatest risk for AMD. Glaucoma is also a major public health problem and the number one cause of blindness in African Americans. It is often described as a silent thief of sight, because there may be no symptoms in the early stages of the disease process until the loss of side or peripheral vision becomes noticeable. As the disease progresses, the field of vision narrows until blindness results. Blacks over age 40, everyone over age 60, and people with a family history of glaucoma are at increased risk for glaucoma.

Rationale: The development of effective treatments for AMD has been limited by the complicated nature of the disease and the fact that the pathophysiology of the disease is poorly understood. The genes for other forms of macular degeneration, including Stargardt disease and Best macular dystrophy, have been identified and are being studied to learn whether similar disease mechanisms are involved in AMD. These genes have also been considered as candidate genes for AMD, but the results suggest a complex underlying genetic predisposition or susceptibility to biological and environmental factors in the pathogenesis of this complex disorder. Further investigation of the genes that control this predisposition or susceptibility may improve our understanding of the disease process and ultimately lead to improved treatments or the means to prevent this disease. Glaucoma is not a single disease but rather a group of diseases characterized by a particular type of retinal ganglion cell death that is usually, but not always, associated with an increase in intraocular pressure. Current treatments, whether surgical or pharmacologic, are aimed at reducing intraocular pressure and are often inadequate in preventing vision loss. A variety of mutations have been identified that may play a role in the development of primary open angle glaucoma. The multiple genetic loci and gene associations linked to various forms of glaucoma are further indication of the complex nature of this disease and underscore the need for additional research to clarify the roles of environmental and genetic risk factors in the pathology of this heterogeneous disease.

[1] U.S. Census: Profile of General Demographic Characteristics: 2000.
[2] AREDS Study Group. A Randomized, Placebo-Controlled, Clinical Trial of High-Dose Supplementation With Vitamins C and E, Beta Carotene, and Zinc for Age- Related Macular Degeneration and Vision Loss. Arch Ophthalmology 119: 1417-1436, 2001
[3] Prevent Blindness America. Vision Problems in the U.S.: Prevalence of Adult Vision Impairment and Age-Related Eye Diseases In America. 36 pp. (2002)
[4] Health Service. Publication No. (NIH)73-427. pp. 120-143.

6b - GOAL STATEMENT: By 2011, assess the efficacy of at least three new treatment strategies for reducing cardiovascular morbidity/mortality in patients with type 2 diabetes and/or chronic kidney disease.

Prevalence: Prevalence of both diabetes and kidney disease is rising. These diseases markedly increase the risk for life-threatening cardiovascular disease (CVD).

- In 2000, the prevalence of diagnosed diabetes in the U.S. was 7.3%, a 49% increase since 1990.[1] Currently, an estimated 17 million Americans suffer from diabetes; of these, approximately 16 million have type 2 diabetes.[2]
- CVD accounts for two thirds of deaths in people with diabetes.[3]
- Chronic kidney disease affects an estimated 10 to 20 million Americans[4] and can lead to kidney failure.
- The number of patients with kidney failure (ESRD) has doubled over the last decade, and now stands at nearly 400,000.[5]
- Heart disease and stroke are the leading causes of death in patients with ESRD.[6]

Disease Burden: We face national epidemics of both type 2 diabetes and kidney failure. In 1997, the economic cost of diabetes in the U.S. was estimated at $98 billion.[7] Once considered a disease of adults, type 2 diabetes now increasingly strikes during childhood. Rates of type 2 diabetes are approximately twice as high in African American and Hispanic populations as in Caucasian Americans, and are even higher in Native Americans.[2] Among adults with diabetes, heart disease death rates are two to four times higher than in the general population[8]. Diabetes also negates the protection gender affords non-diabetic women.[9] Even among individuals with impaired glucose tolerance, in whom glucose levels are higher than normal but do not yet indicate diabetes, CVD death rates are elevated 1.4 fold.[10] Chronic kidney disease is also a significant health burden. In its most severe forms it leads to kidney failure, also called end-stage renal disease (ESRD), where either dialysis or kidney transplantation is required to maintain life. About one-half of new cases of ESRD have kidney disease as a consequence of diabetes.[11] The number of patients with ESRD has doubled over the last decade, with the increasing disease burden most marked in minority populations, especially African Americans and Native Americans.[5] The markedly reduced life expectancy of patients with end-stage kidney disease is largely due to death from heart disease and stroke; rates of CVD are 10- to 100-fold greater than in the general population.[6] Notably, even among chronic kidney disease patients with a mild to moderate reduction in kidney function, CVD rates are increased two to four fold.[12] The cost of caring for the ESRD population was $19.4 billion dollars in 2000[13] and consumed about 6% of the Medicare budget.[14]

[1] Mokdad et al., 2001. JAMA 286: 1195-1200.
[2] National Diabetes Statistics, March 2002, NIH Publication No. 02-3892.
[3] Geiss et al., Mortality in Non-Insulin-Dependent Diabetes. In: Diabetes in America. NIH, NIDDK. 1995, pages 233-257.
[4] National Kidney Foundation, 2002, Am. J. Kidney Dis. 39: S1-S266 (suppl).
[5] United States Renal Data System 2002 Annual Data Report, NIH, NIDDK, pp. 44-50
[6] United States Renal Data System 2002 Annual Data Report, NIH, NIDDK, p. 167; Sarnak and Levey, 1999, Seminars in Dialysis 12: 69-76.
[7] 1998, Diabetes Care 21: 296-309.
[8] Haffner et al., 1998, N. Eng. J. Med. 339: 229-34.
[9] Wingard and Barrett-Conner, Heart Disease and Diabetes. In: Diabetes in America. NIH, NIDDK. 1995, pages 429-448.
[10] Saydah et al., 2001, Diabetes Care 24: 447-453.
[11] United States Renal Data System 2002 Annual Data Report, NIH, NIDDK, pp. 60-70.
[12] Sarnak and Levey, 2000. Am. J. Kidney Dis. 35: S117-31.
[13] United States Renal Data System 2002 Annual Data Report, NIH, NIDDK, p. 18.
[14] Eggers, 2000, Seminars in Nephrology 20: 516-522.

Rationale: For both diabetes and kidney disease, premature CVD is the major cause of death. CVD in patients with type 2 diabetes and with kidney disease is associated with some of the same risk factors as in the general population, including obesity, hypertension, and abnormal blood lipid levels, but these diseases confer substantial additional risk for CVD. Recent clinical trials have established the benefit of intensive management of blood pressure and LDL-cholesterol in reducing CVD risk, but a number of potential strategies to reduce the risk of CVD in these conditions require further exploration. While even moderate weight loss can dramatically reduce the development of type 2 diabetes in those at high risk, a benefit of weight loss in preventing cardiovascular complications in people with diabetes has not yet been established. While improved blood glucose control dramatically reduces the eye, kidney, and nerve complications of diabetes, its benefits in reducing CVD are not fully established, nor is it known whether insulin-providing or insulin-sensitizing strategies for glucose control are optimal for reducing CVD. Lowering of LDL cholesterol has been shown to prevent CVD, but type 2 diabetes is associated with a distinct lipid profile, with low HDL cholesterol and increased triglycerides, and research is needed to establish optimal management of lipids and blood pressure to reduce CVD in type 2 diabetes. Homocysteine levels rise as the kidneys fail, and homocysteine has long been known as a risk factor for CVD. Folate and B-vitamin supplementation can normalize homocysteine levels in patients with mild chronic kidney disease. It is not yet clear, however, whether this will reduce the risk of CVD. A major goal of NIH-funded research is to discover and evaluate strategies to reduce risk factors for, and to effectively treat, CVD in patients with diabetes and/or kidney disease. If successful, this research would extend lifespan and improve quality of life.

6c - GOAL STATEMENT: By 2012, develop a knowledge base on Chemical Effects in Biological Systems using a Asystems toxicology@ or toxicogenomics approach.

Background: Toxicogenomics is a new scientific field that examines how chemical exposures disrupt biological processes at the molecular level. This knowledge could be catalogued in a way that would allow researchers to predict adverse health effects of relatively unstudied chemicals and drugs; they could even predict how individuals would differ in their response to these compounds. Toxicogenomics would accomplish these feats by studying how the basic building blocks of biological systems – our genes – respond to environmental toxicants and other stressors. When a person is exposed to a chemical, cells in the body may respond by switching on (upregulating) some genes and switching off (downregulating) others, potentially changing the proteins that are produced by the cells. The pattern of regulation of various genes is different for different chemicals, creating a characteristic pattern or Asignature,@ which scientists hope will be useful in classifying chemicals and other stressors by their biological activity. This signature pattern would provide a means of predicting effects on human health from chemicals we currently know little about.

Toxicogenomics seeks to use these signature gene expression patterns to go beyond the traditional toxicological tools of testing animals for adverse outcomes that might indicate toxicity. Traditional methods, such as physical examinations, tissue samples, and blood tests, would be replaced with techniques using DNA microarray technology. The power and potential of these new toxicogenomics methods are capable of revolutionizing the field of toxicology. We anticipate that our understanding of mechanisms of toxicity and disease will improve as these new methods are used more extensively and toxicogenomics databases are developed more fully. The result will be the emergence of toxicology as an information science that will enable thorough analysis, iterative modeling, and discovery across biological species and chemical classes.

Rationale: Quicker and safer development of therapeutic drugs and commercially important chemicals would result if there were a better way to predict adverse reactions early in the development process. This goal is one of the possible outcomes of investments now being made in toxicogenomics.

The pharmaceutical industry is greatly interested in this technology because of their need to speed up the process of toxicological assessment of new R & D products. Identifying molecular events that serve as precursors of adverse health outcomes early in the development process would eliminate much of the expense (estimated in the billions of dollars annually) associated with the development of new pharmaceutical products.

NIH aims to create a Chemical Effects in Biological Systems (CEBS) knowledge base. More than a "database," the CEBS knowledge base will contain data on global gene expression, protein expression, metabolite profiles, and associated chemical/stressor induced effects in multiple species. With such information, it will be possible to derive functional pathways and network information based on cross-species homology.

7a - GOAL STATEMENT: By 2005, evaluate 10 commonly used botanicals for inhibition/induction of enzymes that metabolize drugs as a method of identifying potential botanical/drug interactions.

Prevalence/Incidence: The CDC reported that 29 percent of American adults used at least one complementary and alternative medicine (CAM) therapy in the past year, of which nearly ten percent used a botanical product.[1] A separate study reported that 18 percent of individuals taking prescription drugs were concurrently using botanical products, high dose vitamins, or both, estimating that 15 million adults are at risk for interactions between drugs and dietary supplements (a large category that includes botanicals, vitamins, amino acids, and similar products other than drugs).[2]

Disease Burden: Heterogeneous in nature, interactions between botanicals and drugs demonstrate a wide range of effects. Peer-reviewed scientific research literature has documented such events. For example, one study of St. John's wort showed it greatly reduced plasma concentrations of the anti-HIV medication indinavir. Similar phenomena have been demonstrated with the cancer drug irinotecan, the immunosuppresant drug cyclosporine, and certain birth control medications. A study of garlic indicated interaction with saquinavir, another anti-HIV medication. Additional studies have documented the potential for herbal products to interact with anesthetic agents.

Rationale: Although botanical products are widely used in the United States, little or no authoritative information is available on potential botanical-drug interactions to either the consumers or health care providers. Likewise, the systematic evaluation of the potential of botanicals to interact with conventional medications has largely gone unexplored. Botanicals are complex mixtures of naturally occurring chemical compounds, some of which proved sufficiently potent to serve as the basis for many current drugs. It could be expected, then, that botanical products could manifest a broad array of interactions with conventional drugs so as to enhance their activity and evoke greater drug toxicity, or to accelerate their metabolism and impair their therapeutic benefits. Compounds contained in some botanical products have already been proven to interact with drugs by inhibiting or inducing specific hepatic cytochrome P450 enzymes that are critical for drug metabolism and elimination. Of this large enzyme system, two specific enzymes, CYP 3A4 and CYP 2D6, are involved in the metabolism of approximately 80 percent of all marketed drugs, thereby providing a rational starting point from which to examine the potential for botanical-drug interactions.

[1] Ni H, *et al*. Utilization of complementary and alternative medicine by United States adults: results from the 1999 national health information survey. Med Care 2002 Apr;40(4):353-8
[2] Eisenberg DM, *et al*. Trends in alternative medicine use in the United States, 1990-1997: results of a follow-up national survey. JAMA 1998 Nov 11; 280(18):1569-75.

7b - GOAL STATEMENT: By 2006, integrate nanotechnology-based components into a system capable of detecting specific biomarker(s) (molecular signatures) to establish proof of concept for a new approach to the early detection of cancer and, ultimately, cancer preemption.

Prevalence/Incidence: Cancer is the second leading cause of death in the United States. During 2001, an estimated 1,268,000 persons in the United States were diagnosed with cancer, including 198,100 prostate cancers; 192,200 female breast cancers; 169,500 lung cancers; and 135,400 cancers of the colon/rectum.[1] These estimates did not include most skin cancers and new cases of skin cancer are estimated to exceed 1 million per year. One-half of all cases of cancer occur in people aged 65 years and over.[2]

Disease Burden: Our Nation's past investments in cancer research are paying major dividends, for example:

- Americans are increasingly adopting good health habits to reduce their cancer risk.[1]
- Overall, cancer rates are dropping, especially for cancers that are diagnosed prior to metastatic spread.[3]
- Overall, the more than 9 million cancer survivors in America are enjoying a higher quality of life than was possible just a few years ago.[1]
- However, in the face of these significant advances, cancer remains a major public healthcare problem and, with the aging and changing demographics of America, expected increases in numbers of new cancer cases loom as a potential healthcare crisis.[3]
- The incidence rates of certain cancers continue to rise. For example, rates of lung cancer in women, non-Hodgkin's lymphoma, and melanoma are increasing.[1]
- The cost of the cancer epidemic is estimated to be in excess of $180 billion per year and this burden will continue to rise as cancer moves to become the number one killer of Americans in the next few years.[3]
- The rates of both new cases and deaths from cancer vary by cancer site, socioeconomic status, sex, and racial and ethnic group.[1]

***Rationale*:** Recent advances in understanding the molecular basis of cancer, and the associated development of novel molecular technologies in areas such as proteomics, portend a future where cancer can be detected early and preempted before it spreads, perhaps on an individualized basis. For example, nanoscience offers unparalleled opportunities to measure and monitor changes within cells at the level of multiple atoms. Nanoscience researchers are developing "nanospheres" that can be deployed in the body to detect real-time changes in normal cells. These nanoparticles can carry a variety of specially designed, molecular-sized attachments allowing them to act as biosensors that can be programmed to detect malignant changes in normal cells and potentially deliver treatment - without harming healthy cells. Applications of nanotechnology have the potential to shift the paradigm of cancer toward earlier detection and prevention, provide a new platform for eventual high-throughput diagnostics, and, ultimately, real-time monitoring of patients.[4]

[1] *Cancer Progress Report 2001;* NIH Publication No: 02-5045, December 2001. page 18.
[2] Ries, L.A.G.; Kosary, C.L.; Hankey, B.F.; et al. *SEER Cancer Statistics Review, 1973–1996.* Bethesda, MD: National Cancer Institute, 1999.
[3] Edwards BK, Howe HL, Ries LAG, Thun MJ, Rosenberg HM, Yancik R, Wingo PA, Jemal A, and Feigal EG. Annual Report to the Nation on the Status of Cancer, 1973-1999, Featuring Implications of Age and Aging on the U.S. Cancer Burden, May 15, 2002 (Vol. 94, No. 10, pages 2766-2792), *Cancer*
[4] *The Nation's Investment in Cancer Research: A Plan and Budget Proposal for Fiscal Year 2004.;* NIH Publication No: 03-4373, October 2002, page 69.

7c - GOAL STATEMENT: By 2005, create the next generation map of the human genome, a so called "haplotype map" (HapMap), by identifying the patterns of genetic variation across all human chromosomes.

***Prevalence/Incidence*:** Virtually all diseases have a genetic component. The DNA sequences of any two people are 99.9 percent identical. However, there are at least 10 million DNA sites where people commonly differ and these variations may greatly affect an individual's risk for disease or response to drugs.

Disease Burden: The goal of much genetic research is to find genes that contribute to disease. Finding these genes allows an understanding of the disease process, thus enabling development of methods for disease prevention and treatment. For "single-gene disorders," diseases with a relatively straightforward genetic basis, current methods are often sufficient to find the genes involved. Most people, however, do not have single-gene disorders, but develop common diseases such as diabetes, cancer, stroke, heart disease, psychiatric disorders, arthritis, or asthma, which occur due to interactions of multiple genetic and environmental factors. Strategies that work well for single-gene disorders lack the power to map such multi-gene disorders; thus, relatively little is known about the genetic basis of these common diseases, or of the factors that determine individual risk of disease, clinical course, or response to treatment.

Rationale: By understanding the way in which genetic variations are correlated in DNA "neighborhoods," considerable savings in time, effort, and cost can be achieved in uncovering the hereditary factors in common diseases like diabetes, cancer, and mental illness. Sites in the genome where individuals differ in their DNA spelling by a single letter are called single nucleotide polymorphisms (SNPs). Recent work has shown that about 10 million SNPs are common in human populations. SNPs are not inherited independently; rather, sets of adjacent SNPs are inherited in blocks. The specific pattern of particular SNP spellings in a block is called a haplotype. Although a region of DNA may contain many SNPs, it takes only a few SNPs to uniquely identify or "tag" each of the haplotypes in the region. This presents the possibility of a major shortcut in identifying hereditary factors in disease. Instead of testing 10 million SNPs, a rigorously chosen subset of about 400,000 SNPs could provide all of the essential information.

Most common haplotypes occur in all human populations, although their frequencies may vary considerably. Initial studies also indicate that the boundaries between the blocks are remarkably similar among populations in Europe, Asia, and Africa. These data indicate that a human haplotype map built with samples from these three geographic areas would apply to most populations in the world, although further testing of this conclusion is needed.

The NIH has taken a leadership role in the development of the HapMap, a catalog of the haplotype blocks and the SNPs that tag them. The HapMap will be a tool that can be used by researchers studying many diseases, to find the genes and variants that contribute to those diseases. In addition to its use in studying genetic associations with disease, the HapMap will be a powerful resource for studying the genetic factors contributing to variation in individual response to disease once it does occur, as well as to drugs and vaccines. It will also be a potent tool for better understanding the interactions of genes with environmental factors. Ultimately, the development of this powerful tool will allow the biomedical research community to understand complex genetic diseases much more fully and will lead to improved treatments and, ultimately, cures for many of these disorders.

8a - GOAL STATEMENT: By 2007, determine the genome sequences of an additional 45 human pathogens and 3 invertebrate vectors of infectious diseases.

Background: Genome sequencing reveals the lineup of paired chemical bases that make up the pathogen's DNA, the language of life. The potential payoffs of sequencing pathogens are enormous. Sequencing information can be exploited in many ways: to identify molecules for vaccine and drug development; to identify mutations that contribute to drug resistance; to compare the genomes of different strains of pathogens and to note differences that may effect the virulence of a microbe or its ability to evoke disease; and to trace microbial evolution. When scientists identify genes that are unique to a particular microbe, drugs can be targeted to these genes, and the products of these genes can be incorporated into experimental vaccines. Furthermore, strategies can be devised to counteract genetic mutations that cause a microbe to become drug resistant. Once genes are found that are associated with infectious disease, researchers can attempt to disable them. Moreover, genetic variations detected in different strains of the same pathogen can be used to study population dynamics of these strains, such as the spread of a virulent form of an organism in a susceptible population. In addition, understanding the genetic basis for both virulence and drug resistance also may help to predict disease prognosis and influence the type and extent of patient care and treatment.

Recognizing the incredible potential of microbial genomics research, the NIH has made a significant investment in the large-scale DNA sequencing of the genomes of human pathogens and invertebrate vectors of disease, including microorganisms considered to be potential agents of bioterrorism. The genome sequences of thirty-two bacterial pathogens, a parasitic protozoa, 1 chromosome of another parasitic protozoa, and an invertebrate vector (an organism that spreads disease, e.g., a mosquito) have been completed with NIH support (http://www.nidr.nih.gov/research/dbts/microbial_lrg_scale_DNA_prjs.asp; http://www.niaid.nih.gov/dmid/genomes) and these sequences have been released rapidly to the scientific community through a publicly accessible web site. NIH has funded projects to sequence the full genomes of a number of medically important microbes, including the bacteria that cause tuberculosis, gonorrhea, chlamydia, cholera, strep throat, scarlet fever, and foodborne diseases. Recently, the complete genome sequences of *Plasmodium falciparum* and *Anopheles gambiae*, the most lethal malaria-causing parasite and its mosquito vector, respectively, were published, providing a valuable resource to the scientific community. This work, which was supported in part by NIH, will provide the basis for further experimental studies to understand the pathogenesis of the parasite and its vector, and provides potential molecules that could serve as the basis for the next generation of drugs, vaccines, and diagnostics.

Rationale: Significant progress in DNA sequencing technology has allowed genomic DNA to be sequenced more efficiently and cost-effectively. In fact, it is now possible to sequence a bacterial genome in a month or less. DNA sequencing technology is being improved further, and innovative new sequencing technologies that will revolutionize the speed, efficiency and cost by several orders of magnitude are in the immediate future. A critical companion to state-of-the-art DNA sequencing techniques are the bioinformatics, computational tools and databases that provide the scientific community with the needed resources to query, analyze and annotate the sequencing data, and assemble genomes.

8b - GOAL STATEMENT: Identify and characterize two molecular interactions of potential clinical significance between bone-forming cells and components of bone. Such interactions are defined as those having significant impact on the accrual of bone mass or the actual mechanical performance of bone (i.e., fracture resistance) in laboratory animals.

***Background*:** Both skeletal health and the maintenance of normal blood calcium levels depend upon the process of bone turnover, in which small regions of bone are broken down (resorbed) and then replaced with new bone. The regulation of the balance between bone resorption and new bone formation, which can be affected by nutritional, endocrine, and pharmacological factors, is critical to maintaining bone mass and preventing fracture. An excess of resorption over formation underlies many bone diseases, such as post-menopausal osteoporosis.

Osteoblasts are the cells that form new bone during bone turnover. In addition, some osteoblasts remain embedded in the bone, becoming osteocytes. Recent work has shown that osteocyte survival is an important requirement for skeletal health. Bone itself is composed of mineral crystals embedded in a matrix made up of many different proteins. There is evidence that interactions between matrix proteins and proteins found at the cell surfaces of osteoblasts and osteocytes produce signals that are important for the regulation of bone turnover and the survival of osteocytes. However, the molecular details of cell-matrix interactions have been explored in only a few instances. If known, the mechanisms of these interactions could yield targets for new drugs that might act to stimulate bone formation or block bone resorption.

Rationale: Recent advances, particularly in the genetic manipulation of mice, make it possible to define the function of different matrix proteins and the cell surface proteins that interact with them. For example, mice can be created that either lack a certain matrix protein or produce abnormally large amounts of the protein. Cell surface proteins thought to interact with matrix proteins can also be tested in this way. It is important to conduct these experiments with intact, genetically modified mice, rather than in cell culture, for two reasons. First, although osteoblasts can be induced to produce bone matrix in culture, the interaction between cells and matrix in culture is not normal. For example, osteoblasts do not become osteocytes within the bone produced in culture. Second, the consequences of interfering with specific cell-matrix interactions can be assessed thoroughly by examining the bones of mice. This can even indicate the ultimate effect on the mechanical strength of the bones.

It is clear from work to date that altering cell-matrix interactions can produce changes in bone remodeling activity and bone mass. However, in order to accelerate progress toward this goal, we need to refine our understanding of known cell-matrix interactions, and identify new interactions with important roles in the maintenance of skeletal health.

8c - GOAL STATEMENT: Build a publicly accessible Collection of Reference Sequences to serve as the basis for medical, functional, and diversity studies. A comprehensive Reference Sequence Collection will serve as a foundation for genomic research by providing a centralized, integrated, non-redundant set of sequences, including genomic DNA , transcript (RNA), and proteome (protein product) sequences, integrated with other vital information for all major research organisms.

Background: The Reference Sequence Collection will provide a unified view of our genetic knowledge of organisms. A single, high-quality collection of reference sequences for multiple species that is richly annotated and highly connected to other information sources will make it possible to undertake large-scale comparative analyses. The ability to make discoveries in one organism (such as mouse models of a human disease) and immediately apply them to another organism (such as humans) is one of the most powerful aspects of molecular biology. The academic and pharmaceutical research communities use reference sequences in this way to investigate basic molecular biological processes and medical problems, such as different disease susceptibilities for individuals or targeted individual drug treatment approaches. The availability of a Reference Sequence Collection means that time once spent identifying resources, gathering data, and reviewing its quality is freed for research.

Rationale: Hundreds of millions of dollars have been invested by Federal agencies, international governments, and charitable foundations to obtain genomic and transcript sequence data for organisms from human to viruses. Although a wealth of sequence data is now available, it exists in multiple formats and locations and is not connected to other information; furthermore, the data produced by different groups are often redundant, inconsistent, or partially overlapping. Without a cohesive representation of the data, it is difficult to reap the full benefit of the massive public investment in obtaining the data. The Reference Sequence Collection will serve as a foundation for genomic research by providing a centralized sequence set integrated with other information including publications, phenotypes, and disease catalogs. This collection must be built and maintained through both computational and expert analysis in order to integrate large quantities of disparate data while also providing a high-quality resource. Both the computational and expert tasks must be ongoing so that: 1) the collection stays current as new data become available; 2) quality is ensured; and 3) new opportunities that add value are identified.

9a - GOAL STATEMENT: By 2009, assess the impact of two major Institutional Development Award (IDeA) programs on the development of competitive investigators and their capacities to compete for NIH research funding.

***Background*:** The IDeA program was authorized within the NIH Revitalization Act of 1993. State eligibility is based on the aggregate level of NIH grant funds received by research institutions collectively within a state over the preceding 5 consecutive years and the average success rate of research applications over that same time span. Between 1997-2001, states that received less than $75 million in NIH grant awards and/or held a success rate of less than 20 percent over than time span, qualified for the IDeA program. The 23 IDeA eligible states include Alaska, Arkansas, Delaware, Hawaii, Idaho, Kansas, Kentucky, Louisiana, Maine, Mississippi, Montana, Nebraska, Nevada, New Hampshire, New Mexico, North Dakota, Oklahoma, Rhode Island, South Carolina, South Dakota, Vermont, West Virginia, and Wyoming as well as Puerto Rico.

The IDeA program is intended to develop the research infrastructure and competitiveness of faculty among institutions located in states that historically have not fully participated in NIH research programs. Although the program was first established in FY 1993, the limited budgets had precluded development of major programs. However, as the appropriation for the IDeA program has become more robust in recent years, the IDeA program has been restructured and further enhanced. The funding level of the program has increased from $750,000 in FY 1993 to $160 million in FY 2002.

Investigators in IDeA states earned a significant increase of NIH awards between 1997 and 2002. In 1997, investigators received 477 NIH research project grant awards and 679 in FY 2002, a 42.3 percent increase. Over that same time span, the number of applications submitted by IDeA state investigators increased from 2355 to 2744, a 16.5 percent increase. The appropriation for the IDeA program increased from $750,000 in FY 1993 to $10 million in FY 1999. In FY 2000, the program increased to $38.5 million, which allowed the design of the first major IDeA program and its implementation.

The new program, Centers of Biomedical Research Excellence (COBRE), was specifically designed to enhance the pool of well-trained investigators who could successfully compete for NIH grant awards. The COBRE program augments and strengthens institutional biomedical research capacities by expanding or modifying research facilities, equipping laboratories with modern research equipment and providing support for developing research faculty through support of a multi-disciplinary center, led by a peer-reviewed, NIH-funded magnet investigator.

In FY 2001, the IDeA program increased to $100 million, which allowed the IDeA program to add another program, the Biomedical Research Infrastructure Network (BRIN). BRIN is intended to enhance the caliber of scientific faculty at undergraduate schools, which will, in turn, attract more promising students to those institutions.

Rationale: Strong congressional interest in IDeA, along with significant increases in funding of IDeA programs, have led to questions about whether programs designed to enhance competitiveness of investigators have led to more NIH research grants to more investigators. The planned study will assess the impact of the IDeA programs on NIH research funding, as a percent of total NIH funding among the cohort of eligible states, and determine the factors that have had the greatest impact on enhancing investigator competitiveness.

9b - GOAL STATEMENT: By 2010, demonstrate through research a capacity to reduce the total years lost to disability (YLDs) in the U.S. by 10 percent by (1) developing treatment algorithms to improve the management of treatment-resistant and recurrent depression and (2) elucidating the mechanisms by which depression influences at least two comorbid physical illnesses (e.g., heart disease, cancer, Parkinson's disease or diabetes). Major depression is now the leading cause of YLDs in the Nation.

Prevalence/Incidence: Depressive disorders are serious medical illnesses that affect more than 20 million Americans ages 18 and older and an estimated 4-5% of children and adolescents.

- ~9.9 million adults (5%) have major depressive disorder (MDD) [1]
- ~10.4 million adults (5.4%) have dysthymia, a moderate but chronic form of depression; an estimated 40% people with dysthymia meet criteria for MDD or bipolar disorder (BP) in a given year.[1]

The failure of depressed patients to respond satisfactorily to an adequate clinical trial of antidepressant medication or psychotherapy (an estimated 50%), and the frequency with which patients are left with unresolved symptoms or impairments (an estimated 20%) are important issues because residual symptoms are associated with significant functional impairment and substantially increase the risk of relapse and recurrence. Estimates from clinical populations indicate that patients with major depression will experience an average of four lifetime episodes of 20 weeks each in duration, but risk may vary by the number of prior depressive episodes. Those with at least three prior episodes show 70-80% relapse rates, compared to those with no depression history who show 20-30% relapse rates.[2]

Disease Burden: Depressive disorders are prevalent, disabling, often chronic, and potentially fatal illnesses; about 60% of people who die by suicide have had a mood disorder (e.g., major depression, bipolar disorder, dysthymia).[3] The WHO *World Health Report – 2001* identifies major depressive disorder as the leading cause of Disability-Adjusted Life Years (DALYs), i.e., the composite of Years Lost to Disability (YLDs) and potential Years of Life Lost (YLLs) to premature death, in the U.S.[4] Depression accounts for 2.6% of total DALYs among men, and 6.77% among women.[5]

Mediated through the brain, mood disorders disrupt every facet of a person's life: emotions, thought processes, behavior, and physical health. In addition to the inherent effects of depression on health through sleep and appetite dysregulation, self-medicating substance abuse, and physiologic disturbances (e.g., sticky platelets) that are just beginning to be understood, major depression can influence significantly the outcome of comorbid general medical illnesses. Depression is seen frequently in people with coronary heart disease (CHD) and other cardiac illnesses; for example, among patients with congestive heart failure, estimates of the prevalence of major depression range from 17- to 37%. Untreated depression increases the risk by as much as six-fold of dying from heart disease. People with comorbid diabetes and depression have an eight times greater relapse rate than those with depression but without other medical conditions. The prevalence of major depression in patients after a stroke is approximately 20%, and estimates of lifetime rates of depression among persons living with HIV range from 22 to 45 percent. [6]

Rationale: Efficacious and effective treatments benefit millions of persons with major depression; however, a significant proportion (~25-33%) of persons with depression are not

[1] The Numbers Count: Mental Disorders in America. NIH Publication No. 01-4584
[2] Judd L (1997) The clinical course of unipolar major depressive disorders. Arch Gen Psychiatry 54:989-991.
[3] Conwell Y, Brent D (1995). Suicide and aging. I: Patterns of psychiatric diagnosis. Int Psychogeriatr (2):149-64.
[4] WHO: The World Health Report 2001. Mental Health: New Understanding, New Hope. WHO, 2001
[5] Michaud CM, Murray CJL, Bloom BR (2001), Burden of Disease: Implications for Research. JAMA 285:535-539.
[6] The Strategic Plan for Mood Disorders Research of the National Institute of Mental Health. NIH Publication No. 02-5121.

helped or do not fully recover when given a standard pharmacological or psychosocial intervention, or depression is not recognized in the context of other general medical illnesses. In pursuit of this goal's focus on treatment resistant depression, NIH will assess effective pharmaceutical algorithmic approaches as well as study how to improve clinicians' capacities for timely identification of patients who have unrecognized or refractory depression. With respect to the goal's focus on reducing the recurrence of depression, NIH will continue to encourage new treatments that build in relapse/ recurrence components to initial treatment approaches. With regard to the focus on depression comorbid with general medical illnesses, NIH will seek to develop (a) fundamentally new clinical interventions and (b) algorithms for the informed use of existing treatments in the face of treatment resistance. Similarly, NIH will target research on identifying biological, behavioral, psychosocial, cultural, and environmental risk and protective factors linking mental and medical disorders. The aim of these studies will be to ascertain those factors that account for the greatest relative variance in the prevalence of depression-comorbid illness and those that are modifiable. The premise of this goal is that targeted research on these topics will have a disproportionate impact on the overall reduction of YLDs associated with depression.

9c - GOAL STATEMENT: By FY 2010, identify culturally appropriate, effective stroke prevention programs for nation-wide implementation in minority communities.

Disease Burden: Although stroke remains the third cause of mortality in the United States and the leading cause of adult disability, the burden of stroke is greater among minority racial/ethnic groups by virtual of its higher incidence and mortality in these populations. The incidence of ischemic and hemorrhagic stroke is disproportionately high in the African American population, occurs at younger ages, and these disparities may be increasing.[1,2] Mortality from stroke among African Americans is nearly twice that of Caucasian Americans.[3] Moreover, among several minority racial/ethnic groups (including African, Hispanic and Native Americans), the disparity in stroke mortality (both ischemic and hemorrhagic) is especially evident among younger individuals – those aged 45-64 years.[3] However, the burden may be even greater than the stroke incidence and mortality rates indicate. Initial evidence suggests that African Americans may experience more severe strokes and greater residual physical deficits, although these deficits may not be fully reflected by impairment in ability to perform activities of daily living.[4-6] It remains to be determined if other minority racial/ethnic groups also experience more severe and disabling strokes than Caucasians.

Rationale: There is a wide range of hypothesized causes of the excess stroke mortality in the Southeastern U.S. (the "Stroke Belt") and among African Americans. The prevalence of stroke risk factors and the potential impact of reducing those factors vary among racial/ethnic groups, with potentially greater impact associated with reduction or elimination for minorities.[7] Patterns of accessing the existing health care system for acute stroke also vary among racial/ethnic groups; for example, minorities are less likely to use the emergency medical system when experiencing a stroke.[8] The reasons for these racial/ethnic variations in stroke-related risk factors and utilization of health care are not fully understood, but will need to be in order to identify the most effective stroke prevention and treatment programs for minority communities. Prevention programs are a preferred strategy for reducing or eliminating the observed racial/ethnic disparities in stroke, and include both primary and secondary prevention approaches. Primary prevention programs target stroke risk factors to reduce the occurrence of stroke. Secondary prevention programs seek to improve access to timely acute stroke care, thereby reducing mortality and morbidity, and target the use of interventions to prevent subsequent stroke in stroke survivors.

The HHS Research Coordination Council (RCC) has identified "Understanding Health Disparities – Closing the Gaps" as a priority for FY2004, and in its stated priority areas has recognized to need to understand factors contributing to disparities in the development of diseases, injuries, and disabilities; to improve detection and diagnosis of diseases that contribute to health disparities, such as stroke; to improve approaches to delay onset or prevent diseases, injuries, and disabilities that contribute to health disparities; to improve treatments for diseases and disabilities that contribute to health disparities; to expand research using bioinformatics and genomics, including pharmacogenomics, in addressing health disparities; and to enhance research on the intersection between non-genetic and genetic factors in health disparities.

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2.1.3.2 Communication of Results

Communicating scientific results and health information to the medical research community, health care providers, patients, and the general public has long been recognized as a critical NIH function. Information dissemination helps ensure that the science NIH conducts and supports is applied appropriately, whether by other scientists, health care providers, patients, or the public. Without the flow of information, important scientific findings would languish at the researcher's bench.

The Public Health Service Act of 1944 authorized NIH and the other PHS agencies to collect and make available, through publications and other appropriate means, information relevant to the practical applications of research [Title III, Sec. 301 (1)]. In addition, the legislation that enables and directs the development of NIH programs emphasizes NIH's important role in informing the public about the results of health-related research. Similarly, the authorizing legislation for the NIH Institutes and Centers (ICs) includes "dissemination of health information" as an integral part of each IC's basic mission.

All of the NIH ICs conduct programs to collect, disseminate, and exchange information on medical and biological science, medicine, and health. The National Library of Medicine (NLM), the world's largest medical library, is a component of NIH and works closely with the ICs to ensure the effective communication of research results. NLM has a broad congressional mandate, not only to collect and organize the literature of the health sciences and to provide information services, but also to develop programs to transfer the latest scientific findings to the scientific community, health professionals, researchers, and the general public worldwide.

To effectively communicate scientific results and health information to NIH's key challenges are to 1) increase the audience members' awareness that useful information is available, 2) ensure that they have access to the types of information they need, and 3) improve their understanding of how to use the information. A related challenge is to design communication strategies that will enhance public and provider awareness and understanding of clinical research, and increase their willingness to participate in NIH-sponsored clinical trials.

To address these challenges and enhance the effectiveness of its communication efforts, NIH established five performance goals:

a) Increase awareness of NIH-sponsored research among health care providers to promote research application.
b) Increase awareness of NIH-sponsored research results among racial/ethnic minorities and high-risk, underserved, and/or affected publics.
c) Increase awareness of NIH-sponsored research results among the general public.
d) Increase awareness of clinical research and support participation in clinical trials.
e) Establish a Clinical Trials Database, as required by the FDA Modernization Act.

NIH's efforts to achieve these performance goals include developing health education materials and implementing communication campaigns to reach specific audiences with information on significant health problems. Other activities focus on enhancing NIH operations, using techniques such as telehealth technology and consolidated databases, developing web-based tools, and strengthening collaborations with other organizations to improve the communication of research results and increase clinical trial participation. In addition, NIH conducts needs assessments and other evaluation studies as needed to improve communication initiatives within a competitive information environment.

Performance Goal Details - Communication of Results

Goal a) Increase awareness of NIH-sponsored research among health care providers to promote research application.

NIH's research mission–to develop new knowledge that leads to better health–depends on translating research advances into improved patient care. This goal contributes to that translation significantly, because the first step in the "chain of events" in using research results to improve patient care is to ensure that health care providers learn about the latest research findings.

To increase awareness of NIH-sponsored research among health care providers, NIH is concentrating on three major activities: 1) disseminating and evaluating the use of clinical practice guidelines, 2) developing and implementing communication campaigns designed to reach health professionals, and 3) exploring the potential of telemedicine and other technology for improving health care delivery.

Performance Targets Note: Annual targets are grouped by activity	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Disseminate and evaluate the use of clinical practice guidelines:						
Evaluate the use of clinical practice guidelines on treatment of high blood pressure and high blood cholesterol by physicians who provide care to African-American patients.	→	◆				
Evaluate the use of clinical practice guidelines on high blood pressure and obesity.	→	◆				
Disseminate and encourage the use of clinical practice guidelines for the treatment of high blood pressure, high blood cholesterol, and other conditions by physicians who provide care to African-American patients.		◆				
Use continuing medical education programs based on the web-based Asthma Management Model System to disseminate and encourage the use of clinical practice guidelines on asthma.			◆			
Collaborate with the National Committee for Quality Assurance to foster implementation of cholesterol clinical practice guidelines.					◇	

Performance Targets	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Develop and implement communication campaigns designed to reach health professionals:						
Use telehealth technology and TV cable networks for education projects with nursing organizations and academic institutions: broadcast select conferences and workshops to nursing organizations and academic institutions and add website components that will allow users to interact on-line with live discussions, conferences, and other types of meetings.		→	◆			
Expand the "Not Just Once, But for a Lifetime" mammography campaign to reach health professional organizations, physicians, nurses, and other health and medical practitioners to increase awareness of the importance of mammography screening and the Medicare mammography benefit, and referrals for women, particularly those aged 65 and older.		→	◆			
Complete web accessibility for viewing nursing education programs and establish a once-a-month chat room where program directors will be available to answer questions from nursing students and nurse researchers.			◆			
Increase awareness of NIDA-sponsored clinical treatment among health care providers.			◆			
Use a partnership with the American Academy of Family Physicians to increase the knowledge of primary care physicians about the diagnosis and treatment of mental disorders.			◆			
Develop a communications campaign to build support and enhance the recruitment for domestic and international HIV vaccine trials.				◆		
Disseminate resources to assist hearing health professionals, such as nurses, audiologists, pediatricians, and family practice doctors, in all 50 states, in following up on early identification of hearing loss.						◇
Explore the potential of information technologies to educate health professionals:						
Fund a series of demonstration projects applying telemedicine and other technology to improve the speed of reaching heart attack victims with lifesaving treatment.		◆				

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- **NIH has disseminated clinical practice guidelines via the Internet.** In FY 2000, NIH completed an evaluation of the use of clinical practice guidelines on the treatment of high blood pressure and high blood cholesterol by physicians providing care to African American patients. To disseminate and encourage use of the guidelines, NIH developed model case studies on 11 aspects of treating cardiovascular disease in African Americans. The case studies were used to develop interactive web-based programs for physicians and nurses, and continuing education credits were offered to participants. In the next year, as a means of fostering the use of its clinical practice guidelines on asthma, NIH developed and marketed a comprehensive Internet-based Continuing Medical Education (CME) module covering all aspects of asthma clinical management. By the end of FY 2001, approximately 800 physicians had accessed the module and completed the course for full CME Category I credit.

- **NIH reaches health professionals through communication campaigns.** NIH made substantial progress in FY 2001 in reaching health professionals with important health information. The Breast Cancer and Mammography Education program and the National Institute on Drug Abuse (NIDA) Clinical Trials Network were expanded to reach more health professionals, and NIH used its partnership with the American Academy of Family Physicians to develop training programs on mental disorders. These programs included a monograph on the diagnosis and treatment of depression and a public education campaign to increase awareness of mental health problems and the role of the family physician in managing these problems. In FY 2002, the National Institute of Allergy and Infectious Diseases (NIAID) led a campaign to inform health professionals about HIV vaccine research, particularly vaccine clinical trials. The campaign reached professionals who work with HIV patients and organizations through lectures, workshops and exhibits at seven key national and international conferences, and through an advertising campaign that targeted health care providers in minority communities. This effort was an extension of a broader communication campaign that was launched in May 2002 (see FY 2001 targets for communication Goal b).

- **NIH continues to explore the potential of telemedicine and other technology for improving health care delivery.** NIH funded ten awards in FY 2000 for demonstration projects, which included investigating the potential of various high-tech applications to expedite the delivery of treatment to heart attack patients. NIH also collaborated with two other organizations to develop a videotape series for health care providers on end-of-life care. More recently, NIH has explored how personal digital assistants (PDAs) may be used to provide medical information to health professionals. By the end of FY 2001, over 4,200 users had downloaded the Palm Pilot version of NIH's guidelines for the treatment of asthma.

Goal b) Increase awareness of NIH-sponsored research results among racial/ethnic minorities and high-risk, underserved, and/or affected publics.

Many NIH research results apply to particular segments of the public, such as those at greater risk of contracting a specific disease or those who may not visit a health care provider regularly. However, these research results may not reach the populations most likely to benefit from them.

One of NIH's major priorities is to help reduce the striking disparities in the burden of illness and death experienced by certain groups in the U.S., particularly racial/ethnic minorities and other high-risk and underserved populations. The goal of increasing their awareness of NIH-sponsored research results responds to this priority directly. To achieve this goal, NIH focuses on developing and implementing 1) health education materials for non-English speaking and low-literacy audiences, 2) communication campaigns designed to reach high-risk populations, and 3) communication campaigns on specific health issues.

To reach high-risk populations, NIH develops targeted communication campaigns to disseminate information and resources on diseases and conditions that are more prevalent in certain populations, such as diabetes, drug abuse, and noise-induced hearing loss. Campaigns are designed to reach specific minority groups (e.g., African American, Hispanic, American Indian, and Asian communities), seniors, and other populations at high risk.

NIH also develops broad communication campaigns on a variety of specific health issues, such as sports injury prevention, the health implications of obesity, and eye health.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Develop health education materials for non-English speaking and low-literacy audiences:						
Establish a centralized site on the NIH home page for access to NIH materials in Spanish.	◆					
Evaluate several selected NIH outreach programs: cardiovascular health outreach activities for Latinos.	◆					
As an activity of the NIH Hispanic Communications Initiative (HCI), conduct a Spanish-language "media summit" that will detail strategies for developing continuous and sustainable working partnerships between NIH information offices, national Spanish-language media outlets, and national Hispanic intermediary organizations.		→	◆			
Develop and disseminate easy-to-read and Spanish language health education materials on health issues to targeted special populations.		◆				
Expand programs on anxiety disorders and depression to audiences for whom language or literacy are challenges.		◆				
Develop and disseminate Asian language materials communicating the benefits of mammography.				→	◆	
Develop and implement communication campaigns designed to reach high-risk populations:						
Extend the "Back to Sleep Campaign" to minority populations.	◆					

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
In collaboration with African American organizations, community health and other local officials, and faith-based organizations, conduct regional summit meetings to train and motivate individuals who will implement SIDS risk reduction activities in their communities.					◇	
Evaluate effectiveness of summit meetings as a means of extending "Back To Sleep" campaign to African American populations.						◇
In collaboration with American Indian and Alaska Native leaders, draft, pilot test, and publish culturally appropriate SIDS risk reduction materials to disseminate widely in American Indian and Alaska Native communities.						◇
Pursue new outreach and collaboration initiatives to disseminate information and resources on rheumatic diseases in minority populations.		◆				
Develop and implement diabetes awareness campaigns that target minority populations and their health care providers.		◆				
Develop and disseminate motivational messages related to breast and cervical cancer screening to African American, Hispanic, and Asian communities.		◆				
Increase the available information on the benefits of exercise to older people.		◆				
Increase awareness of autoimmune diseases (such as rheumatoid arthritis, lupus, and scleroderma) among minority populations who are disproportionately affected.			◆			
Increase understanding about the seriousness of diabetes and the importance of blood glucose control among African Americans, Asian/Pacific Islanders, and American Indians.			◆			
Increase awareness about how to prevent fully preventable noise-induced hearing loss (WISE EARS! Campaign) among industrial workers, Hispanic/Latino/Latina individuals, and Native American youth and their parents.			◆			
Use the results of the WISE EARS! evaluation to conduct outreach activities for targeted audiences, including African American, Hispanic/Latino/Latina individuals and Native American youth and their parents.						◇
Increase knowledge among Hispanic parents of the effects of drugs on the brain and encourage them to talk with their children about drug abuse.			◆			
Increase awareness of the effects of drug abuse among Native American Indians.			◆			
Provide support and technical assistance to NHLBI's Enhanced Dissemination and Utilization Centers to conduct heart-health education projects in high-risk communities.				◆		
Develop and implement communication campaigns on specific health issues:						
Develop and implement NIH information, education, and outreach programs on specific health issues: Breast Cancer and Mammography Education Program.	◆					
Develop and implement an HIV/AIDS vaccine communication campaign to increase awareness of HIV/AIDS vaccines before the initiation of a large efficacy trial.			→	◆		
Increase awareness of sports injury prevention among parents.			◆			
Develop messages and materials to communicate the health implications of obesity.				◆		
Develop and implement an eye health awareness campaign.				→	◇	
Implement a campaign to provide information on noise-induced hearing loss.					◇	
Use a variety of media approaches (TV and radio news inserts, etc.) to communicate the importance of eating five fruits and vegetables a day.					◇	

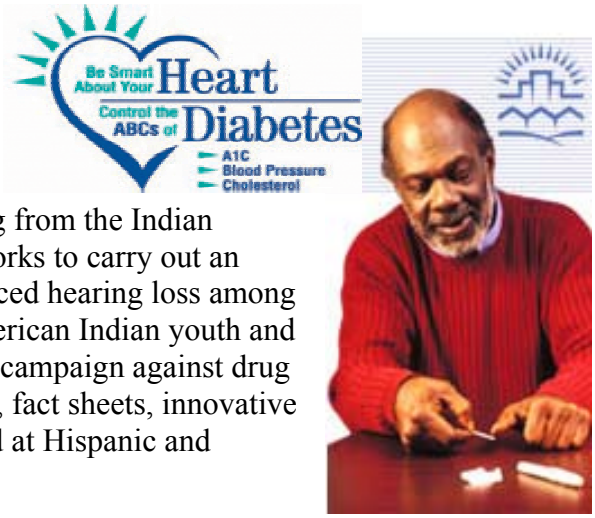
◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- **NIH develops and distributes health education materials to non-English speaking audiences.** NIH has successfully met all of its targets to date aimed at reaching non-English speaking and low-literacy audiences. Specifically, NIH established and launched a centralized site on the NIH home page offering access to NIH materials in Spanish. NIH also developed posters about type 2 diabetes that were translated into 11 languages for Asian American and Pacific Islander audiences. In addition, NIH wrote articles for Spanish-language newspapers (with a readership of about 800,000 people), developed a Hispanic/ Latino diabetes awareness campaign that included TV public service announcements (PSAs) in Spanish, and distributed Spanish-language booklets on the effects of drugs.

  Most recently, based on the National Cancer Institute's (NCI) Surveillance Epidemiology and End Results' cancer statistics showing that the Asian women's population has an increased breast cancer rate, NCI developed Asian language brochures entitled "Do it for Yourself, Do it for your Family." These brochures, printed in English, Chinese, Vietnamese, and Tagalog, communicate the importance of regular breast screening for early detection of cancer. NCI disseminated the brochures nation-wide, particularly in cities with high Asian populations, through Asian church groups, cultural organizations, women's organizations, and social organizations. Dissemination was completed in October 2002.

- **NIH reaches high-risk populations through communication campaigns.** NIH has met all of its targets to date to develop and implement communication campaigns for high-risk populations. Specifically, NIH launched five major diabetes education campaigns intended for those populations that suffer disproportionately from the disease–African Americans, American Indians, Asians and Pacific Islanders, and Hispanics/Latinos. NIH also created a coalition of partners ranging from the Indian Health Service to the Migrant Clinicians Networks to carry out an education campaign to help prevent noise-induced hearing loss among industrial workers, Hispanics/Latinos, and American Indian youth and their parents. To enhance its public awareness campaign against drug abuse, NIH developed and distributed booklets, fact sheets, innovative calendars, and radio and television PSAs aimed at Hispanic and American Indian audiences



  In FY 2002, NIH met its target to assist a network of 12 community-based centers that apply lessons learned about prevention, identification and treatment of heart disease to high-risk populations. The National Heart, Lung and Blood Institute (NHLBI), which funds the centers, provided technical assistance to help the centers launch and promote educational projects, share information within the network and with NHLBI, access daily health news

and other resources, and distribute state-of-the-science information about heart health. Specifically, NHLBI produced recruitment and promotional videos for the community-based projects, advised them on media outreach strategies for kick-off events, provided regular teleconference support for the network, created an extranet for the network, and provided the centers with numerous publications on heart health for distribution at events.

- **NIH successfully launched two campaigns to increase awareness on specific health issues.** After implementing the Breast Cancer and Mammography Education Program in FY 1999, NIH continued to develop campaigns addressing specific health issues. For example, NIH collaborated with over 2,700 YMCAs and YWCAs in FY 2001 to educate parents about childhood sports injuries and how to prevent them. The campaign included the development, distribution, and promotion of a fact sheet entitled, *Childhood Sports Injuries and Their Prevention: A Guide for Parents with Ideas for Kids*. In FY 2001, in preparation for the launch of a major HIV/AIDS vaccine clinical trial, NIH laid the groundwork for an awareness campaign by planning, researching, developing strategies, and meeting with stakeholders. The campaign, launched in May 2002, is designed to create an atmosphere in which the concept of a preventive HIV/AIDS vaccine is better understood by the general public, leading to more study volunteers.

  In FY 2002, a series of Spanish-language publications was developed for the Hispanic population at high risk for obesity and diabetes. The materials, created by the National Institute of Diabetes and Digestive and Kidney Diseases, were designed to help families, adults, and older Hispanic Americans adopt healthy eating and physical activity practices to reduce their incidence of disease. NIH also will target Hispanic Americans in a campaign to increase awareness about vision complications of diabetes. The National Eye Institute (NEI) completed planning, research, and development of campaign messages and strategies in FY 2002, in collaboration with intermediaries in Hispanic communities as well as a Hispanic community at high risk for eye diseases. NEI will refine the campaign based on results from three test markets. Originally scheduled for launch this year, the campaign will begin in FY 2003. It took longer than anticipated to gain community consensus on selection of focus group locations and test market sites, but in the end NIH gained the buy-in from Hispanic leaders and the community that will ensure success.

Goal c) Increase awareness of NIH-sponsored research results among the general public.

NIH disseminates new knowledge resulting from research as broadly as possible to increase public awareness. Often, the public can use information that results from NIH-sponsored research to improve their health and the health of their families. Therefore, the broader the dissemination of selected, widely applicable research results, the greater the number of individuals and families who are likely to benefit. To achieve this goal, NIH is focusing on 1) enhancing NIH operations to improve the communication of research results, 2) strengthening collaboration with other organizations involved in health communications, 3) developing and implementing communication campaigns on specific health issues, and 4) increasing the public's awareness of specific health issues and the role of NIH.

To improve the communication of research results, NIH is developing an easily navigable website intended for older Americans so they can more easily access health information; it also is planning a process evaluation of the effectiveness of pap test information materials.

To strengthen collaborations with other organizations involved in health communications, NIH develops relationships with constituency groups nationwide to increase awareness of the latest scientific information. To reach the broadest audience across the nation, NIH uses mass media materials distributed to the print media, cable networks, and broadcast television and radio stations in all 50 states.

Many Americans are unaware that NIH funding and basic research are major factors in almost every U.S. biomedical discovery. To increase public awareness of its crucial role, NIH uses mass media communications and promotes direct public access to information.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Enhance NIH operations to improve the communication of research results:						
Implement a system to track customer service interactions, measure response times, and record customer feedback on NLM products and services.	◆					
Provide a single toll-free telephone number to reach NLM customer service staff.	◆					
Complete the restructuring of NIMH's mental health education and information dissemination programs.	◆					
Increase the availability of consumer health information, publications, and reports under NIH's Centralized Consumer Health Information area by 20 percent.	◆					
Increase the usage of NLM's existing catalog-based databases for books, serials, and audiovisuals by 15 percent.		◆				
Increase the number of "health topics" in the web-based MEDLINEplus to 300.		◆				
Ensure that no less than 85 percent of respondents to a customer feedback instrument rate NLM services at least satisfactory.		◆				
Introduce an easily navigable site on the World Wide Web that can increase older adults' awareness of health information and, based on the National Institute on Aging-supported cognitive research findings, enhance the online learning experience for people age 60 and over.				→	◇	
Perform a process evaluation of the effectiveness of pap test information materials produced in FY 2001.				◆		
Launch three new services to enhance the online health information resource, MEDLINEplus.					◇	
Strengthen collaborations with other organizations involved in health communications:						
Strengthen relationships with universities, voluntary health associations, and other organizations that communicate health and scientific information to expand the options for communicating NIH research results.	◆					
Increase collaboration with professional associations of journalists, science writers, and health communicators to increase their coverage of NIH-funded research results.		◆				
Implement WISE EARS! communications program by developing a coalition of more than 70 groups representing government, industry, the worker, children and older individuals as well as organizations directly committed to preventing noise-induced hearing loss and providing them with the resources to reach children under 17, mid-life adults, and older Americans as a means to provide information about preventing fully preventable noise-induced hearing loss with messages in at least 50% of states by 2001.		◆				
Strengthen relationships with constituency groups nationwide to increase awareness of the latest scientific information about drug abuse and addiction prevention and treatment and to provide a channel for feedback about emerging grassroots issues.			◆			
Improve the public's access to health information by expanding the NLM's consumer health information program to ensure that a medical library in every state is working with public libraries and community organizations.			◆			

Performance Targets	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Increase the public's awareness of specific health issues and the role of NIH by developing and implementing communication campaigns:						
Develop and implement NIH information, education, and outreach programs on specific health issues: low vision.	◆					
Increase awareness among the general public about the achievements of publicly-funded vision research.			◆			
Expand the outreach of the "Milk Matters" campaign beyond parents and health professionals to focus directly on activities and products that help children and teens recognize the benefit of calcium in building strong bones.		◆				
Increase awareness among young people of the importance of calcium in their diet.			→	◆		
Develop campaign materials about the importance of calcium from milk and other sources for strong bones.				◆		
Generate a minimum of 30 million media impressions through placements in newspapers and magazines nationwide and on national and local television and radio programs, to raise awareness among all Americans of the importance of eating at least five servings of fruit and vegetables a day.		◆				
Increase the number of people who know the symptoms of stroke and rapidly seek treatment.			◆			
Implement a stroke awareness campaign.				◆		
Extend the impact of the *Know Stroke: Know the Signs. Act in Time.* campaign to increase awareness among the general public about the symptoms of stroke and the need to seek treatment rapidly by partnering with providers and volunteers in at least five communities to circulate materials and hold educational events.						◇
Increase the public's understanding of cancer research, advances, and opportunities.			◆			
Increase awareness among the general public that drug addiction is a brain disease.			◆			
Increase awareness among university presidents, program planners, and policy makers about college drinking and related problems.			→	◆		
Develop and implement a campaign for parents/guardians on the importance of early identification and treatment of hearing loss.						◇

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- **NIH enhanced its operations to improve the communication of research results.** Prior to this reporting year, NIH successfully implemented a computer system to track inquiries, measure response times, and record customer feedback on NLM services. A toll-free number was also established to improve the public's access to NLM customer service staff, which has generated more than 30,000 calls per year. In addition, NIH restructured the mental health education and information dissemination programs administered by NIMH. Enhanced operations also resulted in substantial increases in the number of on-line health information publications in NIH's Centralized Consumer Health Information area, the usage of NLM's

catalog-based databases, and the number of health topics on MEDLINEplus. An FY 2000 evaluation found that 98% of users surveyed rated NLM's services as satisfactory or better, and 92% rated the services as good or excellent. These results exceeded the target of 85% of users rating the services as satisfactory.

In FY 2002, the National Institute on Aging (NIA) and the National Library of Medicine (NLM) developed *NIH Senior Health*, a web site that presents aging-related health information in a senior-friendly format based on NIA's cognitive aging research. The site went up for beta testing on March 19, 2002 and has been tested with groups of older adults to ensure that they can use it easily. NLM is developing new technology to facilitate the expansion of the site before its launch, scheduled for FY 2003. When launched, the site will cover 10 health topics. Three topics were made available on the NLM's website in FY 2002.

Also in FY 2002, the National Cancer Institute (NCI) conducted a process evaluation of new and revised Pap test-related publications that are designed to raise awareness about the need for Pap tests, particularly among older women, and to increase awareness of the change in Medicare coverage from every 3 years to every 2 years. The evaluation included telephone surveys of health professionals who picked up the publications at national conferences and tracking activities to measure use of a press release, print public service announcements, online publications, and targeted web sites. A formal final report summarizing the lessons learned regarding materials dissemination and usage was issued (*Cervical Cancer Evaluation Support: Final Report* - Process Evaluation of Health Professional Pap Test Packet, Press Release, and PSA; March 2002).

- **NIH strengthened collaborations with other organizations involved in health communications.** NIH has met all of its targets, which included strengthening its relationships with universities, voluntary health associations, and other organizations involved in health communications. For example, in FY 2000 NIH developed a broad coalition for the WISE EARS! communications campaign, which included 78 organizations. In FY 2001, NLM collaborated with the National Network of Libraries of Medicine to ensure that at least one NLM-supported medical library in every state is working with public libraries and other community organizations to improve the public's access to reliable electronic health information. NIH has continued to collaborate with professional associations of journalists, science writers, and health communicators to promote coverage of NIH-funded research results.

- **NIH increased public awareness by initiating communication campaigns.** In FY 1999, FY 2000, and FY 2001 NIH successfully developed and implemented campaigns on vision, nutrition (calcium, milk, fruits and vegetables), stroke, cancer, and drug addiction. In FY 2002, NIH launched a new, expanded, version of the "Milk Matters" website http://www.nichd.nih.gov/milk/ to provide more information for parents and children on the importance of calcium for strong bones and how to get enough calcium for bone health. Much of the website and associated activities are designed to increase awareness among young people of the importance of calcium in their diet. The Website has received considerable media coverage including a brief article in the Boston Globe. The website includes a page that allows visitors to view and order calcium education materials. In

addition, many of the materials can be downloaded and bulk quantities of the materials can be ordered from the NICHD Information Resource Center. Building on the website, the National Institute of Child Health and Human Development (NICHD) partnered with the Powerful Bones Powerful Girls campaign to distribute calendars, stickers, and other campaign materials concerning the role of calcium in building strong bones. NICHD also developed the Website in Spanish. In December 2002, NICHD expanded the website to include games and quizzes to engage children and teens while teaching them about calcium's role in health and sources of calcium.

NIH also continued to implement the public education campaign launched in May 2001, *Know Stroke. Know the Signs. Act in Time*. The campaign has been well received, and several organizations have incorporated campaign materials into their activities. For Stroke Awareness Month in May 2002, National Institute of Neurological Disorders and Stroke (NINDS) conducted community education activities by initiating a partnership with the American Stroke Association (ASA) to implement the *Know Stroke* campaign. Five ASA chapters co-branded *Know Stroke* materials and distributed them to hospitals and senior organizations in their markets. In addition, the Centers for Medicare and Medicaid Services sent community education kits to its 47 peer review organizations, which in turn disseminated materials to hospitals in all States. In the Southeast U.S., known as the Stroke Belt, the Tri-State Stroke Network co-branded and disseminated *Know Stroke* materials to public health departments and health care providers in North Carolina, South Carolina and Georgia. NINDS also placed public service messages, for example, messages that aired in 10 major markets during morning news shows such as *Today* and *Good Morning America* to an estimated audience of 2.1 million viewers. Finally, NINDS made media outreach efforts. NINDS stroke experts conducted radio interviews in five major U.S. markets, including Boston, Washington, and San Francisco. Experts also were interviewed on three nationally syndicated radio shows, including one for AARP and one for the American Urban Radio Network.

In FY 2002, The NIAAA Task Force on College Drinking released several publications on the effectiveness of current alcohol prevention strategies and recommendations on future research to improve college drinking prevention programs, including a report titled, *A Call to Action: Changing the Culture of Drinking at U.S. Colleges*; 24 commissioned papers, most of which were published in a special supplement of the March 2002 issue of the *Journal of Studies on Alcohol*; and brochures targeting specific audiences, such as parents, peer educators, and resident advisors. Publication of the report was delayed from FY 2001 to April 2002 in order to ensure that new research findings could be included. The report and accompanying materials have been sent to all college and university presidents in the United States. Follow-up activities have included a series of workshops for college presidents, alcohol prevention staff, and local government officials.

Goal d) Increase awareness of clinical research and support participation in clinical trials.

Increasing public awareness of NIH's need for clinical trial participants is one of the agency's greatest communications challenges. NIH researchers conduct clinical trials to determine if new drugs or treatments are safe and effective. Because carefully conducted clinical trials are the fastest and safest way to find treatments that work, NIH needs a steady, diverse, and substantial pool of volunteers including patients and members of the general public. NIH's ability to improve the nation's health care depends on physicians having the opportunity to refer patients to current studies and on patients having the information they need about participating in clinical trials.

In striving to achieve this challenging goal, NIH has focused on increasing clinical trial participation by 1) strengthening collaborations with other organizations and 2) developing web-based tools and other materials. Because increasing and maintaining awareness regarding clinical trials is difficult, NIH continues to build collaborative relationships with physicians, community groups, and consumer and advocacy groups from across the nation. Web-based tools also serve as important mechanisms for disseminating information to encourage participation in clinical trials.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Strengthen collaborations with other organizations to increase clinical trial participation:						
Initiate a broad-based communications and public outreach program to reach physicians, and eventually, community groups and the general public.	◆					
Build and maintain networks of communication and support for clinical research between NIMH and consumer and advocacy organizations and professional groups nationally, regionally, and locally.		→	◆			
In partnership with community-based organizations, develop rheumatic disease health education materials and increase awareness of opportunities to participate in clinical studies.				◆		

Performance Targets	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Develop web-based tools and other materials to increase clinical trial participation:						
Develop methods and materials to improve communication with minorities and ethnic groups and to encourage them to participate in (NIMH-sponsored) clinical research, thus meeting a critical public health need.		→	◆			
Increase visitors to NCI's CancerTrials website and the amount of information about cancer trials to patients, health professionals, the public and the media in all areas including prevention, detection, diagnosis, and treatment.		◆				
Develop simplified and easy-to-understand informed consent forms to help patients better understand a study's treatments and tests and their possible benefits and risks before deciding whether or not to participate.		◆				
Develop web-based clinical trials tools that will improve the development, conduct, and ease of participation in NCI-sponsored clinical trials.			◆			
Improve NCI efforts to increase participation and retain minorities, underserved populations, and the elderly in clinical trials.			◆			
Educate the public about the importance of NIMH-supported clinical research and interest individuals and their families in participating in clinical studies.			◆			
Increase the number of initial contacts about clinical trials with the Patient Recruitment and Public Liaison Office (PRPL).			◆			
Develop messages and materials about participating in clinical studies.					◇	

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	× Target Not Met

Summary of Performance Results

- **NIH successfully strengthened collaborations with other organizations to increase clinical trial participation.** Although NIH did not fully achieve the FY 2000 target to build and maintain networks of communication and support between NIMH and other organizations, a major five-year communications initiative was successfully launched in FY 2001. The new initiative, called the Constituency Outreach and Education Program (COEP), enlists state organizations in a nationwide partnership to help speed the translation of science into mental health services.

  In FY 2001 and FY 2002, the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS) in collaboration with local community partners and national voluntary organizations, produced 14 English, Spanish, bilingual, and easy-to-read publications on rheumatic diseases. Specific messages about participation in clinical trials appear in some of the publications. The Institute also developed a newsletter, IRPartners, for clinical trial participants and people interested in intramural research program activities. The NIAMS-funded Community Health Center in Washington, D.C. offers local rheumatic disease patients the opportunity to participate in research studies. In its first 14 months of operation,

the Center, in collaboration with community leaders, has increased participation of people from minority communities in NIH clinical studies, including more than 300 patients seen at the Center.

- **NIH developed new web-based tools to increase clinical trial participation.** NIH met all of its targets to date to develop and maintain web-based tools and other materials designed to educate the public about clinical trials and how to participate in them. Many of the communication activities were directed toward minorities, ethnic groups, and other populations that historically have been underrepresented in clinical trials. For example, NIMH carried out a variety of media activities to increase enrollment in trials, with special attention given to minority audiences. In addition, NCI created the Special Populations Network to address the unequal burden of cancer experienced by certain population groups, focusing on building strong relationships with research institutions and community-based programs. In FY 2001, NCI extended its outreach efforts to more medical institutions that reach underserved populations including Meharry Medical College (MMC) and the MMC/Vanderbilt-Ingram Cancer Center Partnership. NCI also developed a number of communication tools to increase participation in clinical trials among minority groups and the elderly. Ongoing NCI efforts in this area have raised the proportion of minority participation in treatment clinical trials to nearly 20 percent.

  To educate the general public about participating in clinical trials, NIMH developed new web-based and print materials. NCI also developed several web-based informatics "products" to improve the reporting and exchange of clinical trial information, including a clinical data update system and an adverse event reporting system. NCI continued to update its clinical trials database, Physician Data Query (PDQ), which contained more than 1,800 protocols at the end of FY 2001. Also in FY 2001, the Patient Recruitment and Public Liaison Office within the NIH Clinical Center achieved a 37% increase in the number of inquiries it received about participating in clinical trials. Accomplishments in previous years included the development of a simplified informed consent form and a considerable increase in the range of information available on NCI's CancerTrials website and number of website hits.

Goal e) Establish a Clinical Trials Database, as required by the FDA Modernization Act.

Many of today's most effective interventions are the direct result of knowledge gained through clinical trials–studies that evaluate the safety and effectiveness of new drugs and other interventions. Therefore, facilitating access to information on clinical trials is an important national goal. In accordance with this goal, the Food and Drug Administration Modernization Act (FDAMA) of 1997 mandated the establishment of a registry of both Federally and privately funded clinical trials of experimental treatments for serious or life-threatening diseases submitted under Investigational New Drug (IND) regulations.

In collaboration with other Federal agencies, the pharmaceutical industry, and academic and other nonprofit organizations, NIH developed the Clinical Trials Database, a consolidated source of information on clinical trials for drugs for serious or life threatening diseases and conditions. Prior to the development of this database, the NIH homepage provided access to eight separate clinical trials databases: NCI Physician's Data Query (PDQ), AIDS Clinical Trials Information System, Clinical Center Studies, Rare Disease Clinical Trials database, NEI Clinical Trials database, NHLBI Clinical Trials database, NIA Alzheimer's Disease Clinical Trials database, and NIAID Clinical Trials database.

Improving access to clinical trial information is critical to increasing public and provider awareness, understanding, and willingness to participate in clinical trials. NIH continues to focus on improving and promoting the Clinical Trials Database, including investigating the establishment of toll-free telephone access to the database. Toll-free access can be complex and expensive due to uncertainties regarding the demands that might be placed upon the system (i.e., the number of calls), the many possible designs for the system, and the varying levels of service that might be provided. In FY 2001, NIH completed an implementation study to determine the optimal design for the toll-free telephone service, along with operating strategies and associated costs. NIH is in the process of reviewing the results of the implementation study and discussing the potential allocation of resources.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY[1] 2003	FY[1] 2004
Improve and promote the Clinical Trials Database:						
Develop and implement the Clinical Trials Database.	◆					
Expand the Clinical Trials Database to include trials from other Federal agencies and the private sector.		◆				
Develop options for implementation of toll-free telephone access to information in the Clinical Trials Database.		→	◆			
Complete an implementation study to determine the optimal design and function of a toll-free telephone to facilitate access to the Clinical Trials Database.			◆			
Expand the number of industry-sponsored clinical trials in the database by 250.			→	◆		
Expand the number of clinical trials in the database sponsored by other Federal agencies by 100.			◆			
Promote the database as a resource for patients, physicians, researchers, community health groups and others.			◆			

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	× Target Not Met

Summary of Performance Results

- **NIH made considerable progress in improving and promoting the Clinical Trials Database.** At the end of FY 2001, the website for the Clinical Trials Database (ClinicalTrials.gov) was receiving about 2 million hits per month and hosting approximately 5,300 visitors each day. NIH had previously met its FY 1999 and 2000 targets to develop a prototype system, implement the official Clinical Trials Database, make it available to the public (which occurred on February 29, 2000), and expand the database to include clinical trials sponsored by other Federal agencies and the private sector. By the end of FY 2001, the number of trials in the database had risen to 5,771. The number of trials sponsored by other Federal agencies more than tripled from 104 in FY 2000 to 383 in FY 2001, far exceeding the target, and the number of industry-sponsored clinical trials increased by 109. NIH worked closely with the Food and Drug Administration to develop a guidance document outlining the steps and procedures for companies to provide information on their clinical trials to ClinicalTrials.gov. The FDA Final Guidance Document was released in March 2002. As a result, companies have been actively working to send their trials to ClinicalTrials.gov and the FY 2001 target was met a few months into FY 2002. By the close of FY 2002, the number of industry sponsored clinical trials stood at over 1200.

[1] This goal has been met and will be dropped from future GPRA plans.

2.1.3.3 Technology Transfer

The broad purpose of NIH's technology transfer activities is to facilitate and enhance the development of new drugs, other products, and methods of treatment that benefit human health by promoting the efficient transfer to the private sector of new technology resulting from NIH research. Federal legislation empowers NIH to interact directly with industry to expedite the transfer of technological discoveries into commercial products that will benefit the public. In addition to improving public health, technology transfer contributes to the global competitiveness of the nation's businesses and to the nation's economic prosperity.

NIH's technology transfer program is one of the most active in the Federal Government. NIH patents new drugs, vaccines, devices, and other products developed by its scientists and issues licenses to organizations in the private sector that are interested in the commercial application of these products. To protect the public's research investment, NIH oversees licensee progress and receives royalties from licensees based on product sales. NIH has forged numerous partnerships with industry and other external research organizations, thereby enhancing its capacity to expedite the commercial application of new technologies.

To achieve its full potential in this area, NIH faces a number of challenges: 1) developing effective, well-articulated technology transfer policies and procedures that are understood by NIH scientists, 2) building the organizational structure necessary to facilitate technology transfer for NIH-supported investigators and measure outcomes, and 3) monitoring licensee activities and taking appropriate action against infringers of NIH intellectual property rights. NIH is working with Congress, DHHS, and its research partners to establish and implement rational technology transfer policies that facilitate the patenting and licensing of new technologies and the expansion of cooperative research projects within the NIH intramural research program.

To address these challenges and enhance its technology transfer efforts, NIH established three performance goals:

a) Increase the number of scientists who have received training in technology transfer.

b) Develop a system to identify and measure the health outcomes of technologies licensed by the NIH.

c) Maintain oversight and protection of the public investment in NIH research through increased monitoring of licensee activities.

NIH's efforts to achieve these performance goals include developing new training modules on technology transfer procedures, designing a methodology to measure the health outcomes of NIH-licensed technologies, and developing systems to monitor the progress of NIH licensees and ensure that royalties owed to NIH are paid in a timely manner.

Performance Goal Details - Technology Transfer

Goal a) Increase the number of scientists who have received training in technology transfer.

To maximize NIH's ability to offer cutting-edge technology to the private sector for further development and commercialization, NIH's intramural researchers and technology transfer staff must have access to up-to-date information on the laws, policies, and procedures that govern technology transfer. They must have full knowledge of the Federal legislation that empowers NIH to interact directly with industry to expedite the transfer of new technology.

To ensure that NIH scientists (approximately 4,000[1] research investigators) are able to take advantage of the varied opportunities for translating research results into commercial products, NIH has undertaken two major activities: 1) designing and implementing a web-based training module on technology transfer procedures, and 2) increasing the percent of NIH scientists who have received training in technology transfer. In addition to developing and encouraging participation in the web-based training module, NIH will continue to provide opportunities for its scientists to attend seminars and annual retreats focused on technology transfer policies and related information.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Design and implement a web-based training module on technology transfer procedures:						
Contractor development of a web-based training module.	→	◆				
Implement training module.		→	◆			
Increase the percent of NIH scientists who have received training in technology transfer:						
Contact 20% of NIH scientific staff.		→	◆			
Seek to have 15% of scientists complete the training module and/or attend technology transfer seminars.			◆			
200 scientists complete the web-based training module.				◆		
1,000 scientists complete the web-based training module.					◆	
Seek to have the web-based training module incorporated as a standard requirement for all new scientists at NIH.						◆

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	× Target Not Met

[1] In addition to principal investigators, this includes postdoctoral fellows and others.

Summary of Performance Results

- **NIH successfully implemented the web-based training module on technology transfer procedures.** Although NIH encountered software development delays, the web-based training module was activated successfully in the first month of FY 2001 (October 2000).

- **Almost two-thirds of NIH scientists have received training in technology transfer.** Approximately 2,450 of the 4,000 NIH scientists (63%) attended the training seminars. As a result, NIH surpassed its training targets for FY 2001, FY 2002, and FY 2003. During FY 2002, over 1000 scientists were trained using the NIH On-Line Technology Transfer Training module. Specifically, 1231 scientists completed the web-based module, and the module was incorporated as a standard requirement for all new scientists at NIH. Furthermore, NIH also met the FY 2004 target of making completion of the NIH On-Line Technology Transfer Training an ongoing requirement for employees. With completion of all targets, this goal has been met and will be dropped from future GPRA plans.

Goal b) Develop a system to identify and measure the health outcomes of technologies licensed by NIH.

Effectively measuring the public health outcomes that result from technologies licensed by NIH is challenging and complex. Traditionally, efforts to measure the impact of technology transfer activities have focused on countable outputs, such as the number of licenses issued. However, this approach does not provide very useful information and may, in fact, distort the importance of NIH's technology transfer achievements.

To address this challenge, NIH established in FY 2001 a new goal to develop a system for measuring key outcomes of interest to managers and policymakers. NIH's strategy for developing a better measurement system focuses on designing and testing a methodology for measuring the outcomes of new drugs, vaccines, and devices. The first step was to establish an internal working group to identify potential data sources and outcome measures. Then specific data sources and outcome measures were selected and NIH conducted a limited pilot-test of the proposed methodology. Next, NIH will apply the final methodology to a sample of licensed technologies that have been incorporated in commercially available products.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Design and test a methodology for measuring outcomes of new drugs, vaccines, and devices.						
Establish a working group in the Office of Technology Transfer and obtain recommendations on potential outcome measures and sources for obtaining reliable data for those measurements on therapeutic drugs, vaccines, and devices.			→	◆		
Develop two case studies to test the proposed methodology.				◆		
Finalize the approach and apply the methodology to 10% of all exclusively licensed technologies that are part of commercially available products.					◇	
Review and modify the methodology as needed and continue to apply the metrics to at least 10% of all exclusively licensed technologies that are a part of commercially available products.						◇

◆ **Target Met**	◇ **Target Active**	→ **Target Not Met and Extended**	✕ **Target Not Met**

Summary of Performance Results

- **An NIH working group recommended health outcomes measures and conducted two case studies to test the proposed methodology.** During the FY 2002 meetings of the NIH Health Outcomes Working Group, background research, potential study design, and prospective sources of data were considered and the working group recommended potential measures of the outcomes from introduction of therapeutic drugs and vaccines. The potential

measures included dosages prescribed or used, reduction in mortality/morbidity, reduction in number of sick days used, and extension of life. The group also suggested reliable sources of data for the outcome measures. In addition, the working group selected two products to utilize as case studies. In FY 2002, to test a methodology for measuring outcomes of new drugs, vaccines, and devices, the working group completed two pilot case studies analyzing the public health outcomes of two commercially available products incorporating exclusively licensed NIH technologies. The reports are located on the NIH Office of Technology Transfer website (ott.od.nih.gov).

Goal c) Maintain oversight and protection of the public investment in NIH research through increased monitoring of licensee activities.

Whereas the transfer of technology from research to commercialization is an important concern, it is equally important to ensure that licenses of technologies to commercial entities are implemented properly and that the correct amount of royalties is paid to the government. To minimize the risks associated with noncompliance with licensing agreements, NIH established a new goal in FY 2001 to implement a new monitoring strategy for licensed technologies.

The monitoring effort consists of three major activities: 1) designing and implementing a system for monitoring the progress of NIH licensees, 2) ensuring that royalties owed to NIH are made in a timely and appropriate manner, and 3) pursuing litigation against entities who are infringing on NIH intellectual property rights.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY[1] 2003**	**FY[1] 2004**
Design and implement a system for monitoring the progress of NIH licensees:						
Recruit and select personnel to establish a special license-monitoring unit to provide oversight of licensee progress in developing and commercializing technologies licensed from NIH.			→	◆		
Implement a data system that includes all license monitoring milestones and benchmarks for all exclusive licenses.				◆		
Ensure that royalties owed to NIH are made in a timely and appropriate manner:						
When indicators show that sales and royalty information may be incorrect, conduct audits of up to three licensees during the year.			◆			
Reduce the number of delinquent payments over 180 days by 50% from the number in place at the end of FY 2000.			◆			
Reduce the number of terminated licensees with outstanding royalty amounts owed by 10% from the number at the end of FY 2000.			◆			
Reduce delinquencies over 180 days and bring that number to zero by the end of FY 2002, except for cases that are being actively negotiated due to the affect on public health.				◆		
Ensure that all delinquent payments associated with terminated licenses will be submitted to the NIH Debt Collection Officer within 120 days of termination.				◆		
Perform audits on up to 3 licensees during the year, if warranted.				◆		
Pursue litigation against entities who are infringing on NIH intellectual property rights:						
Develop and implement a process to refer infringers of NIH intellectual property rights to the Department of Justice with recommendations for bringing a Federal lawsuit against such entities.			◆			

[1] This goal is part of the normal technology transfer process and will not be continued past FY 2002.

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- **Design and implement a system for monitoring the progress of NIH licensees.** NIH recruited personnel to establish a license-monitoring unit. Following a long recruitment effort that was initially unsuccessful, two Technology License Monitoring and Enforcement Specialists were selected in FY 2002. This unit will oversee the progress of licensees towards commercializing technologies licensed from NIH.

  NIH established a data system for monitoring license milestones and benchmarks. Launched October 1, 2002, the new data system contains a module to monitor the movement of licensees toward meeting milestones and benchmarks.

- **Ensure that royalties owed to NIH are made in a timely and appropriate manner.** NIH reduced the number of delinquencies over 180 days to zero; and reported all delinquencies for debt collection. NIH took numerous steps in FY 2001 to ensure that royalties owed to NIH were paid. Audits were conducted on 5 licenses, which exceeded the target (3 were planned). Revised procedures enabled NIH to reduce the number of delinquent payments (over180 days late) from 78 in FY 2000 to 35 in FY 2001. That decrease represented a 55% reduction in the number of delinquent payments owed, which was 5% more than targeted. By the close of FY 2002, there were no outstanding cases over 180 days delinquent. Additionally, NIH improvements led to the decline in the number of terminated licenses with outstanding balances from 21 in FY 2000 to only 12 in FY 2001. Furthermore, a method was employed to ensure that all delinquent payments associated with terminated licenses are automatically submitted to the NIH Debt Collection Officer within 120 days.

  NIH conducted two audits found to be warranted during FY 2002. The two audits were completed and requests for support of three additional audits were submitted to NIH Institutes and Centers for action. These three audits will be conducted in FY 2003.

- **Pursue litigation against entities who are infringing on NIH intellectual property rights.** NIH implemented a new litigation process against infringers. A process for referring infringers of NIH intellectual property rights to the Department of Justice has been developed and implemented. NIH has initiated three cases; one was settled before being filed with the court and two are pending.

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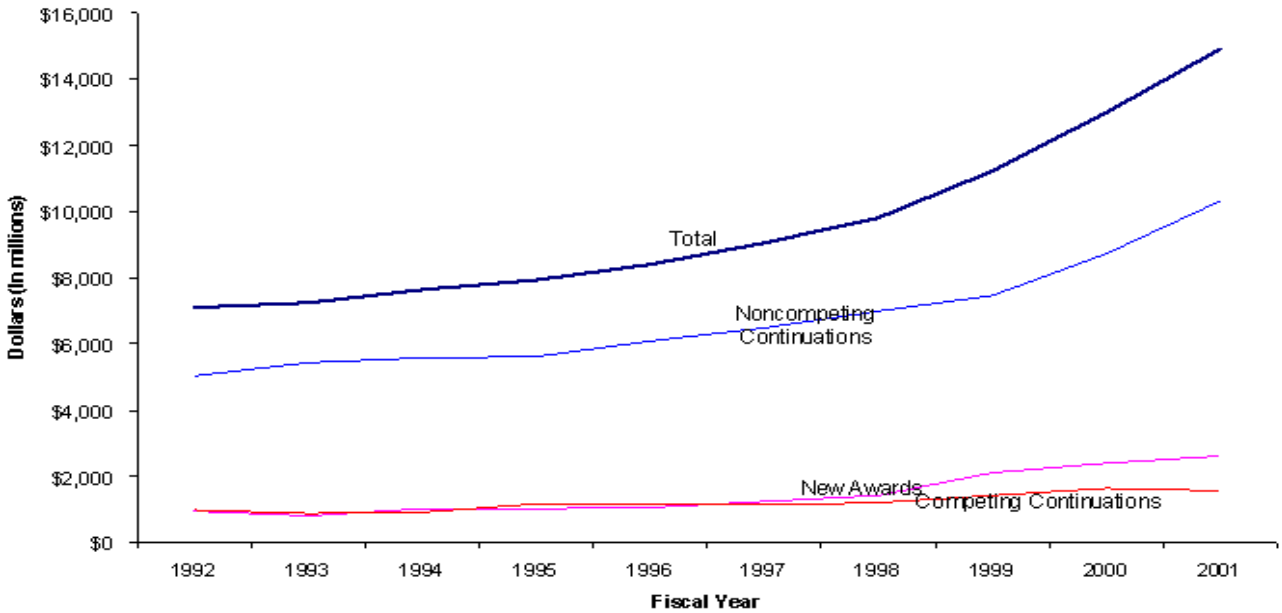
2.1.3.4 Grants Administration and Peer Review

Approximately 80 percent of NIH's budget supports research conducted in universities, medical centers, and other institutions around the country, with most of the research funding provided through competitive research grants. Therefore, it is essential for NIH to maintain effective and efficient processes for reviewing, selecting, and administering extramural research grants.

To ensure that the most meritorious research projects are considered for funding, NIH employs a peer review process in which prominent scientists from around the country evaluate each request for support, and through this process, provide advice to NIH staff in the selection process. NIH strives to ensure that the peer review process is fair, reflective of state-of-the-art knowledge of the science being reviewed, and able to accommodate the many applications for research support and training that NIH receives. After NIH awards funds to a particular project, administrators in the sponsoring Institute or Center (IC) provide oversight to ensure that the research is carried out appropriately.

In addition to ensuring that the highest quality research is selected for support, NIH is committed to providing support to investigators in a timely fashion so that their research can proceed efficiently. The expenditure of the nation's financial resources for the conduct of research also demands appropriate oversight, which requires effective grants administration, prompt customer service, and efficient communication and reporting processes. Related challenges include strengthening NIH's capacity to receive and transmit grant information via the Internet using electronic Research Administration (eRA) technology and improving the Edison electronic invention reporting system.

NIH Research Grants
New, Competing, and Noncompeting Continuations and Supplements
(Supplements are included in the total)



To address these challenges and enhance the effectiveness of grants administration and peer review activities, NIH established five performance goals:

a) Improve electronic research administration (eRA) technology by developing capability for end-to-end electronic research administration by 2004.

b) Ensure proper stewardship of public funding for research.

c) Ensure that the NIH peer review process keeps pace with current advances in research and that the expertise of the peer reviewers is appropriate for the needs of modern science.

d) Develop innovative business practices to facilitate government/public interactions.

e) Improve grantee reporting of inventions developed with Federal funds.

NIH's efforts to achieve these performance goals include developing new modules for the NIH Commons system to enable NIH to receive grant applications and progress reports electronically, simplifying the administrative processes required of grantees, enhancing the design of the Edison invention reporting system, improving the organization of the peer review system, and increasing educational outreach to improve institutional compliance with NIH requirements.

Performance Goal Details – Grants Administration and Peer Review

Goal a) Improve electronic Research Administration (eRA) technology by developing capability for end-to-end electronic research administration by 2004.

eRA is NIH's infrastructure for conducting interactive electronic transactions for the receipt, review, monitoring, and administration of NIH grant awards to biomedical investigators worldwide. The objective of eRA is to eliminate the hundreds of millions of pieces of paper currently generated each year during application, initial peer review, secondary council review, award and post award administrative processing of the approximately 44,000 research and training applications received each year. eRA integrates two parallel systems–the NIH Commons and IMPAC II. The NIH Commons enables NIH communication with over 2,000 research institutions worldwide[1]; IMPAC II supports efficient data transmission for over 5,000 extramural staff conducting extramural business at NIH. eRA is mandated by Public Law 106-107, which requires Federal agencies to migrate from paper-based to electronic systems. ERA systems development incorporates government-wide standards and will integrate with the other NIH, HHS and Federal Commons systems.

NIH is placing significant emphasis on development of the NIH Commons systems to accommodate electronic application and progress report submission. Major activities in this area include 1) designing and testing several new modules within the NIH Commons system, 2) testing and implementing the electronic receipt of grantee progress reports, and 3) testing and implementing the electronic receipt of competing R01 applications. The following components of the NIH Commons are in various stages of development/deployment:

- The Grant Applications/Awards Status module allows investigators and grant administrative officials to obtain information about the current status of their pending grant applications and awards electronically.
- The Electronic Notice of Grant Award (NGA) feature enables NIH grant and cooperative agreement recipients to receive the official notification of awards electronically.
- The Streamlined Noncompeting Award Process (SNAP) modules enable principal investigators and administrative officials at grantee institutions to replace the hard copy Form 2590 with electronic submission of the annual progress report and other information required for continuation of a multi-year grant award. There are two SNAP modules: e-SNAP handles the simpler awards and CNAP handles the more complex awards.

[1] Includes all forms of grantee organizations, e.g., academic, small business, non-profits, etc.

- The Accounts Administration (Admin) module permits the addition, deletion, and modification of user accounts and allows grants administrators and principal investigators to view and update professional and institutional profiles electronically.
- The Trainee Activities System (X-Train) module allows grant administrative officials to submit and obtain information about their trainee appointments electronically.
- The Fellowships module will enable fellowship applicants and administrative officials to submit an electronic application for an individual PHS National Research Service Award (NRSA), replacing the hard copy Form 416-1.
- The Competing Grant Award Process (CGAP) module will enable principal investigators and grant administrative officials to submit an electronic application for an R01 or other type of competing grant award, replacing the hard copy Form PHS 398.

Guiding the NIH Commons development effort is a Commons Working Group made up of representatives from a cross-section of positions at research institutions. The Commons Working Group serves to publicly represent the interests of the extramural community by working closely with NIH policy, grants management, program, and review staff to make recommendations on system requirements. The 65 research universities and non-profit research centers participating in the Federal Demonstration Partnership (FDP) are being used to pilot-test the new NIH Commons modules.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Design and test new modules within the web-based NIH Commons system:						
Design and test new systems.	◆					
Streamline post-award reporting while continuing to ensure appropriate oversight and enhancement of recipients' compliance with reporting and accountability requirements.	◆					
Full deployment of key eRA business process modules.		◆				
Release NIH Commons modules in the new architecture.				◆		
Test and implement electronic receipt of grantee progress reports:						
Implement electronic progress reporting with all 65 newly on-line institutions participating in the Federal Demonstration Partnership.		→	→	→	◇	
Begin pilot testing of progress reporting for multi-project mechanisms.		→	→	→	→	→
Expand availability of electronic progress reporting to all grantee institutions.					◇	
Test and implement electronic receipt of competing R01 applications:						
Scan all incoming competing R01 applications in preparation for pilot testing receipt of R01 applications in 2003.				◆		
Pilot-test electronic receipt of simple (non-clinical, non-human) competing R01 applications.						◇

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- **NIH designed, tested, and deployed new modules in the NIH Commons system.** Prior to FY 2002, NIH deployed a series of NIH Commons modules to a limited audience. Information gained was used to strengthen the design of the architecture and features of the new modules. Four modules were deployed in the new architecture in August 2002, and three more were deployed in the fall of 2002. NIH continues to work closely with other agencies to develop standard data dictionaries that will be compatible with the Federal e-Grants initiative.

- **More effective electronic progress reporting technology was pilot tested in FY 2002.** During previous pilot testing, NIH found the technology used in the prototype for electronic receipt of grantee progress reports to be inadequate. As a consequence, both FY 2000 targets were reassessed and extended to FY 2002. In FY 2001, NIH developed the infrastructure needed to support a more effective progress reporting technology. Pilot deployment of electronic progress reporting began in November 2002. NIH expects the electronic progress reporting system to open to the FDP institutions in early 2003. Electronic progress reporting for multi-project mechanisms has been reprioritized to align the NIH focus more closely with the Federal e-Grant emphasis on the competitive application process. Accordingly, development of a system to accommodate progress reporting for multi-project mechanisms has been postponed until 2005.

- **Testing and implementation of electronic receipt of competing R01 applications is on schedule for FY 2004.** All incoming applications are being scanned and made available electronically to NIH staff as of January 2002.

Internal systems have been deployed with features to accommodate end-to-end electronic processing of competitive grant applications (electronic handling from the submission of applications, through their processing and award, to reporting). In spite of delays with regard to particular targets, NIH anticipates achieving the goal of capability for end-to-end electronic research administration by 2004.

Goal b) Ensure proper stewardship of public funding for research.

With the receipt of NIH grant awards or other types of public funding for research, principal investigators and grantee institutions accept the responsibility to conduct scientific studies ethically and honestly and to provide proper stewardship of NIH funds. Because of the nature of the assistance relationship, which is predicated largely upon trust between the sponsor (NIH) and the recipient (grantee institution), the need for effective internal and external compliance programs is essential. To minimize the risks associated with noncompliance, NIH established a new goal in FY 2001 to ensure proper stewardship of public funding for research. This crosscutting goal involves NIH Institutes and Centers working in partnership with grantee institutions and national professional organizations to improve institutional compliance with NIH requirements.

NIH's strategy for enhancing compliance is to develop a proactive grants compliance program. The program currently focuses on the following major activities: 1) enhancing administrative oversight by creating a new organizational component within NIH with the capacity to perform annual proactive compliance site visits, 2) increasing educational outreach by providing compliance seminars and providing web-based information and tools to help grantees understand their stewardship role and improve their institutional compliance programs, and 3) creating an internal NIH compliance program to provide management control and exercise oversight for implementation of grant-related policies. The compliance program will involve a newly established Internal Controls Board.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Enhance NIH's administrative oversight of sponsored research:						
Create an organizational component within NIH with FTEs devoted expressly to compliance-related activities.			◆			
Perform a minimum of 10 compliance site visits.			×			
Conduct eight proactive compliance site visits to grant recipient research institutions.				◆		
Conduct five proactive compliance site visits.					◇	◇
Perform a risk assessment and develop a plan for reviews of compliance with grant-related policies.					◇	
Begin internal compliance reviews.						◇
Increase educational outreach to improve institutional compliance with NIH requirements:						
Publish a compendium of observations and examples of compliance in action in the conduct and administration of sponsored programs at NIH's recipient institutions.				◆		

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Provide Internet-accessible resource information and/or tools for implementing institutional compliance programs.					◇	

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- **Administrative oversight of sponsored research was enhanced.** NIH established the Division of Grants Compliance and Oversight in August 2001 with three full-time equivalents (FTEs) assigned to compliance-related activities. NIH also completed 8 proactive compliance site visits in FY 2001. Based on the findings and recommendations from the FY 2000 site visits, NIH determined that focusing on different types of institutions (e.g., Historically Black Colleges and Universities and Research Institutes) would be more productive and efficient than visiting the 10 originally planned institutions. The Division of Grants Compliance and Oversight completed the eight proactive compliance site visits planned for FY 2002.

- **NIH is increasing educational outreach to improve institutional compliance.** By the end of FY 2002, NIH will have performed dozens of presentations and seminars on compliance-related topics. A compendium of findings and observations that integrates findings from the FY 2000 and the FY 2001 proactive compliance site visits was completed in FY 2002 and posted on the grants Compliance and Oversight web pages at http://grants1.nih.gov/grants/compliance/compendium_2002.htm. Interactive Internet tools will be available on the NIH website in FY 2003.

Goal c) Ensure that the NIH peer review process keeps pace with current advances in research and that the expertise of peer reviewers is appropriate for the needs of modern science.

NIH's peer review system is recognized as the cornerstone of the NIH extramural program because it is the principal mechanism by which the Institutes and Centers identify high-quality research that is worthy of funding. Established over 50 years ago, the system has been outstandingly successful and, in fact, may be the most important single reason for the remarkable success of our Federally funded biomedical research enterprise. The NIH Center for Scientific Review (CSR) manages the peer review process for approximately three-fourths of the more than 44,000 research and training applications submitted to NIH each year. Grant applications are initially reviewed for scientific and technical merit by peer review groups (study sections), which are composed primarily of non-government research scientists.

For decades, the individual study section served as the functional unit of review. To enhance the review process, NIH more recently adopted the Integrated Review Group (IRG), a cluster of scientifically related study sections, as the functional unit of review. The IRG is an administrative unit including a number of study sections encompassing a broad scientific domain (analogous to an academic department). Currently in CSR there are 150 standing study sections clustered within 20 IRGs.

To ensure that the NIH peer review process can adequately address the rapid progress in biomedicine and accelerating rate of change, NIH has undertaken an initiative designed to improve the organization of the peer review system in a way that allows it better to keep pace with advances in science.

A comprehensive examination of the organization and function of the CSR peer review system is being conducted by the Panel on Scientific Boundaries for Review (PSBR), a group established by the CSR Advisory Committee in 1998. It consists of highly recognized experts in the biomedical and behavioral sciences. The Panel's Phase 1 report, completed on schedule in FY 2000, identified a current need for 24 IRGs, 17 of which would require the design of new study sections. In the Phase 2 process, internal Steering Committees were established for each of these 17 IRGs to identify their key scientific areas and to recommend experts outside NIH to serve on Study Section Boundary (SSB) Teams, charged with designing the IRG's study sections. SSB Team recommendations, which include consideration of comments from the outside scientific community, are presented to CSR's Advisory Committee to determine on a case-by-case basis the steps necessary to implement the modified IRGs.

The reorganization process has been by design methodical and slow. Comment and participation by relevant scientific groups and stakeholders have been strongly encouraged to ensure broad support. NIH's focus on this reorganization ensures that the peer review system will be able to accommodate the review of more complex, diverse, and multidisciplinary research proposals.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Improve the organization of the peer review system to keep pace with advances in science:						
Conduct various assessments of the functions and organization of NIH study sections.	◆					
Complete Phase 1 of the Panel on Scientific Boundaries for Review (PSBR) and develop an implementation plan for Phase 2.		◆				
Create four Steering Committees and their respective Study Section Boundary (SSB) Teams.			◆			
Increase the number of Steering Committees and SSB Teams to 10.				◆		
Present the recommendations of at least three IRB SSB Teams to the CSR Advisory Committee each year, and subsequently develop implementation plans for reorganizing each of those IRGs.					◇	◇
Complete formation of all Steering Committees and SSB Teams.[1]					◇	
Enhance study section operations by forming more external IRG Advisory Groups:						
Double the number of external IRG Advisory Groups from three to six.		◆				
Increase the number of external IRG Advisory Groups to 14.			◆			
Complete the formation of all external IRG Advisory Groups.				◆		

◆	Target Met	◇	Target Active	→	Target Not Met and Extended	✕	Target Not Met

Summary of Performance Results

- **Peer review system improvements are on schedule to keep pace with advances in science.** NIH created seven Steering Committees and their respective Study Section Boundary Teams, exceeding its target of four, in FY 2001. In prior years, NIH completed the final Phase 1 PSBR report and initiation of the Phase 2 implementation plan. Integration of the neurosciences and AIDS study sections, and the behavioral and social science study sections were also completed. NIH exceeded the FY 2002 target by increasing the number of Steering Committees and their respective SSB Teams to 12.

- **Enhancements of study section operations were completed ahead of schedule.** The FY 2000 target to double the number of external IRG Advisory Groups from 3 to 6 was significantly exceeded with 10 Advisory Groups formed during the year. Seeking to build on this success, NIH revised the FY 2001 target to 14. By forming 9 additional groups,

[1] The Phase 1 report of the PSBR found that there is a current need for 17 IRG Steering Committees and SSB Teams.

NIH's actual performance exceeded the revised FY 2001 target considerably. The remaining 5 Advisory Groups were formed in FY 2002. Reports from all 19 Advisory Groups have been received and a summary was presented to the CSR Advisory Committee in January 2002.

Goal d) Develop innovative business practices to facilitate government/public interactions.

NIH is committed to increasing the efficiency of its policies and procedures to improve customer service to grantees without diminishing its ability to provide appropriate oversight of its research portfolio. To help simplify grants administration processes, the NIH Office of Extramural Research (OER) has identified two priority areas: 1) shortening the time between submitting a grant application and receiving research funds, and 2) simplifying the administrative processes required of grantees.

Reducing the grant award cycle will allow the highest quality research to begin sooner and permit already productive research programs to continue uninterrupted. Even in instances where funding cannot begin earlier (e.g., funding a competing continuation application must await the end of the previous noncompeting segment), earlier *notification* of pending awards will provide more stability to the research enterprise. A major success in expediting the awards process has been the recent establishment of procedures to expedite Institute Advisory Councils' concurrence with study section recommendations–a procedure called "expedited en bloc concurrence." In essence, Council concurrence for the most meritorious applications (generally defined as those with technical merit ratings in the top 15% across NIH) can now be obtained prior to the next scheduled Council meeting, permitting more timely awards.

To simplify the administrative processes required of grantees, NIH has responded to suggestions from the extramural research community to identify ways to reduce the number of steps and information required for a noncompeting continuation of a multi-year grant award. For example, NIH will begin a small pilot test to provide more flexibility to grantee institutions using the streamlined noncompeting award process (SNAP), allowing the required information to be submitted directly by each grant's principal investigator instead of the institution's business office. To further simplify administrative processes, NIH has also begun to work with the extramural research community to identify ways to streamline the competitive application process.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Shorten the time between submitting a grant application and receiving research funds:						
Identify approaches to expedite the processing and award of grant applications.	◆					
Expedite the processing of the most meritorious grant applications by extending to all NIH Institutes the use of expedited en bloc Council review procedures.		◆				
Further facilitate expediting the processing of the most meritorious grant applications by reducing the receipt-to-award cycle from 9-10 months to 6-7 months.			◆			
Simplify the administrative processes required of grantees:						
Pilot test ways to further simplify NIH's Streamlined Noncompeting Award Process (SNAP).				→	◇	
Evaluate the results of the simplified SNAP pilots and make recommendations.					◇	
Identify opportunities for reengineering and streamlining data requirements for NIH's competing application (PHS 398).					◇	
Pilot test opportunities for reengineering and streamlining application requirements for the PHS 398.						◇

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	× Target Not Met

Summary of Performance Results

- **Significant progress has been made in shortening the application-to-award cycle.** NIH successfully shortened the time between grant submission and receipt of funds or notification by establishing the process of expedited en bloc Council concurrence. By the end of FY 2001, 13 Institutes were employing the process and expedited 50-75% of Type 1 (new) awards and provided early notification for Type 2 (competing continuation) awards, thus making or providing notification of awards to the most meritorious grant applications in approximately 6-8 months from application receipt. Additional Institutes were expected to begin using the expedited process within the next year. Importantly, NIH has identified a number of factors beyond NIH control that limit the ability to reduce time to award across NIH. These factors are related to applicants' abilities to accept an award and the annual Federal budget process.

- **Recommendations to streamline administrative processes associated with non-competing applications have been developed and approved.** Simplification of the administrative processes required of grantees has been integrated into the new electronic progress reporting system. Staged deployment of this system is scheduled to begin in early 2003.

Goal e) Improve grantee reporting of inventions developed with Federal funds.

The Bayh-Dole Act was enacted in 1980 to ensure the transfer of technology from Federally funded extramural research facilities to the commercial/public sector. The Act stipulates that all grantees must report on inventions, patents, and licenses resulting from Federally funded research. To support this requirement, NIH has developed a web-based system called "Edison," which is designed to receive, store, sort, and provide reports on inventions, patents, licensing, and invention utilization. Edison is the first secure interactive website developed as part of the NIH electronic Research Administration (eRA) system.

A recent analysis of trends in NIH award and invention reporting found that more than 90% of grantee/contractor institutions that do routine invention reporting use Edison to meet their patent and invention reporting needs. Another study found that the use of Edison has significantly reduced the number of cycles of paper correspondence typically involved in patent and invention reporting from 15 to 3, dramatically reducing reporting time and effort. In addition, Edison makes more information available in a usable format for grants administrators.

To further improve grantee reporting of inventions, the NIH Office of Extramural Research (OER) is focusing on two major activities: 1) fully establishing the web-based Edison invention reporting system, and 2) improving the implementation and design of the Edison system. In addition to demonstrating the use of Edison by all grantee institutions registered to do electronic commerce with NIH, its use will also be expanded to other government agencies. A variety of improvements to the system will also be made, including improving the quality of the historical data in the system, and developing a best practices document for constituents based on information gathered during compliance site visits. The redesigned Edison system will be deployed to 350 grantee/contractor organizations in FY 2003.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Fully establish the web-based Edison invention reporting system:						
Enhance recipient compliance with reporting and accountability requirements.	◆					
Fully establish the Edison system for use by all grantee institutions, and expand its use to other government agencies.		◆				
Improve the implementation and design of the Edison system:						
Identify ways to improve the quality of historical invention reporting data.			◆			
Further educate constituents of their invention reporting obligations.			◆			
Integrate Edison into the Federal Commons (a governmental grants and contracts administration system).				×		
Deploy a redesigned Edison system to 350 grantee/contractor organizations.					◇	

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- **The web-based Edison invention reporting system has been established and is producing results.** The FY 1999 and 2000 targets to fully establish the Edison system were both met and, as a result, at the end of FY 1999, 189 grantee institutions were using Edison, a 40% increase since FY 1998. Also, two additional Federal agencies signed Memoranda of Understanding indicating they would begin using Edison to meet their patent and invention reporting requirements. By the end of FY 2000, the Edison system was capable of being used by all grantee institutions registered to do electronic commerce with NIH. Altogether, 273 institutions and 12 government agencies were registered to use Edison, which represented a 44% increase in the number of institutions and a 20% increase in the number of agencies since FY 1999.

- **The NIH has significantly improved the implementation of the Edison system.** In FY 2001, NIH added two contractors to perform analysis of historical records to improve the quality of historical invention reporting data. As part of their duties, these staff interact directly with NIH grantee organizations to increase awareness of historical invention records and reporting obligations, to reconcile historical records, and to resolve any other outstanding data issues. NIH staff provide substantial outreach and education to the extramural community to continue to reinforce the importance of statutory invention reporting to its constituency. In FY 2002 these outreach efforts took the form of 8 proactive compliance site visits, 4 educational seminars, many presentations at national and regional meetings of professional societies, 2 NIH regional seminars, and web postings. Next, NIH will focus its attention on the targets intended to address improvements to the design of the Edison system, first providing a redesigned system and subsequently integrating the new system into the e-Grants initiative (formerly called the Federal Commons). However, integration with the e-Grants will have to wait until the e-Grants system is developed, and development of that system is an activity beyond the jurisdiction of NIH. Because e-Grant development plans do not include invention reporting in Phase I activities that have been laid out through 2004, it is unlikely that integration of the systems will occur in the next couple of years.

2.1.3.5 Management and Administrative Support

To maintain its world-class stature in research, NIH must maintain effective internal management systems and provide strong administrative support to the research community. NIH provides all of the Institutes and Centers (ICs) with centralized management systems and support services to enable them to pursue their research goals unimpeded by administrative obstacles and inefficiencies.

Centrally provided support services include 1) advising the NIH Director and staff on all phases of NIH-wide management and administrative activities, 2) providing leadership and direction to all aspects of management, and 3) overseeing administrative functions (e.g., budget and financial management, personnel management, management policy, management assessment, program integrity, contract procurement and logistics management, engineering services, safety, space and facilities management, support services, and security operations).

In addition to these ongoing responsibilities, current challenges in the management and administrative support area include ensuring that NIH's decisions regarding information technology lead to sound investments that support the overall NIH mission, and improving the efficiency and effectiveness of all NIH procurement and contracting activities.

To address these challenges and provide improved management and administrative support, NIH has eight performance goals:

a) Improve the efficiency of the simplified acquisition process by continuing to expand the Purchase Card Program.

b) Expand the use of Performance Based Contracting (PBC).

c) Identify and pilot new approaches to providing human resource services that increase manager satisfaction with personnel system flexibility and ease of use.

d) Recruit and retain highly qualified extramural investigators to biomedical/behavioral research through the use of student loan repayment programs.

e) Implement government-wide initiative on delayering management levels and streamlining organization.

f) Implement the NIH Business Research and Support System.

g) Improve accountability for organizational performance results and support for the President's Management Agenda by linking the employee performance management plans/contracts to NIH's program and management priorities.

h) Identify and develop potential successors for critical leadership positions by (1) developing and implementing a NIH-wide succession planning process that assesses the gaps between

senior leadership needs and talent available; (2) identifying leadership competencies that will be critical to the mission of NIH now and into the future; and (3) providing developmental opportunities that will prepare our potential successors to meet the demands required of senior leadership positions.

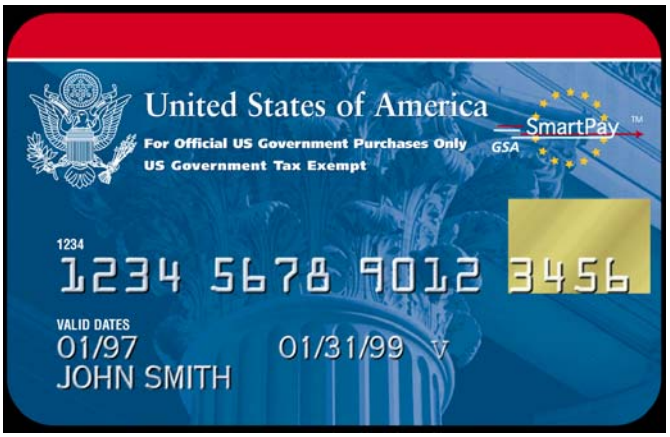
Performance Goal Details - Management and Administrative Support

Goal a) Improve the efficiency of the simplified acquisition process by continuing to expand the Purchase Card Program.

The Federal Supply Service of the General Services Administration (GSA) instituted the Purchase Card Program to help reduce the administrative burdens associated with making small purchases. The Purchase Card Program has been in effect at NIH since June 1995. At NIH, this simplified acquisition method is used for purchases up to $2,500 where authorized and feasible.

The program is operated through NIH's Automated Data Base System (ADB), a computerized database that integrates acquisition, financial, and inventory information. This system gives purchase card holders the ability to review their purchases and credits, including fiscal year-to-date summary data, and provides approving officials with the ability to review the purchases and credits made by card holders and to perform transaction adjustments.

Use of purchase cards by both scientific and administrative staff is substantially improving the acquisition process. Supplies and services are obtained more quickly, more efficiently, and at a substantially lower cost. In FY 2001, NIH shifted its focus from training staff and increasing the number of card holders to increased use of the card by card holders. This focus is reflected in the new FY 2002 target that measures performance by the annual dollar volume of purchase card orders and number of annual purchase card orders/transactions. This focus will continue as NIH explores broadening the array of commodities that can be acquired with the card and use of the card as a mechanism for making contract payments.



Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Increase the dollar volume of purchase card orders made by NIH staff:						
$110 million in orders	◆					
$160 million in orders		◆				
$200 million in orders			✕			
$210 million in orders				◆		
$212 million in orders					◇	
$215 million in orders						◇
Increase the number of NIH scientific and administrative staff trained to use purchase cards[1]:						
3,000 people trained to use cards	→	◆				
3,600 people trained to use cards		→	◆			
Increase the total number of NIH purchase card holders[1]:						
1,600 card holders	→	◆				
2,000 card holders		→	✕			
Increase the number of annual purchase card orders/transactions:						
365,000 orders/transactions				◆		
380,000 orders/transactions					◇	
395,000 orders/transactions						◇

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- **The Purchase Card Program expanded in terms of dollar volume.** The dollar volume of purchase card orders achieved in FY 1999 and FY 2000 exceeded targeted amounts by $20 million and $13 million, respectively. In FY 2001, the dollar volume increased to $196 million, but fell $4 million short of the target. In FY 2002, the dollar volume of purchase card orders was $225 million, which exceeds the targeted amount by $15 million.

  The targets for these measures are set based on data on prior increases in dollar volume of

[1] Note: In FY 2001, NIH shifted its focus from training staff and increasing the number of card holders to the most important measures of performance for this program: dollar volume of purchase card orders and number of annual purchase card orders/transactions.

purchase card use and data on changes in the numbers of card holders. NIH actively encourages greater use of purchase cards by keeping the purchase card program visible through updates and discussion at meetings of the Simplified Acquisition Committee, NIH Executive Officers, and Acquisition Management Committee and through informational workshops conducted by program staff. NIH has worked to eliminate any barriers to using the purchase card. For example, NIH has improved the reconciliation process, which used to be a stumbling block for users. Finally, NIH is exploring additional uses for the purchase card (e.g., as a payment mechanism for contracts, payment of utilities, and as a SmartCard.)

- **The number of staff trained to use purchase cards continued to increase.** Although training the targeted number of staff took longer than planned, 3,391 staff had been trained to use the purchase card by the end of FY 2000, and 3,922 were trained by the end of FY 2001.

- **The number of purchase card holders has increased.** The FY 1999 through FY 2001 targets to increase the total number of NIH purchase card holders were not met, however, the numbers did increase each year. There were 1,485 card holders at the end of FY 1999, 1,729 card holders at the end of FY 2000, and 1,866 card holders at the end of FY 2001. NIH continues to encourage additional employees to become card holders through presentations at meetings, articles in the monthly acquisition newsletter, and informational workshops.

  The targets for this measure were set based on existing data on the average annual number of new card holders. The number of card holders is not being used as a measure past FY 2001, however, as it is not considered a valid indicator for the program. More recently, NIH determined that dollars expended through use of the purchase card and the number of card transactions are more accurate measures of the effectiveness of the Purchase Card Program.

- **NIH increased the number of annual purchase card orders/transactions.** In FY 2002, the number of purchase card orders/transactions was 385,000, exceeding the targeted amount by 20,000.

Goal b) Expand the use of Performance Based Contracting (PBC).

One of the major challenges for government management and administration is improving the efficiency and effectiveness of contracting and procurement activities. Historically, government policies, regulations, and attention have been directed at acquisition of supplies rather than services. A 1997 OMB memorandum requires that all Federal agencies use Performance Based Contracting (PBC) methods, where practicable, and match acquisition and contract administration strategies to specific requirements. In this way, PBC complements the government's overall emphasis on managing for results by linking payments to results rather than to effort or process.

PBC involves using performance requirements that define the work in measurable, mission-related terms, with performance standards of quality, quantity, and timeliness tied to those requirements. PBC also requires a quality assurance plan describing how the contractor's performance will be measured against the performance standards. In cases where a contract is either mission-critical or requires a large dollar amount, incentives are tied to the quality assurance plan measurements.

PBC provides NIH with useful indicators of contactor performance and allows vendors to be innovative in responding to requirements for specific products and services. NIH is therefore strongly committed to increasing the amount of NIH contracting dollars allocated to performance-based contracts. As new contract requirements and contract renewals arise, NIH will review each situation to determine if using PBC is appropriate.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Increase the amount of NIH contracting dollars allocated to performance-based contracts:						
Allocate $19.8 million of the available NIH contracting dollars to PBC-eligible contracts.		◆				
Allocate $21.2 million of the available NIH contracting dollars to PBC-eligible contracts.			◆			
Allocate $207.0 million of the available NIH contracting dollars to PBC-eligible contracts.[1]				◆		
Allocate $226.0 million of the available NIH contracting dollars to PBC-eligible contracts.					◇	◇

◆ **Target Met**	◇ **Target Active**	→ **Target Not Met and Extended**	× **Target Not Met**

[1] The nearly ten-fold increase in the dollar volume of the performance target in FY 2002 is primarily due to a single large performance-based contract awarded in FY 2000

Summary of Performance Results

- **The amount of contracting dollars allocated to PBC exceeded expectations.** In FY 2000, NIH significantly exceeded the target by allocating $198.5 million to PBC– more than 10 times higher than the targeted amount–due primarily to the award of a single, large, performance based contract. In FY 2001, NIH also exceeded the target, allocating $36.5 million of available contracting dollars to PBC contracts. The $21.2 million target was set based on the actual dollars allocated in FY 2000 not including the single exceptionally large performance-based contract that was awarded that year. In FY 2002, NIH contracting offices obligated over $417 million toward performance-based contracts, an amount more than twice the FY 2002 target. The FY 2002 target was developed using estimates based on previous and anticipated PBC obligations. The size of the increase represents a major shift in the way NIH writes contracts and reflects a commitment to the Administration's PBC goals.

Goal c) Identify and pilot new approaches to providing human resource services that increase manager satisfaction with personnel system flexibility and ease of use.

In 1997, NIH initiated a comprehensive evaluation, in conjunction with the National Academy of Public Administration (NAPA), to assess the utility and flexibility of NIH's personnel system. This systematic assessment of the personnel system was warranted because NIH was faced with implementing many human resource services previously conducted by DHHS.

NAPA's 1997 baseline survey of NIH managers found that less than 20% viewed the personnel system as customer-focused, less than 20% found the system flexible and easy-to-use, and only about 30% found the system contributed to organizational goals and objectives.

In response to those findings, NIH moved to increase the satisfaction of managers with the system's flexibility and ease of use. To that end, the NIH Director redelegated many of the personnel administration and management authorities to Institute Directors. In addition, managers and staff received training to ensure that they exercised the new authorities properly.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Delegate human resource authorities to the ICs:						
Complete the delegations of authority and related training.	◆					
Increase managers' satisfaction:						
A 10% increase in manager satisfaction with personnel system flexibility and ease of use as reflected in the 1999 survey outcome against the 1997 baseline.		×				
Complete distribution of the final year management satisfaction survey, interviews, and collect and analyze data for the final report due in 2002.[11]				◆		

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	× Target Not Met

Summary of Performance Results

- **Human resource authorities were delegated on schedule.** NIH met the FY 1999 target to complete the delegation of Title 5 authorities. In addition, the NIH Office of Human Resources Management (OHRM) conducted briefings for senior managers to inform them of the scope and content of the delegated authorities and of their responsibilities to ensure that they are exercised in a proper manner. Managers and administrative staff who would be

[1] This goal will be discontinued after FY 2002.

exercising the new authorities received more intensive briefings and training, including a series of training modules developed by OHRM. In addition, several ICs developed and implemented their own training procedures.

- **Personnel delegations significantly increased managers' satisfaction, although the increases took longer to realize than expected**. Manager satisfaction was examined over a five-year period with baseline, midpoint, and final surveys. The mid-point survey in 1999 (cited by the target) showed a 6% increase in satisfaction. By 2002, results of the Year 5 Evaluation of Personnel Delegations revealed that 47% of surveyed managers agreed or strongly agreed when asked: Have the delegations made the overall personnel system faster and easier to use? This is a far greater increase in satisfaction (38%) than the targeted 10%. The midpoint survey was used to inform implementation of the delegation pilot, and, after the 1999 evaluation, redelegation efforts were tracked and the Director and Office of Human Resources Management encouraged those who were lagging behind in redelegating authorities to implement redelegation. Also, interviews with a small sample of high level Institute managers and human resource directors suggested that delegations had not gone down deeply enough into the organization and that further redelegation was necessary to achieve the full potential of delegation. The eventual success in significantly increasing satisfaction likely is attributable to those actions. Because the evaluation schedule called for a review at the midpoint and end of the 5 year project, NIH does not know exactly when, between 1999 and 2002, the increase in satisfaction reached and then surpassed the 10% mark.

Goal d) Recruit and retain highly qualified extramural investigators to biomedical/behavioral research through the use of student loan repayment programs.

To address the negative impact that staggering educational debt has on career choices for health professionals, NIH implemented several intramural Loan Repayment Programs (LRPs) to provide a financial incentive for highly qualified health professionals to join the NIH intramural research program. The LRPs are designed to attract highly qualified and diverse physician-scientists and bench researchers to the intramural research program by providing for the repayment of their educational loans and to encourage these highly qualified health professionals to pursue a career in basic and/or clinical research at the NIH, other Federal agencies, universities, teaching hospitals, or academic health centers. Through FY 2001, over 400 intramural researchers had received approximately $25 million in benefits from the intramural Loan Repayment Programs.

After the early and apparent success of NIH's intramural LRPs as recruitment and retention incentives, the extramural research community became interested in creating loan repayment programs to address other areas in need of biomedical/behavioral health professionals. In 1997, the Contraception and Infertility Research Loan Repayment Program became NIH's first LRP for extramural researchers. By 2001, Congress had authorized NIH to implement five extramural loan repayment programs (described below). These extramural LRPs are designed to encourage a diverse pool of highly qualified health professionals to engage in specific areas of inquiry, such as clinical research, pediatric research, contraceptive and infertility research, and minority health disparities research.

It is well documented that the pool of physician-scientists has been shrinking for more than two decades. By offering loan repayment to extramural investigators, NIH strives to enhance the recruitment of highly qualified health professionals, especially investigators who are in the early stages of their careers. The LRPs will help to replenish the pool of physician scientists by encouraging more physicians to apply for postdoctoral fellowships. Provided below are descriptions for each of the loan repayment programs.

- **CIR – Contraception and Infertility Research Loan Repayment Program.** The CIR program provides an incentive for health professionals to conduct research related to contraceptive development and/or infertility diagnosis and treatment. The purpose of the program is to recruit highly qualified health professionals into these areas of reproductive research and to stimulate the commitment of those researchers to sustaining a career focus on contraception and/or infertility research.

  While participating in the CIR, individuals must be employed or training at an NICHD Intramural laboratory or eligible NICHD-supported extramural site. Eligible NICHD-supported extramural sites include 1) Cooperative Specialized Contraception or Infertility Research Centers 2) Cooperative Specialized Research Centers in Reproduction Research, 3) Women's Reproductive Health Research Career Development Centers, 4) reproductive

medicine units identified as clinical or data-coordinating sites for the National Cooperative Reproductive Medicine Network, or 5) contraceptive clinical trial units identified as sites for the Contraceptive Clinical Trials Network. These programs currently represent 56 awardee institutions and five consortium sites.

- **CR – Loan Repayment Program Regarding Clinical Researchers**. The CR program provides for the repayment of the educational loan debt of qualified health professionals who agree to conduct clinical research. The program provides for the repayment of up to $35,000 of the principal and interest of the educational loans of extramural researchers for each year of obligated service. The purpose of the CR is to recruit and retain highly qualified health professionals as clinical investigators.

- **ECR – Extramural Clinical Research Loan Repayment Program for Individuals from Disadvantaged Backgrounds**. The objective of the ECR-LRP is the recruitment and retention of highly qualified health professionals from disadvantaged backgrounds to clinical research careers. The emphasis on clinical research and individuals from disadvantaged backgrounds highlights the need for the involvement of a cadre of competent physician scientists in clinical research. Such a cadre of clinical investigators have the potential of impacting the medical processes within their communities by engaging in as well as promoting the development of clinical research programs that reflect an understanding of the variety of issues and problems associated with health status.

  An "individual from a disadvantaged background" is one who comes from a family with an annual income below a level based on low-income thresholds according to family size published by the U.S. Bureau of the Census, adjusted annually for changes in the Consumer Price Index, and adjusted by the DHHS Secretary for use in all health professions programs. The Secretary periodically publishes these income levels in the Federal Register.

- **HDR – Loan Repayment Program for Minority Health Disparities Research.** The objective of the HDR Program is the recruitment and retention of highly qualified health professionals to research careers that focus on minority health or other health disparities issues. The HDR- LRP serves as an avenue for NIH and its National Center on Minority Health and Health Disparities (NCMHD) to engage in and promote the development of research programs that reflect the variety of issues and problems associated with disparities in health status. In addition, the Director, NCMHD, is required to ensure that not fewer than fifty percent (50%) of the contracts are awarded to qualified health professionals from health disparities populations. This requirement highlights the need for the involvement of a cadre of culturally competent scientists in minority health and other health disparities research and "promotes a diverse and strong 21st century workforce" able to address society's diverse needs.

  For purposes of the HDR Program, the Director, NCMHD, after consultation with the Director, Agency for Healthcare Research and Quality (AHRQ), has defined a health disparity population as a population that experiences significant disparity in the overall rate of disease incidence, prevalence, morbidity, mortality, or survival rates as compared to the

health status of the general population. The following populations are classified as specific health disparity populations: Blacks/African Americans, Hispanics/Latinos, Native Americans, Alaska Natives, Asian Americans, Native Hawaiians, Pacific Islanders and the medically underserved such as individuals from the Appalachian region. The following table provides specific information regarding the populations represented in the HDR Program:

POPULATIONS	FY02 AWARDEES
Asian American	8
African American	39
American Indian	4
Caucasian	30
Hawaiian/ Pacific Islander	2
Hispanic	20
Unknown	9
Total	112

- **PR – Pediatric Research Loan Repayment Program.** The PR program provides for the repayment of the educational loan debt of qualified health professionals who agree to conduct pediatric research. The program provides for the repayment of up to $35,000 of the principal and interest of the educational loans of extramural researchers for each year of obligated service. The purpose of the PR is to recruit and retain highly qualified health professionals as pediatric researchers.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Enhance recruitment of highly qualified health professionals, especially investigators who are early in their career:						
50% of eligible sites use the CIR for recruitment.				◆		
50% of applicants apply because of the opportunity to participate in the CIR.				◆		
15% of CR awardees are first-time grant or other award recipients.				◆		
25% of CR awardees are first-time grant or other award recipients.					◇	◇
30% of ECR applicants are in training or recently commenced their research careers.				◆		
50% of ECR applicants are in training or recently commenced their research careers.					◇	◇
30% of HDR applicants are in training or recently commenced their research careers.				◆		
50% of HDR applicants are in training or recently commenced their research careers.					◇	◇
15% of PR awardees are first-time grant or other award recipients.				◆		
25% of PR awardees are first-time grant or other award recipients.					◇	◇
50% of HDR awardees are from health disparities populations.				◆		

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Retain health professionals as biomedical/behavioral researchers:						
50% of past participants conduct contraception and/or infertility research two years after completing the CIR.				◆		
75% of past participants conduct contraception and/or infertility research two years after completing the CIR.					◇	◇

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- NIH met all FY 2002 targets to enhance the recruitment and retention of highly qualified health professionals as researchers:

  The CIR plays an important role in recruitment: The National Institute of Child Health and Human Development (NICHD) queried all 53 sites to determine if they use the CIR-LRP for recruitment. Of the 34 sites that responded, 77 percent (26) of the mentors/supervisors stated that the CIR-LRP plays an important role in recruiting individuals into their programs. *50% of applicants apply because of the opportunity to participate in the CIR-LRP*: Forty-five (45) current and former CIR-LRP participants were queried regarding participation in the CIR-LRP. Of the 28 participants who responded, 79% (22) stated that the availability of the CIR-LRP influenced either their choice of institution/program or their decision to remain at the institution.

  More than 45% of CR awardees are first-time grant or other award recipients: During the FY 2002 application cycle, NIH limited eligibility for the Clinical Research loan repayment program to current recipients of NIH grants or awards. Several of these grant or award types are for first-time recipients, specifically the F32 (Postdoctoral Individual National Research Service Award) and the T32 (Institutional National Research Service Award). In addition, eligibility was limited to first time Principal Investigators of R01 (Research Project) grants. In FY 2002 there were a total of 393 awards under the Clinical Research loan repayment program. Of these, 185, or 47 percent, are first-time grant or award recipients. This far exceeded the 15% goal.

  40% of ECR applicants are in training or recently commenced their research careers: The NCMHD exceeded its goal set for fiscal year 2002 regarding the type of individuals that applied to the ECR Program. Seventy-five (75) individuals applied for the ECR Program. Of the 75 applicants, 40% were individuals who were in training or had recently commenced their research careers. This substantially exceeded the 30% goal. For the purpose of the ECR Program, individuals in training or recently commencing their research careers are individuals that are in post-doctoral fellowships, internships, residency programs, or individuals that have recently (1998 to 2002) graduated from a doctoral program.

Over 40% of HDR applicants are in training or recently commenced their research careers: The NCMHD exceeded its goal set for fiscal year 2002 regarding the type of individuals that applied to the HDR Program. Two hundred and five (205) individuals applied for the HDR Program. Of the 205 applicants, 42% were individuals who were in training or had recently commenced their research careers. This substantially exceeded the 30% goal. For the purpose of the HDR Program, individuals in training or recently commencing their research careers are health professionals that are in post-doctoral fellowships, internships, residency programs, or individuals that have recently graduated from a doctoral program (1998 to 2002).

65% of HDR awardees are from health disparities populations: The NCMHD exceeded its goal set for fiscal year 2002 regarding the percentage of awardees from health disparities populations. The NCMHD made 112 awards to individuals that agreed to conduct minority health or other health disparities research. Of the 112 individuals selected to participate in the program sixty-five percent (65%) were members of a health disparities population. This substantially exceeded the 50% goal.

Over 50% of PR awardees are first-time grant or other award recipients: During the fiscal year 2002 application cycle, NIH limited eligibility for the Pediatric Research loan repayment program to current recipients of NIH grants or awards. Several of these grant or award types are for first-time recipients, specifically the F32 (Postdoctoral Individual National Research Service Award) and the T32 (Institutional National Research Service Award). In addition, eligibility was limited to first time Principal Investigators of R01 (Research Project) grants. In FY 2002 there were a total of 168 awards under the Pediatric Research loan repayment program. Of these, 86, or 51 percent, are first-time grant or award recipients. This far exceeded the 15% goal.

Almost all past participants conduct contraception and/or infertility research two years after completing the CIR: The NICHD reports that seven individuals will have completed their participation in the CIR-LRP at least two years prior to January 31, 2003, and six of the seven (86%) are conducting contraception and/or infertility research. The seventh is likely to, but needs to obtain funding having just become an Ob/Gyn faculty member. This far exceeds the goal of 50% conducting contraception and/or infertility research.

Goal e) Implement government-wide initiative on delayering management levels and streamlining organization.

The aim of NIH research is to acquire new knowledge to help prevent, detect, diagnose, and treat disease and disability. At the same time, NIH is responsible for improving its management to further the success of its mission and research goals. To that end, NIH is working to implement five government-wide Presidential Management Initiatives aimed at:

- Strategic Management of Human Capital
- Expanding Electronic Government
- Competitive Sourcing
- Improve Financial Performance
- Budget and Performance Integration

To determine the best course of action for implementing these initiatives, a NIH Restructuring Committee was formed to make recommendations to the NIH Director. The committee's deliberations focused on the DHHS Secretary's vision of "One Department," views of DHHS senior staff, guidance from the Office of Management and Budget (OMB), and the recommendations of the 1997 Arthur Andersen study of NIH administrative activities. The committee's recommendations, outlined in the NIH Restructuring Report, include centralizing the NIH Servicing Personnel Office, using the new NIH Business System as the model for implementing the Unified Financial Management System (see Goal f, which follows), and supporting the development of Departmental Information Technology and Enterprise Human Resource Project systems.

In addition, NIH developed this performance goal focused on "delayering," or reducing the number of layers in management in an effort to streamline its organization. Reducing management layers will lessen the distance between citizens and decision-makers, thereby allowing NIH to be more responsive to public health needs.

A preliminary review revealed that although NIH Institutes and Centers (ICs) require different organizational designs to meet their individual scientific objectives, four management layers at the IC level is likely to be sufficient in most cases. However, certain complex organizational issues may preclude some ICs from effective performance under this management model. These issues include the organization of some intramural research laboratories that provide services through widely disbursed and mobile staff and the size and scope of organizations such as the National Cancer Institute (NCI).

The proactive NIH compliance effort will use the following process to achieve the delayering targets and goal:

- Assess current organizational reporting structures and identify the mission and support areas impacted.
- Establish review groups within the Institutes and Centers.
- Review functions for impact on science or delivery of services, including review of personnel issues and supervisory ratios.

- Formulate and announce delayering plans.
- Complete organizational changes and reassignments.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004[1]
Identify NIH organizational units for possible delayering:						
Complete assessment of NIH organizational level structure and rationale for current patterns.				◆		
Identify organizational units for delayering.				◆		
Delayer NIH organizational units:						
Develop implementation plans to accomplish delayering for each organizational unit.				◆		
Develop specific numeric targets for the implementation plans.				◆		
Complete delayering for each organizational unit identified.					◇	

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- **NIH identified organizational units for possible delayering and developed an implementation plan with numeric targets.** All FY 2002 targets were met. The NIH Office of Management Assessment completed a review of the NIH organizational structure and worked with NIH Institutes and Centers to develop delayering plans, complete with timelines for implementation. The delayering plan was submitted to the Department in August 2002. Six organizations were identified for delayering. Of these, three of the organizations have completed organizational delayering and the remaining are on schedule to complete delayering by September 2003.

[1] When delayering is completed in FY 2003, this goal will be met.

Goal f) Implement the NIH Business Research and Support System.

After an extensive review of its administrative processes and current information technology support, NIH decided to implement an Enterprise Resource Planning system known as the NIH Business Research and Support System (NBRSS). NBRSS is the combination of the NIH Business System (NBS) and the Enterprise Human Resources and Payroll System (EHRP). The NBS will replace selected administrative operations of the aging legacy Administrative Data Base (ADB) and the EHRP will replace the human resources system currently used by the Department of Health and Human Services and its Operating Divisions.

The NBS will encompass seven functional areas that are currently included in the ADB:

- Financial management
- Property management
- R & D Contracts
- Acquisition
- Service and supply funds operations
- Supply management
- Travel management

The EHRP stems from a DHHS initiative to replace its existing human resources system with a new, state-of-the-art human resources product. EHRP implementation at NIH, which is coordinated with the NBS through the NBRSS Steering Committee, will be deployed in 2002.

The implementation of these Enterprise Resource Planning systems will create an integrated transaction processing system that promotes data sharing and provides information in "real time", ultimately providing more efficient and cost effective administrative support to achieve NIH's scientific mission. Beyond sheer automation, this project seeks to combine the latest technology with proven best business practices and to provide a new level of support to research.

Implementation of NBRSS is one of several administrative improvements that demonstrate NIH's commitment to the principles behind the President's Management Agenda, including Improved Financial Improvement and Expanding Electronic Government. Specifically, 1) it will allow for greater integration of business processes with the financial system and 2) financial systems captured by NBS will comply with all applicable requirements and standards.

The NBRSS is also an important component of the One-HHS initiative. NBRSS serves as a central building block for the HHS Unified Financial Management System (UFMS) and will feed into the Department's single financial management system. NIH has provided resources to the DHHS UFMS and has ensured that the NBS will be compatible with the UFMS. NIH staff actively participate in DHHS UFMS and EHRP teams in order to meet common goals and address cross-Departmental challenges.

Performance Targets	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Implement EHRP				◆		
Deploy the GL/Budgeting module					◇	
Deploy the Property module					◇	
Deploy the Travel module					◇	
Deploy the Contracts/Acquisition/AP/Supply module						◇
Deploy the Service and Supply Fund Activities module						◇

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- **NIH implemented Enterprise Human Resources and Payroll (EHRP) system.** In FY 2002, NIH implemented the Department's EHRP core software, and is on schedule to implement the general ledger and travel modules as targeted.

Goal g) Improve accountability for organizational performance results and support for the President's Management Agenda by linking the employee performance management plans/contracts to NIH's program and management priorities.

> *"We must have a Government that thinks differently, so we need to recruit talented and imaginative people to public service. We can do this by reforming the civil service with a few simple measures. We'll establish a meaningful system to measure performance. Create awards for employees who surpass expectations. Tie pay increases to results. With a system of rewards and accountability, we can promote a culture of achievement throughout the Federal Government."*
>
> *George W.Bush*

In February 2001, GAO added human capital management to the government-wide "high-risk list" of Federal activities. Inspector Generals at nine major Federal agencies have listed workforce problems among the top 10 most serious management challenges that their agencies face.

The first major government-wide initiative under the President's Management Agenda (PMA) is the Strategic Management of Human Capital. The underlying goals of this initiative are:

- Workforce analysis must focus on planning for retirements and resulting skill imbalances
- Reduce layers between civil servants and citizens we serve
- Link budget to individual performance
- Enable government to attract, recruit, retain, develop, and reward good talent and high performers.

The Department of Health and Human Services (DHHS) outlined a program in support of the PMA by delineating "One HHS Management and Program Objectives". One of DHHS's management objectives is to "Improve the strategic management of human capital. The means to accomplish this objective include:

- Conduct ongoing workforce planning to assess the skills we need to accomplish the Department's mission now and in the future
- Attract, hire, and retain exceptional individuals in critical occupations throughout HHS
- Hold employees accountable for achieving measurable results through performance contracts linked to the Department's program and management priorities.
- Encourage managers to demonstrate appreciation by recognizing performance that exceeds expectations.
- Provide better access to learning opportunities for all HHS employees so they can enhance their critical competencies.
- Design effective succession planning and career development programs to build the next generation of HHS leaders.

The NIH is fully supportive of the PMA and DHHS's "One HHS" Management objectives as reflected in the following strategic human capital goal(s).

As required by law, every Federal employee must have a performance plan or contract that clearly outlines responsibilities and duties on which he/she will be evaluated on an annual basis. These responsibilities and duties should be directly linked to the position, that in turn, supports work necessary to the immediate organization. The results of an employee's performance evaluation can influence the granting of awards for excellence, with-in-grade increases, performance improvement actions, etc.

The current performance management system has been criticized by many as ineffectual for a variety of reasons, some of which are the lack of measurable results and the absence of clear links to organizational mission. To remedy this, the goals outlined in the PMA mandate that human capital strategies will be linked to organizational mission, vision, core values, goals, and objectives.

The initial step taken by DHHS to address this issue was to introduce a new format to the Senior Executive Service (SES) performance contracts that enabled employees to identify outputs and outcomes in program areas and identify how each would support the PMA. The intent is to use clear and carefully aligned performance standards and elements for individual employees and leadership. The expectation is that organizations will meet or exceed established productivity and performance goals that could be the basis for performance awards.

HHS undertook the establishment of the new SES performance contracts that eventually would "cascade" through the organization, and charged HHS Operating Divisions and Agencies (OPDIVS) with implementing this system. Initially it was applied only to SES members and OPDIV Heads. NIH has expanded this to include all NIH supervisors and managers in two-grade interval professional positions (those with two-grade promotion patterns, e.g., GS-9 to11). We are now in the process of "cascading" the new performance contract format/methodology throughout the organization.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Incorporate outputs and outcome methodology in managers and supervisors' performance plans					◇	
Additional targets to be determined						

◆ **Target Met**	◇ **Target Active**	→ **Target Not Met and Extended**	✕ **Target Not Met**

Goal h) Identify and develop potential successors for critical leadership positions by (1) developing and implementing a NIH-wide succession planning process that assesses the gaps between senior leadership needs and talent available; (2) identifying leadership competencies that will be critical to the mission of NIH now and into the future; and (3) providing developmental opportunities that will prepare our potential successors to meet the demands required of senior leadership positions.

> *Much of the downsizing was set in motion without sufficient planning for its effects on agencies' performance capacity. Across government, federal employers reduced or froze their hiring efforts for extended periods of time. This helped reduce their number of employees, but it also reduced the influx of people with new knowledge, new energy, and new ideas - the reservoir of future agency leaders and managers.*
>
> *(GAO Report 01-263, 2000)*

The Comptroller of the General Accounting Office (GAO) has stated that there is a human capital crisis in the Federal Government due to the potential wave of retirements from the baby boom generation. The entire Federal government faces an impending wave of retirements of long-servicing, highly competent Federal employees. From this arises a large-scale strategic human resource planning issue. Numerous GAO documents have been published about the "Human Capital Crisis" indicating the need to conduct workforce planning and succession planning as a means to ensure the right skills and competencies exist as well as ensure knowledge management. The Office of Personnel Management recently identified *Human Capital Standards for Success*, which include an assessment of how well departments are ensuring continuity of leadership through succession planning and executive development.

At NIH, succession planning will be crucial if we are to maintain adequate institutional knowledge and effectively carry out our mission during periods of high workforce turnover. For example, we know that the average Senior Executive Service (SES) employee at NIH today is 60 years old with 25 years of service. Of these, 73.3% will be eligible to retire by the end of 2005. While the exodus of talent will not happen overnight, NIH must plan now to maintain required levels of experience, competencies, and knowledge at all levels.

The consolidation/streamlining and competitive sourcing activities, coupled with the potential number of retirements at NIH, makes succession planning extremely critical to ensure the recruitment, retention, and training of employees for a seamless succession of leadership. Voluntary Early Retirement Authority was recently given to NIH for the human resources function and subsequently to those functional areas potentially affected by the competitive sourcing studies. In total, approximately 3,300 employees at all levels have been identified as potentially being affected. Together with normal attrition and retirements, the exodus of potential skills, competencies, and knowledge would be devastating without a plan for remedy.

A major management challenge will be to ensure that we have experienced employees in key positions. Adequate funding and careful planning will be critical to our success in meeting this challenge, allowing NIH to balance the need to meet our present workload demands with the need to build and train our workforce of the future.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Data collection and analysis						
Conduct study and report on average age, years of service, and retirement eligibility. Assess future potential impact.					◇	
Conduct study and report on current state. Assess strengths, weaknesses and needs for changes in current practices.					◇	
Steering/oversight comittee						
Establish Steering Committee					◇	
Provide guidance and direction for NIH-wide succession planning efforts, including succession planning pilot (see below)						◇
Succession planning framework						
Identify industry best practices. Develop a succession planning process to meet the needs of NIH.					◇	
Develop automated tools to support succession-planning processes.						◇
Leadership competencies						
Conduct study to identify competencies needed of NIH leaders that will drive future development efforts					◇	
Incorporate competencies into Individual Development Plan (IDP) goals for target positions.						◇
Succession planning pilot for critical positions in administrative management functions						
Identify potential successors for critical administrative positions and prepare IDPs to guide their development efforts,[1] as the first steps in preparing to conduct the pilot						◇

◆ **Target Met**	◇ **Target Active**	→ **Target Not Met and Extended**	✕ **Target Not Met**

[1] Once potential successors have been identified, an assessment of individual strengths will need to be made based on agreed upon leadership competencies. The NIH will work closely with DHHS and the new Corporate University to provide training and development opportunities to address areas for improvement (e.g. leadership courses, SES Candidate Development Program, etc.).

2.2 Research Training and Career Development Program

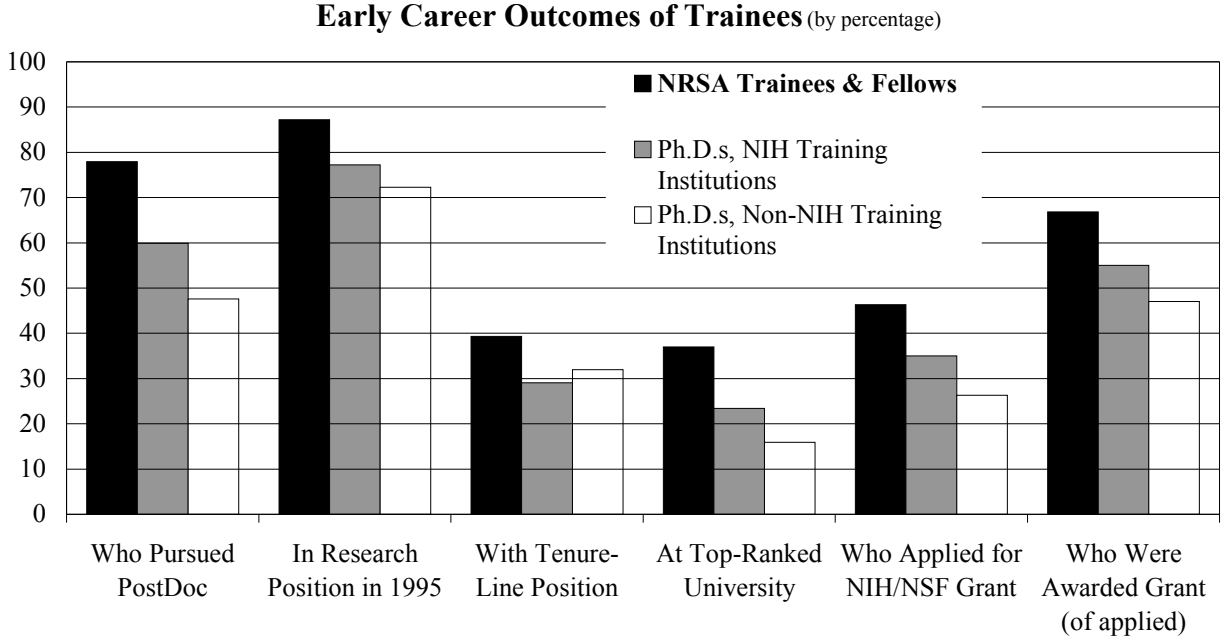
2.2.1 Program Description and Context

The Research Training and Career Development Program addresses NIH's objective to "promote the development of a suitable talent base of well qualified, highly trained, and diverse investigators capable of generating the scientific discoveries of the future." To achieve this outcome, NIH provides training support through the National Research Service Award (NRSA) and various other types of career development programs. These programs are designed to increase the nation's ability to attract and retain the best and brightest minds in biomedical research and to develop a group of well–trained, highly skilled scientists who are ready to meet society's needs for health-related researchers. In addition, NIH's training and career development programs are aimed at enhancing the quality and diversity of the biomedical research labor force.

Predoctoral training. NIH research training and career development support is tailored to the needs of individuals at different career levels. At the predoctoral level, students who are beginning graduate training need to learn the conceptual and theoretical aspects of the science they hope to practice. For individuals at this level, NIH supports broad, multidisciplinary training grants. This kind of support allows universities to assemble a sufficient number of students to justify the development of an educational curriculum in biomedical, behavioral, or clinical research. Students learn the fundamentals in a structured but competitive atmosphere that accelerates knowledge acquisition. These didactic experiences are usually supplemented with laboratory rotations that help students identify the frontiers of modern science and the methods used to address current research problems. Laboratory rotations also teach students that there are often a number of experimental approaches to a specific problem. Once students select a dissertation project, the content of training needs to change. In this phase of their studies, students operate primarily as apprentices, working on some aspect of a funded research project. Most biomedical graduate students receive support during this phase of their training by working as a research assistant on their mentor's research grant.

This approach to graduate training has been widely praised. The widely cited report, *Reshaping Graduate Education*, published in 1995 by the National Academy of Sciences (NAS), recommended that all Federal agencies emulate this approach. Also, a recent NIH evaluation study[1] found that individuals who received at least nine months of NRSA support during their predoctoral research training in the biomedical sciences are more likely to be employed by top-ranked academic institutions and are more likely to have been awarded a research grant by NIH or the National Science Foundation (NSF) than their colleagues who did not receive NRSA training. NRSA recipients have also published more papers and those papers are more highly cited when compared to their colleagues. These NIH programs encourage academic institutions to provide high quality research training, and recipients of this support to make substantial contributions to the biomedical sciences.

[1] Pion, Georgine M., The Early Career Progress of NRSA Predoctoral Trainees and Fellows, NIH Publication 00-4900, March 2001; Table 5.1.



Postdoctoral training. At the postdoctoral level, NIH supports an extension and expansion of the apprenticeship approach. For individuals who continue their formal education in the biological or behavioral sciences, NIH offers training grants, fellowships, and research assistantships to fund this period of intense research activity. The primary focus at this level is on the acquisition of knowledge and skills necessary to launch an independent research career. For physicians and other clinicians with specialized skills and little training in health-related research, NIH offers career development awards that offer more competitive salaries, which are necessary to attract individuals who have completed training in other areas. These awards often include an initial didactic phase to provide instruction in the concepts the candidate will need as an independent researcher. The individual then proceeds to work as an apprentice on a specific project. In most cases, the candidate is ready to apply for his/her own research support by the end of the three-to five-year grant period.

Initiatives to increase diversity. The NIH Institutes and Centers (ICs) provide research training support through NRSA and/or other institutional and individual training awards. The ICs also use various award mechanisms to recruit individuals from racial and ethnic groups that are underrepresented in science. Every NIH institutional training grant must have a minority recruitment plan in place prior to award. Examples of awards designed to increase the diversity of the pool of research scientists are the Minority Access to Research Careers (MARC) and the Career Opportunities in Research (COR) programs. By funding research training experiences for undergraduate honor students at universities having a substantial minority enrollment, these award mechanisms serve an important role in attracting underrepresented students into careers in health-related research. Research Supplements for Underrepresented Minority Individuals permit high school, college, graduate, and postdoctorate students, as well as faculty members, to work on an existing NIH research grant. The NIH has also expanded eligibility for the Supplements for Underrepresented minorities to include individuals who have completed their doctorates but wish to gain an experience in research before enrolling in a graduate or medical

degree program. Increasing the diversity of the research community is seen as an important factor in reducing the disparity in health outcomes observed in the U.S. population.

NIH is also concerned about the participation rates of women in biomedical and behavioral research. The issues associated with the involvement of women in research, however, differ substantially from that of underrepresented racial and ethnic groups. Women are close to parity with men in biomedical sciences at the graduate and postdoctoral levels. In behavioral science fields, women represent a clear majority of the training pool. However, women remain underrepresented at the faculty level, with relatively few serving in high-level academic and administrative positions. Women are also underrepresented among NIH principal investigators. NIH has initiated a number of programs to address this problem, including career development awards and administrative supplements to research grants to encourage fully trained scientists to re-enter research careers after taking time off to attend to family responsibilities.

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2.2.2 Summary of Performance – Research Training and Career Development Program

Comprehensive summary tables covering all of the goals and targets in NIH's Research Training and Career Development Program follow. These tables provide updated information on the status of all of the program's performance targets for Training Support and Outreach activities. More extensive information on each goal, including a chart summarizing the performance results for each target, can be found at the referenced page number.

TRAINING SUPPORT AND OUTREACH			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference**[1]
a) Respond to the National Academy of Sciences (NAS) quadrennial report on the future needs for health-related researchers.	**FY 2004** 1. Increase NRSA stipends 10% above the FY 2003 level. 2. Receive electronic appointment forms on 90% of the estimated number of graduate and postdoctoral research assistants and associates.	Performance will be reported in February 2005.	SP – 4 D – 206
	FY 2003 1. Increase NRSA stipends 10% above the FY 2002 level. 2. Pilot test electronic appointment forms for graduate students and post doctorates supported by research grants.	Performance will be reported in February 2004.	
	FY 2002 1. Issue a statement to encourage universities to limit graduate training to six years and postdoctoral training to five years. 2. Increase NRSA stipends 10% above the FY 2001 level. 3. Develop regulations that permit identification and data collection on graduate students and post doctorates supported by research grants.	1. A statement was issued as part of the March 26, 2001 Response to the NAS report. 2. Stipends were raised per an announcement in the January 22, 2002 NIH Guide. 3. Regulations will not be developed because legal advice indicates that they are not needed. However, a notice will be placed in the Federal Register.	

[1] —Indicates that the goal is part of the President's Management Agenda
SP—Indicates the DHHS Strategic Plan goal to which each GPRA goal pertains
HP-#—Indicates the Chapter of "Healthy People 2010" to which each goal pertains
AHRQ-#—Indicates that the NIH goal is related to a goal in the AHRQ GPRA plan. See Appendix 1.
D-#—Indicates the page in this report at which details on the goal can be found.

TRAINING SUPPORT AND OUTREACH			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference**[1]
	4. Develop electronic appointment forms for graduate students and post doctorates supported by research grants.	4. Target extended to FY 2004. Due to funding related delays in the eRA project, electronic appointment forms for graduate students and postdoctorates supported by research grants have not yet been developed.	
	FY 2001 Prepare response and implementation plans for the recommendations in the NAS report, *Addressing the Nation's Changing Needs for Biomedical and Behavioral Scientists*. (Note: This refers to the 11th NAS Personnel Needs Study).	A response to the Biomedical segments of the NAS report was issued on March 26, 2001.	
	FY 2000 Respond to the quadrennial assessment of the nation's future need for biomedical and behavioral research scientists issued on August 29, 2000 by the NAS.	A statement in response to the NAS report was published in FY 2001 and is available on the NIH website.	
b) Maintain adequate application and award rates in key training support areas.[2]	**FY 2003 & 2004** 1. Maintain a success rate comparable to historical rates for fellowships (F32s). 2. Maintain a success rate comparable to historical rates for research training grants (T32s). 3. Maintain a success rate comparable to historical rates for career awards for basic scientists (K01s). 4. Maintain a success rate comparable to historical rates for entry-level career awards (K08s).	Performance will be reported in February 2004 and 2005, respectively.	SP – 4 AHRQ – 2 D – 209
	FY 2002 1. An application flow of 40% for fellowships (F32s). 2. An application flow of 60% for research training grants (T32s). 3. An application flow of 40% for career awards for basic scientists (K01s). 4. An application flow of 50% for entry-level career awards (K08s).	1. The application flow for F32s was 39%. 2. The application flow for T32s was 58%. 3. The application flow for K01s was 42%. 4. The application flow for K08s was 52%.	

[2] FY 1999 - 2002 targets and performance reports that refer to "application flow" should be interpreted to refer to "success rate."

TRAINING SUPPORT AND OUTREACH			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference**[1]
	FY 2001 1. An application flow of 40% for fellowships (F32s). 2. An application flow of 60% for research training grants (T32s). 3. An application flow of 40% for career awards for basic scientists (K01s). 4. An application flow of 50% for entry-level career awards (K08s). 5. Monitor the need for new announcements and other outreach activities based on application rates, the age and accuracy of existing announcements, and informal assessments of information needs within the target applicant pool.	1. The application flow for F32s was 45%. 2. The application flow for T32s was 62%. 3. The application flow for K01s was 42%. 4. The application flow for K08s was 50%. 5. NIH monitored the monthly use of program announcements and policy documents available on the NIH training website.	
	FY 2000 1. An application flow of 40% for fellowships (F32s). 2. An application flow of 60% for research training grants (T32s). 3. An application flow of 60% for career awards for basic scientists (K01s). 4. An application flow of 60% for entry-level career awards (K08s). 5. Evaluate the effectiveness of the revised announcements, informational materials, and the new training website.	1. The application flow for F32s was 48%. 2. The application flow for T32s was 67%. 3. The application flow for K01s was 36%. 4. The application flow for K08s was 50%. 5. Updated announcements for research training and career development programs were incorporated into the NIH training website.	
	FY 1999 1. An application flow of 40% for fellowships (F32s). 2. An application flow of 60% for research training grants (T32s). 3. An application flow of 60% for career awards for basic scientists (K01s). 4. An application flow of 60% for entry-level career awards (K08s). 5. Revise and publish announcements related to NIH research training and career development opportunities. 6. Reissue the announcement for Minority and Disability Research Supplements.	1. The application flow for F32s was 44%. 2. The application flow for T32s was 64%. 3. The application flow for K01s was 37%. 4. The application flow for K08s was 52%. 5. Announcements for the F32, K01, K02, K05, K07, K08, K23, K24, and K30 awards were republished. 6. The announcements for these awards were published.	

TRAINING SUPPORT AND OUTREACH			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference**[1]
	7. Republish the booklet, *Research Training and Career Development Programs Supported by the National Institutes of Health*. 8. Re-announce programs as necessary to stimulate the submission of applications.	7. The final document was made available in FY 2000 on the NIH website. 8. NIH concluded that no additional announcements were needed because the standing programs continue to receive a sufficient number of applications.	
c) Increase the pool of clinician researchers trained to conduct patient-oriented research.	**FY 2003 & FY 2004** 1. Issue at least 120 awards in the K23 (Mentored Patient-Oriented Research Career Development) category. 2. Issue at least 50 awards in the K24 category.	Performance will be reported in February 2004 and 2005, respectively.	SP – 4 D – 213
	FY 2002 1. Issue at least 120 awards in the K23 category. 2. Maintain a steady state level of awards in the K24 category.	1. In FY 2002, NIH significantly exceeded the target by issuing 197 new K23s (*Mentored Patient-Oriented Research Career Development Awards)* to support young investigators. 2. In FY 2002, NIH issued 48 K24s (*Midcareer Investigator Award in Patient-Oriented Research Awards*), somewhat fewer than the expected steady state.	
	FY 2001 1. Issue at least 80 awards in the K23 category. 2. Issue at least 80 awards in the K24 category.	1. 184 K23 awards were issued. 2. 58 K24 awards were issued.	
	FY 2000 1. Issue at least 80 awards in the K23 category. 2. Issue at least 80 awards in the K24 category.	1. 189 K23 awards were issued. 2. 75 K24 awards were issued.	
	FY 1999 1. Re-announce the career award components of the Director's Initiative on Clinical Research. 2. Issue at least 80 awards in the K23 category. 3. Issue at least 80 awards in the K24 category.	1. The K23, K24, and K30 programs were re-announced. 2. 85 K23 awards were issued. 3. 81 K24 awards were issued.	

TRAINING SUPPORT AND OUTREACH			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference**[1]
	4. Issue at least 20 awards in the K30 (Clinical Research Curriculum Development Award) category.	4. 35 K30 awards were issued.	
d) Increase the participation of underrepresented racial and ethnic groups, women, and individuals with disabilities in NIH training and research programs.	**FY 2004** Increase the number of individuals from underrepresented minority groups appointed to research training grants at the predoctoral and postdoctoral levels.	Performance will be reported in FY 2006.	SP – 4 AHRQ – 1 D – 215
	FY 2003 Increase the number of individuals from underrepresented minority groups appointed to research training grants at the predoctoral and postdoctoral levels.	Performance will be reported in FY 2005.	
	FY 2002 Increase the number of individuals from underrepresented minority groups appointed to research training grants at the predoctoral and postdoctoral levels. *Also see FY 2001 target #1, which has been extended to FY 2002.*	The most recent data available demonstrates an increase from 906 grants in 1998 to 953 in 1999. Updated results of this effort will be reported when data becomes available, late FY 2004.	
	FY 2001 1. Continue to identify areas within the population of NIH-supported trainees that are not responding to efforts to increase demographic diversity. Develop remedial plans to address these problems as needed. 2. Establish a new paradigm for measuring the race/ethnicity of NRSA recipients to bring NIH into compliance with OMB guidelines. 3. Implement OMB-required race/ethnic data collection and reporting strategy.	1. NIH is sponsoring a National Research Council study to assess NIH minority research training programs. Study results are due in November 2003. 2. The grant application form (PHS 398) and the fellowship application form (PHS 416) were modified to accept data using the two-question race/ethnicity data collection approach. 3. NIH reached full compliance with OMB guidelines by providing a means for applicants to designate more than one race.	
	FY 2000 Plan action as appropriate to identify and address demographic groups for which interest in training is abnormally low or declining.	NIH determined that active enforcement of minority recruitment requirements on training grants and monitoring of participation rates was appropriate and will continue to enforce requirements and monitor participation.	

TRAINING SUPPORT AND OUTREACH			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference**[1]
	FY 1999 1. Issue a Notice of Proposed Rule-making (NPRM) to permit part-time NRSA support and part-time payback options for individuals with pressing family obligations or disabilities. 2. Prepare a report identifying the demographics of the individuals supported by the NRSA mechanisms and career award mechanisms.	1. The NPRM was published in the *Federal Register*. 2. Data were presented in a FY 2000 report. The data indicated that the diversity of the workforce is improving, but very slowly.	
e) Expand capabilities for electronic administration of research training and career development activities.	**FY 2004** 1. Receive at least 90% of all training appointments and termination information electronically. 2. 90% of electronically received appointment information is used to establish trainee appointment records and professional profiles within the IMPAC II system.	Performance will be reported in February 2005.	 SP – 8 D – 218
	FY 2003 50% of electronically received appointment information is used to establish trainee appointment records and professional profiles within the IMPAC II system. *Also see FY 2002 Target, which has been extended to FY 2003.*	Performance will be reported in February 2004.	
	FY 2002 Receive at least 50% of all training appointments and termination information electronically.	NIH expects X-Train to be fully operational by the end of FY 2003. Changing technology limited NIH's progress in the development of X-Train. By June 1 2002, X-Train was deployed to twelve institutions. By the end of FY02, 238 electronic appointment forms were received.	
	FY 2001 1. All electronically received appointment information is used to establish trainee appointment records and personal profiles within the IMPAC II system.	1. NIH did not meet this target until FY 2002. Currently, all electronically received appointment information is used to establish trainee appointment records and professional profiles within the IMPAC II system.	

TRAINING SUPPORT AND OUTREACH			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference**[1]
	2. At least 50% of all training appointments received electronically.	2. Due to the unexpected delay in implementing the X-Train system, NIH did not meet these targets. About 238 (3%) of all training appointments were received electronically in 2001.	
	FY 2000 1. Increase by 40% over the 1999 number of trainees, fellows, and career award recipients who maintain electronic records for career tracking purposes in the NIH Person database. 2. Develop a plan for ongoing evaluations of NIH research training programs as well as a plan for periodic, comprehensive career outcome studies. 3. Increase by 40% over the 1999 number of trainee appointment forms received electronically.	Changing technology limited NIH's progress. The delay in implementing X-Train, which will operate under the NIH Commons, impacted the ability to complete all three FY 2000 targets. These targets are expected to be met in FY 2003 and will be reported on in February 2004.	
f) Improve the capabilities for career outcome tracking for NIH training and career development programs. NOTE: This goal overlaps with goal e (electronic administration of training activities) and will be phased out after FY 2003 in deference to that broader goal.	**FY 2003** Link information in X-Train to the professional profile on 90% of all trainees. *Also see FY 2002 target #1 and FY 2001 targets #1 and #2, which have been extended to FY 2003.*	Performance will be reported in February 2004.	D – 221
	FY 2002 1. Develop a plan for ongoing evaluations of NIH research training programs employing data in the professional profile. 2. Develop and deploy X-Train, version 2.0 as a means of collecting appointment and termination information on NRSA recipients. 3. Develop capacity to use X-Train information to establish a professional profile for trainees. The professional profile will serve as a source of long-term career-tracking information.	1. Target has been extended to FY 2003. 2. Due to funding delays related to the eRA project, target has been extended to FY 2003. 3. Target was met. In FY 2002, NIH was able to establish a professional profile for all incoming trainee appointments presently received through X-Train.	
	FY 2001 1. Complete a report on the career outcomes of recipients of NIH extramural postdoctoral research training support. 2. Conduct an early evaluation of the K23 program based on focus groups composed of recipients.	1. NIH began compiling the data sets in FY 1999 and the data are being analyzed. NIH hopes the report to be published by the end of FY 2003. 2. A focus group analysis of the K23 program was completed.	

TRAINING SUPPORT AND OUTREACH			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference**[1]
	FY 2000 1. Complete a report on the career outcomes of recipients of NIH extramural predoctoral research training support. 2. Initiate preliminary work on the long-term tracking database.	1. NIH completed the report. 2. NIH could not establish the Professional Profile database because of delays in implementing the X-Train system. NIH expects this system to be fully operational by the end of FY 2003.	
	FY 1999 1. Complete an evaluation study of NIH pre- and postdoctoral training programs based on existing data. 2. Add training activities functions to the NIH Commons.	1. A report on NIH predoctoral training programs was completed in FY 2000. A preliminary report on the postdoctoral programs is expected to be complete by the end of FY 2003. 2. NIH could not add training activities functions because of delays in implementing the X-Train system. NIH expects this system to be fully operational by the end of FY 2003.	

GPRA Research Training and Career Development Program						
Budget (dollars in thousands)	**FY 1999 Actual**	**FY 2000 Actual**	**FY 2001 Actual**	**FY 2002 Actual**	**FY 2003 Amended President's Budget**	**FY 2004 Request**
	$811,120	$912,241	$1,023,475	$1,156,532	$1,259,660	$1,304,214

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2.2.3 Program Performance Analysis

2.2.3.1 Training Support and Outreach

The broad purpose of the NIH Training and Career Development Program is to 1) enhance the nation's training programs at the predoctoral, postdoctoral, and early career developmental levels to ensure a continuing supply of capable individuals in areas of national need; 2) encourage participants to pursue research careers; and 3) foster the recruitment and retention of underrepresented groups into careers as researchers. Maintaining a highly trained population of scientists who can address the nation's future health-related research needs is an important task. To achieve this end, NIH offers a flexible and varied series of high-quality training opportunities, tailored to the career needs of recipients who are at different stages of education and career development. Some of these training opportunities are described below by activity code and name of award.

- **T32 - Institutional National Research Service (NRSA) Research Training Grant.** Institutional NRSA training grants provide support to academic institutions that can demonstrate a capacity to assemble a team of high-quality faculty and attract a suitable number of high-quality students or postdoctorates interested in engaging in a period of intensive, supervised research training. Training grants are an ideal way to support graduate students prior to their selection of a dissertation subject, allowing the students to consider scientific issues broadly and rotate through different laboratories to gain exposure to a variety of approaches to addressing research problems. Training grants also help students and postdoctorates learn the theories and practical aspects of research related to a particular disease or organ system. Special NRSA training grants support students in programs leading to a dual research degree, such as the M.D./Ph.D. degree.

- **T34 - Minority Access to Research Careers (MARC) and Career Opportunities in Research (COR).** MARC and COR training grants support undergraduate honor students at academic institutions having a substantial minority enrollment.

- **F31 - Individual NRSA Predoctoral Fellowship.** Predoctoral fellowships support supervised training at the graduate level. Special predoctoral fellowships provide support for disabled and minority graduate students.

- **F32 - Individual NRSA Postdoctoral Fellowship.** Postdoctoral fellowships support doctoral level scientists who need additional research experience to successfully compete for independent research funding. Fellows work as apprentices on a defined research project under the supervision of a sponsor or mentor.

- **K01 - Mentored Research Scientist Development Award.** The K01 award supports mentored career development experiences for fully-trained researchers who may have dropped out of research to attend to family responsibilities or who are switching to a new field of research.

- **K08 - Mentored Clinical Scientist Development Award.** The K08 award provides full-time salary support for individuals who have finished or nearly finished their clinical training and wish to pursue a career in research. Many K08 awardees are physicians who may have had very little prior research experience. The first phase of this award usually consists of a period of largely didactic experience, which is followed by closely supervised and project-focused learning experience. It is expected that most recipients of K08 awards will be ready to apply for independent research support by the end of the five-year award period.

- **K22 - Career Transition Award.** The K22 award is used to support the transition of postdoctoral researchers to an independent research position. Usually, the postdoctoral researcher applies without institutional affiliation. This award offers candidates provisional funding that is activated when a suitable independent research position is negotiated. Some of the K22 awards support a continuing period of postdoctoral experience that is followed by a period of independent research support. Career transition awards operate as a pilot program and NIH ICs use this award mechanism in different ways.

- **K23 - Mentored Patient-Oriented Research Career Development Award.** The K23 award is similar to the K08 award but focuses on research that involves human patients. This award is an important part of the Director's Initiative on Clinical Research that is described later in this section.

- **K24 - Midcareer Investigator Award in Patient-Oriented Research Award.** The K24 award provides up to half-time support for established investigators who want to serve as mentors for developing scientists to increase their capacity to conduct high quality patient-oriented research.

- **K30 - Clinical Research Curriculum Development Award.** The K30 award stimulates training in patient-oriented research by offering support to institutions for the development of curricula designed to provide the theoretical and conceptual understanding necessary for high-quality clinical research.

NIH uses these awards and several others (which are not shown) to help achieve its long-term goal of promoting the development of a suitable talent base of well qualified, highly trained, and diverse investigators capable of producing the scientific discoveries of the future. Planning is required, however, to ensure the continued success of the Research Training and Career Development Program. NIH's strategic planning focuses on the following four areas of responsibility:

- ***Estimating future needs for researchers*.** Estimating the nation's future workforce needs in the area of biomedical and behavioral research is difficult because it is almost impossible to accurately predict emerging public health needs and scientific opportunities. Executing a rapid response to changes in these areas is also difficult because of the long time period required for research training. To help address this challenge, NIH asks the National Academy of Sciences (NAS) to conduct a congressionally mandated study of the national

needs for health-related research personnel every four years, as required in Section 489 of the Public Health Act (P.L. 93-348). NAS released the eleventh edition of this series of reports, *Addressing the Nation's Changing Needs for Biomedical and Behavioral Scientists*, on August 29, 2000. The next study, which should be delivered by the end of calendar 2004, has been initiated. NIH continues to use the quadrennial NAS studies, expert panels, program evaluations, and other means to identify areas that should be addressed to improve its Research Training and Career Development Program.

After each NAS report is released, NIH thoroughly reviews the study and accompanying recommendations to plan the scope and focus of its various training and career development programs. Expert panels are also convened, as needed, to advise on specific issues. For example, in 1998, the NIH Director's Panel on Clinical Research identified a specific need to address current shortages of patient-oriented researchers. Another example is the expert panel that was convened in 1995 to help the NIH develop an equitable and uniform tuition reimbursement policy. Targeted program evaluations, such as the recent NIH evaluation of the NRSA predoctoral fellowship program, provide additional information.

- ***Addressing future needs for researchers***. Given the inherent difficulties in estimating the future needs for researchers, the process for deciding the approximate number of NIH training and career development awards of each type to be given each year is challenging. Maintaining program continuity is an important consideration because research training often takes more than 10 years from the beginning of graduate school until the end of postdoctoral training. To retain the best students and to ensure that their training is of the highest quality, NIH tries to maintain a consistent and therefore predictable level of program support from year to year.

  Because students and postdoctoral researchers frequently select a number of different support options over the course of their training, it is important to make these options as attractive and predictable as possible. NIH monitors trends in application rates for specific awards to ensure that they remain reliable options for training support. This allows individuals to plan their training and entry into biomedical research careers. The NIH also engages in a variety of outreach activities to increase awareness of various training opportunities. Attractiveness of individual award options also requires frequent adjustment of support levels or offering more awards of a particular type.

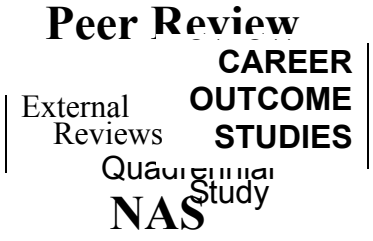
  In addition to offering a variety of awards and monitoring their attractiveness to individuals and academic institutions, NIH uses the quadrennial NAS studies, expert panels, and other means to identify areas that are not being adequately addressed by current training programs. For example, in response to the NIH Director's Panel on Clinical Research, NIH initiated the K23, K24, and K30 awards to address the identified shortages of patient-oriented researchers. Similarly, NIGMS recently launched a training program in bioinformatics and computational biology in response to emerging needs.

- ***Addressing issues associated with diversity and health disparities.*** NIH is strongly committed to training and supporting a research community that reflects the nation's racial and ethnic diversity. Accordingly, NIH offers a number of specific training awards (such as the previously mentioned MARC and COR research training grants and minority

supplements) that are designed to increase the diversity of the pool of research scientists. Different types of training programs are available at the graduate and postdoctoral levels to help ensure that individuals from underrepresented groups are retained throughout the period leading to their independence as research scientists. These programs are especially important as NIH attempts to reduce the disproportionately high incidence and prevalence of disease, burden of illness, and mortality experienced by certain groups, particularly racial and ethnic minorities. The involvement of representatives of all segments of the population within the research labor force should increase the likelihood that relevant health and research issues are addressed.

In addition to underrepresented racial and ethnic groups, NIH is interested in improving the representation of women and individuals with disabilities within the pool of successful research scientists. To that end, NIH has designed specific programs to address the retention of these groups in biomedical research careers. An important aspect of these efforts is monitoring of the demographics of the current workforce and the individuals participating in NIH training programs to assess needs and identify emerging problem areas.

- ***Maintaining high quality research training programs.*** A continuing effort is required to recruit bright, young scientists into biomedical research and maintain the effectiveness of NIH's training and career development programs. The key to maintaining high quality programs is peer review. NIH adjusts review criteria, as needed, and instructs peer reviewers to help identify the applications of institutions that are most likely to provide the best training and attract the best trainees.



To assess the overall impact of the programs, NIH conducts periodic career outcome studies. These evaluations are coupled with external reviews, such as the quadrennial NAS studies. Together, such evaluations help ensure that NIH's Research Training and Career Development Program continues to be of high quality and sufficient magnitude to meet the nation's needs for biomedical and behavioral research. One of the needs identified in the 1993 NAS report, *Meeting the Nation's Needs for Biomedical and Behavioral Scientists*, concerned tracking and assessing the careers of training award recipients. In response to this recommendation, NIH launched a comprehensive evaluation of the predoctoral and postdoctoral NRSA training programs and is developing a web-based trainee appointment and tracking system, called X-Train, to facilitate future career outcome studies.

To address these challenges and enhance its training support and outreach efforts, NIH established six performance goals:

a) Respond to the National Academy of Sciences quadrennial report on future needs for health-related researchers.

b) Maintain adequate application and award rates (success rates) in key training support areas.

c) Increase the pool of clinician researchers trained to conduct patient-oriented research.

d) Increase the participation of underrepresented racial and ethnic groups, women, and individuals with disabilities in NIH training and research programs.

e) Expand capabilities for electronic administration of research training and career development activities.

f) Improve the capabilities for career outcome tracking for NIH training and career development programs.

Goal a) Respond to the National Academy of Sciences quadrennial report on the future needs for health-related researchers.

To achieve its goal of maintaining a highly trained population of scientists that can address the nation's health-related research needs, NIH must complete the difficult task of estimating the nation's future needs for researchers. To address this challenge, NIH asks the National Academy of Sciences (NAS) to conduct a congressionally mandated study of the national needs for health-related research personnel every four years. NAS released the eleventh edition of this series of reports, *Addressing the Nation's Changing Needs for Biomedical and Behavioral Scientists,* on August 29, 2000. It is available on the NIH website at http://grants.nih.gov/ training/nas_report/ index.htm. A contract for the twelfth study has been awarded and will be monitored. That report is due at the end of calendar year 2004.

As part of this quadrennial study, NAS analyzes trends in the current labor force to anticipate future research needs, making specific recommendations about the size, quality, and the nature of NIH's training programs. The NAS studies have proven to be very useful in identifying special and continuing needs for biomedical, behavioral, and clinical scientists. NIH uses the NAS recommendations to make key decisions regarding the scope and focus of its training programs.

In addition to preparing a formal response to the most recent NAS report, NIH intends to improve its research training programs by focusing special attention on four of the NAS recommendations: 1) encouraging universities to expedite graduate and postdoctoral training, 2) increasing stipends paid to NRSA recipients, and 3) designing and implementing a web-based trainee appointment system.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Formally respond to NAS recommendations for improving NIH research training programs:						
Respond to the quadrennial assessment of the nation's future need for biomedical and behavioral research scientists issued on August 29, 2000 by the NAS.		→	◆			
Prepare response and implementation plans for the recommendations in the NAS report, *Addressing the Nation's Changing Needs for Biomedical and Behavioral Scientists*.			◆			
Encourage universities to shorten the time graduate and postdoctoral students spend achieving their degrees:						
Issue a statement to encourage universities to limit graduate training to 6 years and postdoctoral training to 5 years.				◆		

Performance Targets	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Increase stipends paid to National Research Service Award (NRSA) recipients:						
Increase NRSA stipends 10% above the FY 2001 level.				◆		
Increase NRSA stipends 10% above the FY 2002 level.					◇	
Increase NRSA stipends 10% above the FY 2003 level.						◇
Issue regulations for tracking the future careers of NIH trainees:						
Develop regulations that permit identification and data collection on graduate students and postdoctorates supported by research grants.				×		
Design and implement a web-based trainee appointment system:						
Develop electronic appointment forms for graduate students and postdoctorates supported by research grants.				→	→	◇
Pilot test electronic appointment forms for graduate students and postdoctorates supported by research grants.					→	◇
Receive electronic appointment forms on 90% of the estimated number of graduate and postdoctoral research assistants and associates.						◇

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	× Target Not Met

Summary of Performance Results

- **NIH formally responded to the NAS quadrennial report.** NIH officials reviewed the report, developed an initial implementation plan, and published a formal response to the biomedical segments of the report on March 26, 2001. The response stated NIH's intention to implement selected recommendations from the NAS report. To track the implementation of four of the most challenging NAS recommendations, NIH developed specific performance targets for FY 2002 and FY 2003, as shown in the above table.

- **NIH has encouraged universities to shorten the time graduate and postdoctoral students spend achieving their degrees.** In the March 26, 2001 NIH response to the quadrennial NAS personnel needs study, NIH exhorted universities to improve time to degree. NIH plans to monitor the duration of graduate and postdoctoral training, but understands that the impact of actions taken in response to the report and NIH exhortations will not be seen for several years to come.

- **NIH is increasing stipends paid to National Research Service Award (NRSA) recipients.** In FY 2002, NIH raised stipends by 10% (NIH Guide, January 22, 2002). Future increases will be reported in February 2004 and 2005.

- **NIH is preparing to issue a notice regarding tracking the future careers of NIH trainees.** Legal advice indicates that new regulations are not needed to track the future careers of NIH trainees. Accordingly, NIH did not develop and will not issue such regulations. However, NIH does expect to issue a notice in the Federal Register, as soon as the electronic appointment systems that will enable the tracking become available.

- **NIH is designing and implementing a web-based trainee appointment system.** Due to funding related delays in the eRA project, electronic appointment forms for graduate students and postdoctorates supported by research grants have not yet been developed, and pilot testing has also been extended accordingly to FY 2004.

Goal b) Maintain adequate application and award rates (success rates) in key training support areas.

As the preeminent biomedical research program in the world, NIH attracts a high number of applications each year to its prestigious Research Training and Career Development Program. The overall goal of this program is to maintain a population of scientists who are well educated, highly trained, and dedicated to meeting the nation's future health-related research needs. At the same time, NIH must monitor application and success rates constantly to improve the Research Training and Career Development Program and make certain it is addressing the nation's needs.

NIH's efforts to maintain adequate application and success rates (the success rate is the percent of applicants who receive awards) are focused on 1) comparing application and success rates for the most popular awards to historical rates and 2) conducting needed training and career development outreach activities.

Application rates for NIH training and career development programs are rough but important indicators of the continuing attractiveness of these programs to the research community. If an application rate falls below historical rates, NIH determines the reason and responds accordingly. For example, NIH can enhance the attractiveness of a particular award by increasing applicants' probability of success (the success rate), increasing benefits for awardees, or improving outreach.

Success rates affect the attractiveness of an award since applicants who think they are unlikely to receive an award may opt for other sources of support. It is therefore important for NIH to maintain some stability in the overall success rate so that applicants know what to expect. Comparing current application and success rates with historical rates provides NIH staff with the critical information they need to determine whether the eligibility criteria or provisions of an award should be adjusted.

NIH uses a combination of outreach efforts to inform prospective applicants about careers in research and the availability of financial support, which help maintain adequate application rates. Outreach activities of the Research Training and Career Development Program include the following:

- NIH training staff attend national and regional conferences on biomedical research and other scientific meetings to encourage students and post doctorates to engage in training leading to a career in biomedical research.

- The NIH homepage (http://www.nih.gov/) averages more than 10 million hits per month and is one of the most frequently accessed websites in the Federal Government. The homepage for NIH Research Training Opportunities (http://grants.nih.gov/training/index.htm) is the twelfth most frequently hit page and constitutes an important and growing part of NIH's outreach effort.

- NIH announces all grant opportunities online in the *NIH Guide for Grants and Contracts* (http://grants.nih.gov/grants/guide/index.html), which provides information on research,

research training, and career development opportunities in both the intramural and extramural programs. The website organizes the information in the *NIH Guide* into a logical format so potential applicants will have all the information needed to apply for NIH programs. Any interested researcher can receive this electronic document each week by signing up for the extensive list serve. Graduate students and post doctorates are apprised of this resource at all regional and national conferences that focus on research training issues.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Maintain healthy application and success rates for the most heavily utilized award mechanisms:						
An application flow of 40% for fellowships (F32s)[1]	◆	◆	◆	◆		
Maintain a success rate comparable to historical rates for fellowships (F32s)					◇	◇
An application flow of 60% for research training grants (T32s)[1]	◆	◆	◆	◆		
Maintain a success rate comparable to historical rates for research training grants (T32s)					◇	◇
Maintain a success rate of 60% for career awards for basic scientists (K01s)	×	×				
An application flow of 40% for career awards for basic scientists (K01s)[1]			◆	◆		
Maintain a success rate comparable to historical rates for career awards for basic scientists (K01s)					◇	◇
Maintain a success rate of 60% for entry-level career awards (K08s)	×	×				
An application flow of 50% for entry-level career awards (K08s)[1]			◆	◆		
Maintain a success rate comparable to historical rates for entry-level career awards (K08s)					◇	◇
Conduct needed training and career development outreach activities:						
Revise and publish announcements related to NIH research training and career development opportunities.	◆					
Reissue the announcement for Minority and Disability Research Supplements.	◆					
Republish the booklet, *Research Training and Career Development Programs Supported by the National Institutes of Health*.	→	◆				
Re-announce programs as necessary to stimulate the submission of applications.	◆					
Evaluate the effectiveness of the revised announcements, informational materials, and the new training website.		◆				
Monitor the need for new announcements and other outreach activities based on application rates, the age and accuracy of existing announcements, and informal assessments of information needs within the target applicant pool.[2]			◆			

[1] Targets that refer to an "application flow" should be interpreted to refer to a "success rate" and have been superseded by properly stated targets.
[2] Monitoring the need for new announcements and other outreach activities is a routine activity and was therefore discontinued as an explicit target after FY 2001.

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	× Target Not Met

Summary of Performance Results

- **NIH continues to maintain application and success rates for the most popular awards that are comparable to historical rates.** Trends in application and success rates are approximate indicators of the attractiveness and viability of training and career development programs. Consistently high application rates indicate that the programs are properly designed to meet the needs of targeted populations and stable success rates indicate the steady NIH commitment and the continuing interest of highly qualified applicants that are necessary to strong programs. However, both rates are a function of many variables including demographic trends and shifting national needs. Moreover, trends are more significant than absolute rates.

  In FYs 1999 and 2000, NIH received sufficient numbers of applications to maintain a consistent contribution to the support of training in the health-related sciences. The success rates for individual fellowships (F32s), institutional research training grants (T32s), and mentored research scientist and clinical scientist development awards (K01s and K08s) were also sufficiently close to their historical rates.

  In FY 2000, the F32 and T32 success rates (48% and 67%, respectively) exceeded the corresponding target rates of 40% and 60%. The K01 and K08 success rates (36% and 50%, respectively) both fell short of the 60% target rate, and a subsequent analysis indicated that the 60% rate was unrealistically high for these career development awards. Beginning in FY 2001, the K01 and K08 target success rates were revised downward to 40% for K01 awards and 50% for K08 awards.

  In FY 2001, the success rates for F32s (45%), K01s (43%), K08s (50%) and T32s (62%) were all very close to target rates.

  In FY 2002, NIH fully maintained success rates for F32s (39.4%), T32s (58%), K01s (42.4%), and K08s (52.3%). NIH will continue to monitor the application and success rates for all of these programs each year, taking steps to re-announce specific programs and/or change some of their features if NIH observes trends that would warrant such action.

- **NIH conducted needed training and career development outreach activities.** During FY 2001, the need for new announcements and other outreach activities was assessed informally at two regional seminars (one held in Portland, Oregon and one in Honolulu, Hawaii) and at a variety of scientific conferences and conventions. In 2002, regional NIH seminars were held in Michigan and Kentucky. Although there was no demonstrated waning of interest in NIH training and career development programs, plans were implemented to update the T32 and career awards program announcements in FY 2002.

During FY 1999, NIH met three of the four targets in this area. Specifically, NIH revised and reissued announcements for the F32, K01, K02, K05, K07, K08, K23, K24, and K30 awards; reissued announcements for the Minority and Disability Supplement awards in May 1999; and determined that the application rates for the most popular programs were close to historical levels and did not require re-announcements. NIH also completed a draft copy of the on-line booklet, *Research Training and Career Development Programs Supported by the National Institutes of Health*, in FY 1999 and posted the final document on NIH's new training website in FY 2000. This booklet was discontinued in 2002 because of workload and staffing concerns. NIH is comfortable with this change because of nearly universal use of web resources by the applicant pool. The NIH research training and career development website will be limited to programs that are truly NIH-wide and coordinated by the Office of Extramural Research. However, the NIH site will include links to Institute-and-Center specific training websites.

NIH met the FY 2000 target to evaluate the effectiveness of the revised announcements, informational materials, and the new training website. NIH included updated announcements for research training and career development programs on the NIH training website. The program announcements and policy documents available through this venue continue to receive substantial monthly use.

Goal c) Increase the pool of clinician researchers trained to conduct patient-oriented research.

NIH is committed to increasing the number of physicians and other clinicians trained to conduct patient-oriented research, and in 1999, NIH implemented three new career mechanisms to achieve this important goal. These three mechanisms are components of the Director's Initiative on Clinical Research.

Increasing clinicians' expertise in conducting patient-oriented research was strongly recommended by two prestigious groups–an NIH Director's Panel on Clinical Research, and an Institute of Medicine (IOM) Committee on Addressing Career Paths for Clinical Research. Both groups recommended that NIH expand and improve its training programs in patient-oriented research for both entry-level and mid-career clinical investigators so that these researchers could capitalize on recent discoveries based on molecular approaches and translate them to clinical settings.

Accordingly, NIH established three new career development mechanisms designed to issue an adequate number of awards to encourage patient-oriented research. The first mechanism is the *Mentored Patient-Oriented Research Career Development Awards* (K23) to support the career development of investigators who have made a commitment to focus on patient-oriented research. The second mechanism is the *Midcareer Investigator Award in Patient-Oriented Research Awards* (K24) that provides set-aside time for mid-career investigators who want to enhance their own research and mentor younger patient-oriented researchers. The third is the *Clinical Research Curriculum Development Awards* (K30) designed to enhance curriculum development in clinical research. All three award mechanisms appear to be attractive to potential applicants, and NIH expects that they will eventually increase the number of productive scientists working in this important area.

To convey NIH's commitment to training in this area in future years, the Director's Initiative recommended that at least 80 K23 and 80 K24 awards be made each year from FY 1999 through FY 2003 to achieve a steady state of approximately 400 awards of each type. Annual performance targets were accordingly developed for K23 and K24 awards, along with a target to issue at least 20 curriculum development (K30) awards in FY 1999. The K30 target was discontinued in later years. Additionally, the Director's Panel on Clinical Research expressed concerns about the number of clinical researchers available at the mid-career level and data generated in FY 2001, suggest that these concerns may have been well founded.

After the five-year initial period, NIH will examine the continuing need for this program and will set new targets if necessary. After five years' experience, it may be possible to develop routine procedures for managing the application rate through announcements and changes in award provisions, thereby achieving a stable success rate.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Issue an adequate number of awards to encourage patient-oriented research:						
Re-announce the career award components of the Director's Initiative on Clinical Research.[1]	◆					
Issue at least 20 awards in the K30 category.	◆					
Issue at least 80 awards in the K23 category.	◆	◆	◆			
Issue at least 120 awards in the K23 category.				◆	◇	◇
Issue at least 80 awards in the K24 category.	◆	✕	✕			
Maintain a steady state level of awards in the K24 category.				✕		
Issue at least 50 awards in the K24 category.					◇	◇

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- **NIH continues to issue awards to encourage patient-oriented research.** In FY 1999, NIH re-announced three components of the Director's Initiative on Clinical Research (the K23, K24, and K30 awards) and subsequently issued 85 K23 awards, 81 K24 awards, and 35 K30 awards during the fiscal year, meeting all of the targets. In FY 2000, NIH issued 189 K23 awards (more than twice the targeted level) and 75 K24 awards (slightly less than the targeted level). In FY 2001, NIH issued 184 new K23 awards (greatly exceeding the target of 80 awards) and 58 K24 awards (fewer than expected).

  In FY 2002, NIH issued 197 new K23s to support young investigators, again significantly exceeding the target. NIH issued 48 K24s, somewhat fewer than the expected steady state. The K24 results suggest that the pool of mid-career patient-oriented research mentors may be reaching saturation and the annual performance target for K24 awards should be revised downward in future years. The shortage of individuals available for mid-career awards was identified in the report on the Director's Initiative on Clinical Research (http://www.nih.gov/news/crp/97report/execsum.htm). However, NIH still expects the K24 mechanism to continue to facilitate increases in the number of productive scientists working in this important area.

[1] Re-announcing awards to encourage patient-oriented research has become a routine activity and was therefore discontinued as an explicit target after FY 1999.

Goal d) Increase the participation of underrepresented racial and ethnic groups, women, and individuals with disabilities in NIH training and research programs.

NIH believes strongly that training and supporting a research community that reflects the nation's social diversity is a top priority, and it is developing programs to monitor and increase the diversity of NIH-supported trainees.

Accordingly, NIH is designing a number of training programs to provide support to minority graduate and postdoctoral students and to recruit them into research at all career levels. NIH also is developing programs designed to enhance the retention of women in biomedical research careers and provide support for individuals with disabilities. All of these efforts address, in part, the disparities in morbidity and mortality across racial/ethnic and other demographic groups. Continual monitoring of the demographics of the participants in NIH programs is an important aspect of these efforts. This ongoing vigilance permits NIH to assess the value of existing initiatives and identify emerging problem areas.

NIH is particularly interested in implementing OMB's revision of Directive 15 (for details see http://www.whitehouse.gov/OMB/inforeg/race.pdf) related to collecting data on race and ethnicity. Under these new guidelines, all Federal agencies must collect information on ethnicity separately from race, and all agencies must offer respondents the option of indicating more than a single race. OMB offered recommendations for reporting the new data. As NIH moves toward compliance with the new guidelines, it needs to modify data collection forms such as the Statement of Appointment Form (Form 2271), the Grant Application Form (Form 398), and the Individual Fellowship Application (Form 416-1). The electronic equivalents of these data collection instruments, including the Electronic Trainee Activity System (X-Train), must also be modified. Finally, NIH has to modify the data entry screens for IMPAC II and the underlying data structures to accept the new data. The tradeoff here is that the changes in data collection categories will disrupt the continuity essential to longitudinal data analysis; comparison of years before and after the change will not be valid.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Develop programs to monitor and increase the diversity of NIH-supported trainees:						
Issue a Notice of Proposed Rulemaking (NPRM) to permit part-time NRSA support and part-time payback options for individuals with pressing family obligations or disabilities.	◆					
Prepare a report identifying the demographics of the individuals supported by the NRSA mechanisms and career award mechanisms.	→	◆				
Plan action as appropriate to identify and address demographic groups for which interest in training is abnormally low or declining.		◆				
Continue to identify areas within the population of NIH-supported trainees that are not responding to efforts to increase demographic diversity. Develop remedial plans to address these problems as needed.			→	→	→	◇
Establish a new paradigm for measuring the race/ethnicity of NRSA recipients to bring NIH into compliance with OMB guidelines.			◆			
Implement OMB-required race/ethnic data collection and reporting strategy.			◆			
Increase the number of underrepresented minorities appointed to research training grants:						
Increase the number of individuals from underrepresented minority groups appointed to research training grants at the predoctoral and postdoctoral levels.[1]				TBD[2]	◇	◇

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- **NIH increased the diversity of NIH-supported trainees.** NIH met the first FY 1999 target on schedule by issuing a Notice of Proposed Rulemaking (NPRM) for 42 Part 66, which NIH published in the *Federal Register* on June 30, 1999. The NPRM addressed the needs of young women especially, as well as others with pressing family obligations or disabilities, by permitting NIH to award part-time NRSA support and part-time payback options. The final rule was published on May 31, 2001 and is available at http://grants.nih.gov/training/nrsa.htm. For the second FY 1999 target, NIH developed programs to extract race/ethnicity data on individuals supported by NRSA and other career development programs, but the analysis was not completed until FY 2000. The data presented in the FY 2000 report indicated that the diversity of NIH trainees is slowly improving. In addition, NIH met the related FY 2000 target to plan action as appropriate to identify demographic groups for which interest in training is abnormally low or

[1] The FY 2002 target was adapted as an annual target by removing reference to the previous year (FY 2001) as the base year.
[2] To be determined. Data will be available in late FY 2004.

declining. NIH has continued to enforce minority recruitment requirements actively for T32 research training grants and monitor the participation of individuals from underrepresented groups.

In FY 2001, several steps were taken to identify and address problems involving minority recruitment and retention. In-depth discussions initiated by the NIH Training Advisory Committee, as a forum for information and strategy sharing among ICs. Also, the NIH National Center on Minority Health and Health Disparities (NCMHD) engaged the National Research Council in a broad study to assess NIH minority research training programs. The study is addressing three questions: (1) Do the NIH minority research training programs work? (2) Which minority programs and which features of minority programs have been most successful in helping individual students and faculty members move toward productive research careers? (3) What additional factors contribute to minority trainee success? Study results are due in November 2003.

NIH met the other two FY 2001 targets that involved the development and implementation of an improved system for measuring the race/ethnicity of NRSA recipients, as required by OMB. Specifically, NIH successfully established a new paradigm for collecting race/ethnicity data using two questions rather than one, and modified the grant application form (PHS 398), the fellowship application form (PHS 416), and the statement of appointment form (PHS 2271) to reflect the new approach. This data collection approach was also incorporated into X-Train, which serves as the electronic version of the statement of appointment form. The modified forms also permitted applicants to designate more than one race, bringing NIH into full compliance with OMB guidelines. In addition, underlying data structures were modified to accept data using the new approach.

- **NIH will seek to increase the number of underrepresented minorities appointed to research training grants.** The most recent data available demonstrates an increase from 906 grants in 1998 to 953 in 1999. Updated results of this effort will be reported as data becomes available (late FY 2004). Because minority participation is driven by many factors in addition to those NIH can influence, NIH may not meet this target for FY 2002.

Goal e) Expand capabilities for electronic administration of research training and career development activities.

NIH is developing new electronic capabilities to improve and streamline the reporting, processing, and analyzing of training and career development data and to greatly expand its career-tracking capabilities. This electronic administration system will be more effective and efficient than the current system; it will provide access to richer data on training experiences and career outcomes and will facilitate better evaluations of NIH training programs. To enhance the quality of these programs, electronic administration will also be used to match the performance of former trainees and fellows to specific characteristics of their training programs. As a result, NIH should be able to respond better to the constantly changing national needs and priorities for research training.

To expand its capabilities for electronic administration, NIH is focusing on the development of NIH's web-based trainee appointment system. Further development of a web-based interface for electronic submission of trainee appointment and termination information will facilitate reporting by recipient organizations that receive NRSA institutional training grants. This electronic system will be part of the NIH electronic Research Administration system (eRA) and will improve the ease of data entry and processing, in addition to improving the quality of data. The specific features of this system, referred to collectively as the Electronic Trainee Activities System (X-Train), were deployed in a pilot format in December of 2001. A more functional system should be available by the fall of 2003. A link to the future X-Train system is available on the NIH Commons website at https://www-commons.cit.nih.gov/.

When completed, information on appointments entered through X-Train will be electronically linked to the trainee's Professional Profile. The combination of the Professional Profile and the appointment information will begin to form a repository of biographical and transactional information on all individuals affiliated with NIH either as principal investigators, key personnel, trainees, fellows, or career award recipients. By encouraging all affiliated scientists to continually update their web-based biographical information, NIH will be able to improve its analyses of the effect of training support on career outcomes. The database will have the capability to track scientists' academic positions, publications, appointments to advisory groups, and grant awards.

For more than four years, NIH has been working on mechanisms to increase electronic receipt of information about trainee appointments and terminations. In FY 1997, NIH established a precursor system to X-Train and demonstrated the feasibility and value of electronic administration of research training and career development activities. Using that system, NIH received 614 trainee appointment forms electronically. In FY 1998, NIH received more than 1,029 forms electronically, a 68 percent increase over the previous year. In FY 2001, 15 Federal Demonstration Partnership institutions tested the new system, and NIH expects to have this interface fully operational by the end of FY 2003. Expanding the operation of this system during FY 2004 to all institutions with NRSA training grants will increase the volume of electronic information dramatically.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Further develop NIH's web-based trainee appointment system (X-Train):						
Increase by 40% over the 1999 number of trainees, fellows, and career award recipients who maintain electronic records for career tracking purposes in the NIH Person database.		→	→	→	◇	
Develop a plan for ongoing evaluations of NIH research training programs as well as a plan for periodic, comprehensive career outcome studies.		→	→	→	◇	
All electronically received appointment information is used to establish trainee appointment records and professional profiles within the IMPAC II system. (Note: target was extended, then superseded by the target that immediately follows.)			→	◆		
50% of electronically received appointment information is used to establish trainee appointment records and professional profiles within the IMPAC II system.					◆	
90% of electronically received appointment information is used to establish trainee appointment records and professional profiles within the IMPAC II system.						◆
Increase electronic receipt of trainee appointment and termination information:						
Increase by 40% over the 1999 number of trainee appointment forms received electronically.		→	→	→	◇	
At least 50% of all training appointments received electronically. (Note: target was extended, then superseded by the target that immediately follows.)			→	×		
Receive at least 50% of all training appointments and termination information electronically.				→	◇	
Receive at least 90% of all training appointments and termination information electronically.						◇

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	× Target Not Met

Summary of Performance Results

- **Changing technology limited NIH progress in developing its web-based trainee appointment system.** The need to adapt to changing technology, finalize the resource allocation processes, and address the need for compatibility with other Federal systems has delayed the implementation of the X-Train system. However, NIH succeeded in deploying an early version of X-Train to two institutions on October 1, 2001. By June 1, 2002, a total of 12 institutions were using X-Train and, by the end of FY 2002, 238 appointment forms had been obtained electronically. Currently, all electronically received appointment information is used to establish trainee appointment records and professional profiles within the IMPAC II system. By the end of FY 2003, NIH expects X-Train and the related Professional Profile database to be fully operational and capable of handling nearly all appointment and reappointment information for NRSA recipients as the NIH Commons architecture is revamped with J2EE technology. This system will also receive termination

information on NRSA recipients unless the trainee's signature is required to verify a service payback obligation.

The delay in implementing X-Train postpones achievement of all of NIH's targets associated with electronic administration of research training and career development activities. NIH fell short of the targets to increase the number of trainees, fellows, and career award recipients who maintain electronic records. In addition, because NIH's overall approach to future evaluations of the research training programs depends on the web-based appointment data collected via X-Train, NIH could not plan for on-going evaluations and periodic outcome studies. When X-Train is fully operational in FY 2003, will know more about the nature and reliability of the electronic information in X-Train and, as a result, will be better equipped to plan effective evaluations.

- **Unexpected delays were experienced in increasing the electronic receipt of trainee appointment and termination information.** Due to funding related delays in the eRA project, the implementation of the X-Train system was delayed. NIH does not expect to meet the FY 2000 and FY 2001 targets to increase the amount of trainee appointment and termination information received electronically until FY 2003. In FY 2002, the NIH electronically received and processed 238 (3%) of all training appointments. By the end of FY 2004, nearly all NRSA trainee appointments should be received electronically.

Goal f) Improve the capabilities for career outcome tracking for NIH training and career development programs.[1]

NIH recognizes the importance of developing, managing, and improving its training and career development programs based on sound knowledge about their effectiveness. To improve its capabilities for gathering this knowledge, NIH has focused its effort on two major activities: 1) evaluating the career outcomes of recipients of NIH training grants, fellowships, and career development awards and 2) expanding the X-Train system to monitor appointment information and to establish a link to the professional profile on all NIH trainees.

NIH has undertaken several evaluations of career outcomes related to its research training and career development awards. For example, in 1998, NIH initiated a comprehensive study of the career outcomes of the National Research Service Award (NRSA) programs for both predoctoral and postdoctoral research training. The evaluations were designed to examine how many NRSA recipients apply for and receive subsequent NIH fellowship support, apply for and receive subsequent NIH research grant support, publish in peer-reviewed journals, and remain in scientific careers. In March 2001, the NIH published a comprehensive career outcome evaluation for predoctoral NRSA recipients called the *Early Career Progress of NRSA Predoctoral Trainees and Fellow*s. The report very clearly shows that individuals who earn the Ph.D. in biomedical sciences have more successful academic and research careers if they have had nine or more months of NRSA support. This report is available at <http://grants.nih.gov/training/ career_ progress/index.htm>. The study of postdoctoral career outcomes should be available in 2003.

During FY 2001, NIH conducted an early assessment of the effectiveness of the Mentored Patient-Oriented Research Career Development Award (K23) program. The purpose of this assessment was to identify, through focus groups, which career development experiences are related to successful career outcomes. The findings should be useful in improving review criteria and monitoring awards.

To establish a professional profile on all NIH trainees, NIH plans to enhance the Electronic Trainee Activities System (X-Train). In FY 2001, NIH began expanding the use of X-Train to document NRSA appointments. In 2003, the NIH will begin linking X-Train to electronically maintained Professional Profiles. These profiles will serve as a source of long-term career outcome information for NRSA-supported individuals. By 2003, the procedure used to track the appointments and careers of NRSA awardees will be extended to predoctoral and postdoctoral research assistants. This will greatly expand access to information about the larger research workforce and the pool of graduate and postdoctoral trainees and research assistants. It also will provide valuable information about the post-training careers of NIH-supported graduate students and post doctorates.

[1] NOTE: This goal overlaps with goal e (electronic administration of training activities) and will be phased out after FY 2003 in deference to that broader goal.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Evaluate the career outcomes of recipients of NIH fellowships and career development awards:						
Complete an evaluation study of NIH pre- and postdoctoral training programs based on existing data. (Note: This target has been superseded by the two targets that immediately follow.)	→	×				
Complete a report on career outcomes of recipients of NIH extramural predoctoral research training support.		◆				
Complete a report on career outcomes of recipients of NIH extramural postdoctoral research training support.			→	→	◇	
Conduct an early evaluation of the K23 program based on focus groups composed of recipients.			◆			
Develop a plan for ongoing evaluations of NIH research training programs employing data in the professional profile.				→	◇	
Expand the X-Train system to establish a professional profile on all NIH trainees:						
Add training activities functions to the NIH Commons.	→	→	→	◆		
Initiate preliminary work on the long-term tracking database. This is the professional profile database that is already an integral part of IMPAC II.		→	→	◆		
Develop and deploy X-Train, version 2.0 as a means of collecting appointment and termination information on NRSA recipients.				→	◇	
Develop capacity to use X-Train information to establish a professional profile for trainees. The professional profile will serve as a source of long-term career tracking information.				◆		
Link information in X-Train to the professional profile on 90% of all trainees.					◇	

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	× Target Not Met

Summary of Performance Results

- **NIH made progress in evaluating the career outcomes of recipients of NIH fellowships and career development awards.** For efficiency purposes, NIH divided the FY 1999 target to complete a comprehensive evaluation of pre- and postdoctoral training programs into two studies. One study focused on the career outcomes of predoctoral NRSA recipients and the other study focused on the career outcomes of postdoctoral NRSA recipients. NIH made progress on both evaluations, but neither study was completed in FY 1999 as originally planned. However, NIH published the final report for predocs, *The Early Career Outcomes of NRSA Predoctoral Trainees and Fellows,* in March 2001. The predoctoral evaluation found that individuals who receive at least nine months of NRSA support for predoctoral research training in the biomedical sciences perform better than their colleagues without NRSA training on many significant indicators including the length of time to degree, pursuit of postdoctoral training, employment, and grant support. This study also showed that NRSA

recipients publish more scientific papers and those papers are more highly cited when compared to individuals who trained at the same time in the same field but did not have NRSA support. It is clear from this study that the predoctoral NRSA training program is meeting and exceeding its overall mission of producing a cadre of highly skilled health-related researchers. For the postdoctoral study, NIH began compiling the data sets in FY 1999; the data are being analyzed, and NIH hopes the report will be published by the end of FY 2003. The person who originally developed the data for this study worked for NIH under an Interagency Personnel Agreement. The postdoctoral study was delayed after the Agreement expired.

With respect to the K23 program evaluation, focus group analyses were completed on schedule in FY 2001 and the results are posted on the Research Training and Career Development Program website.

- **NIH developed the X-Train system to process appointments to training grants and to establish a professional profile on all NIH trainees, but experienced delays in expanding the X-Train system for other functions.** The X-train software was available for internal testing for several months in FY 1999, but NIH could not establish linkages to the Professional Profile database. By the end of FY 2001, X-Train version 1.5 had been deployed in a limited fashion, and information from the limited pool of users was useful in enhancing the system. In FY 2002, NIH was able to establish a professional profile for all incoming trainee appointments presently received through X-Train. Assuming the removal of funding delays related to the eRA project, NIH expects that a mature version of X-Train system, with appropriate links to the Professional Profile database, will be operational by the end of FY 2003.

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2.3 Research Facilities Program

2.3.1 Program Description and Context

NIH's Research Facilities Program addresses the objective to "secure facilities for research that are modern, efficient and safe." This core program has two functional areas: 1) Intramural Modernization and Improvements and 2) Extramural Assistance.

Intramural Modernization and Improvements. NIH has over 200 Federally-owned facilities that include over 11 million gross square feet of space on 1,380 acres of land, which must keep pace with the demands of rapidly changing technologies and priorities in biomedical and behavioral research.

NIH Intramural Facilities

Regional Campuses

- More than 70 buildings on the Bethesda, Maryland campus
- Leased and Government owned facilities on the Bayview Campus, Baltimore, Maryland
- Facilities at Frederick Cancer Research and Development Center at Fort Detrick, Maryland.
- NIH Animal Center in Poolesville, Maryland.

Field stations

- Rocky Mountain Laboratory in Hamilton, Montana
- A laboratory in New Iberia, Louisiana
- Facilities at the National Institute of Environmental Health Science (NIEHS) campus in Research Triangle Park, North Carolina

Construction of NIH buildings and facilities began more than 60 years ago, dating back to the June 1936 legislation that authorized Building 1 on the Bethesda campus. In fact, most of NIH's intramural facilities were constructed decades ago. As a result, NIH is contending with an aging physical plant. Many buildings, facilities, and utility systems have reached, or are nearing, the end of their useful lives. In addition, the emergence of new technologies, the need to improve physical security on NIH campuses, the evolving scope of medical research, and a variety of other factors lead to facility obsolescence and the need for modernization and replacement of facilities. Managing these capital assets is integral to the success of the NIH intramural research program in achieving its research goals and the national health goals established by DHHS.

NIH's Intramural Modernization and Improvements Program (IMIP) facilitates the development of annual facility planning, programming, budgeting and construction execution strategies, which include the energy reduction objectives of the National Energy Conservation Policy Act and Executive Order 13123, compliance with Executive Order 13148, Greening the Government Through Leadership in Environmental Management, as well as other facility management initiatives. The IMIP is the product of a systematic process of interaction between the facility planning, programming, design, and construction components of NIH and the various NIH Institutes and Centers (ICs). Other program inputs include technologically-driven protocols and advancements; facility assessments and surveys; engineering studies; compliance with building, environmental and other regulatory requirements; accreditation guidelines of the Association of Assessment and Accreditation of Laboratory Animal Care International (AAALAC) and the Joint Commission on Accreditation of Healthcare Organizations (JCAHO) that identify the programmatic and operating requirements for the

patient care and research environment; politically-driven research initiatives; physical security requirements; and recommendations of the approved NIH Facilities Master Plan.

Requirements of the Buildings and Facilities (B&F) program are reviewed and prioritized by a Facilities Planning Advisory Committee (FPAC), which includes senior executives from a cross-section of NIH. The Committee's effort culminates in the development of NIH's Strategic Facilities Plan, which helps shape NIH's Buildings and Space Plan, the Agency Capital Plan, the Federal Capital Improvements Program, and a Six-Year Facility Budgeting Plan.

The B&F Program

- Safety & regulatory compliance
- New construction
- Renovations
- Equipment & systems
- Repair & improvements
- Physical security improvements

The B&F program encompasses six broad program areas that are linked to existing or emerging intramural research needs, regulatory or accreditation guidelines and standards, and/or the unique operating requirements, Federal mandates and government-wide security objectives, and age of the facilities in NIH's inventory. New facilities are programmed for construction or existing facilities are renovated or upgraded on a case-by-case basis, depending on the most viable option to support current and emerging research and technological advancements. The focus of the B&F program is to provide facilities that are in compliance with applicable physical security, safety, accreditation, and other regulatory requirements; efficient in terms of indoor and outdoor environment and energy consumption; and effective in meeting the needs of intramural researchers.

Extramural Assistance. Biomedical research facilities are a critical component of the nation's science and engineering research infrastructure. The availability and condition of biomedical research space directly affects the scope and quality of the biomedical research conducted at the nation's colleges, universities, medical schools, hospitals, and other research organizations. Providing extramural assistance to institutions for the purpose of improving their research capabilities is integral to the success of the NIH extramural research program in achieving its research goals and the national health goals established by DHHS.

The Public Health Service Act (Title IV, Section 481A, *Modernization and Construction of Facilities*) authorizes NIH to "make grants to public and non-profit private entities to expand, remodel, renovate, or alter existing research facilities or construct new research facilities" for biomedical and behavioral research and research training. Under the NIH extramural research facilities construction programs, construction grants for extramural research facilities are awarded competitively. Grantee institutions are required to obtain matching funds for the specific project awarded. NIH construction grants support the costs of design, renovation, and construction of non-federal basic and clinical research facilities. These grants address the biomedical and behavioral research, research training, or research support needs of an institution. Facility construction that may be supported under this program includes construction of new facilities, additions to existing buildings, completion of uninhabitable "shell" space in new or existing buildings, and major alterations and renovations. NIH extramural assistance encompasses "bricks and mortar" modernization as well as total replacement of existing research facilities, providing new capabilities to promote innovative research activity.

To identify current needs for extramural assistance, NIH collaborates with the National Science Foundation in conducting a biennial survey, which is designed to assess the condition of existing facilities nationwide and to identify needs for new and refurbished research facilities. The survey provides the major source of objective data for national research infrastructure policy and planning needs.

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2.3.2 Summary of Performance – Research Facilities Program

Comprehensive summary tables covering all the goals and targets in NIH's Research Facilities Program follow. These tables provide updated information on the status of all of the program's performance targets. More extensive information on each goal, including a chart summarizing the performance results for each target, can be found at the referenced page number.

INTRAMURAL MODERNIZATION AND IMPROVEMENTS			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference[1]**
a) Improve the operating conditions and environment of intramural facilities and the availability and reliability of NIH utility distribution systems to support intramural research. (Note: This goal is part of normal operations and will not be continued past FY 2003.)	**FY 2003** 1. Complete at least 90% of projects planned for each fiscal year to correct building and utility system deficiencies and to improve the interior and exterior of buildings and off-campus facilities consistent with standard industry practices and as necessary to ensure safe and efficient operations of NIH intramural research facilities. 2. Complete construction of Third-Party-Financed 23 megawatts Cogeneration Plant. *Also see FY 2002 target, which has been extended to FY 2003.*	Performance will be reported in February 2004.	D – 241
	FY 2002 Complete at least 90% of planned projects to correct building and utility system deficiencies and to improve the interior and exterior of buildings and off-campus facilities consistent with standard industry practices and as necessary to ensure safe and efficient operations of NIH intramural research facilities. *Also see FY 2001 targets #1 and #2,which have been extended to FY 2002.*	This target was mostly met.	
	FY 2001 1. Continue projects to correct building and utility system deficiencies and to improve the interior and exterior of buildings and off-campus facilities consistent with standard industry practices and as necessary to ensure safe and efficient operations of NIH intramural research facilities. (Note to reader: Interpret to mean complete projects planned for FY 2001).	1. All scheduled projects to correct building and utility system deficiencies were started, and 80% of the scheduled work was completed by the end of FY01. In the 3rd quarter of FY 2002, the goal was met.	

[1] —Indicates that the goal is part of the President's Management Agenda
SP—Indicates the DHHS Strategic Plan goal to which each GPRA goal pertains
HP #—Indicates the Chapter of "Healthy People 2010" to which each Goal pertains
D #—Indicates the page in this report at which details on the goal can be found.

INTRAMURAL MODERNIZATION AND IMPROVEMENTS			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference[1]**
	2. Complete construction of campus-wide utility distribution systems, renovation and modernization of existing boilers, and extension of the power plant to provide the necessary equipment to support the heating and cooling requirements of facilities on the NIH Bethesda campus.	2. By the end of FY 2001, NIH completed construction of the campus-wide utility distribution systems and renovation and modernization of existing boilers. 95% of the power plant extension project was completed. The target was met in January 2002.	
	FY 2000 1. Continue projects to correct building and utility system deficiencies and to improve the interior and exterior of buildings and off-campus facilities consistent with standard industry practices and as necessary to ensure safe and efficient operations of NIH intramural research facilities. (Note to reader: Interpret to mean complete projects planned for FY 2001). 2. Complete construction of 90% of planned utility systems for the west and north sections of the campus; complete utility systems supporting the southeast, south, and southwest sections of the campus.	1. The balance of the exterior and interior projects was 100 % complete in the second quarter of FY 2001. 2. 100% of the site utilities were completed.	
	FY 1999 1. Continue projects to correct building and utility system deficiencies and to improve the interior and exterior of buildings and off-campus facilities consistent with standard industry practices and as necessary to ensure safe and efficient operations of NIH intramural research facilities. (Note to reader: Interpret to mean complete projects planned for FY 1999). 2. Complete construction of the Utility Tunnel Extension Project and the relocation of underground utilities to support the Power Plant Extension Project.	1. The balance of the exterior and interior projects was 100 % complete in the second quarter of FY 2000. The campus utility systems were 100% complete in the third quarter of FY 2000. 2. 98% of the Power Plant project was completed in FY 1999 and it was 100% complete in FY 2000. The Utility Tunnel Extension Project was completed as planned.	
b) Complete the Mark O. Hatfield Clinical Research Center.	**FY 2004** Complete the project within the Congressionally approved, revised - budget.	Performance will be reported in February 2005.	D – 245

INTRAMURAL MODERNIZATION AND IMPROVEMENTS			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference[1]**
	FY 2003 Complete 95% construction of the facility, as determined by actual payments against the construction payment schedule	Performance will be reported in February 2004.	
	FY 2002 Complete 75% of the construction, as determined by actual payments against the construction payment schedule. *Also see FY 2000 target, which was extended to FY 2002.*	NIH completed 75% of the construction for the Mark O. Hatfield CRC project, as scheduled, in FY 2002.	
	FY 2001 Complete 50% of the construction.	Construction reached the 50% phase.	
	FY 2000 Complete the superstructure and exterior wall system.	65% of the superstructure was completed in FY 2000. The target was met in December 2001.	
	FY 1999 Complete the design and the first phase of site work.	NIH completed 95% of the scheduled design and 100% of the site work in FY 1999. The design was completed in FY 2001.	
c) Complete the Warren Grant Magnuson Clinical Center Revitalization Program.	**FY 2004** Complete 25% of the construction for the interim renovations, as determined by actual payments against the construction payment schedule and 100% of the design effort for Phase 1 of the Building 10 Renovation Program.	Performance will be reported in February 2005.	D – 247
	FY 2003 Complete design for and start construction of the interim renovations and complete 80% of the design effort for Phase I of the Building 10 Renovation Program.	Performance will be reported in February 2004.	
	FY 2002 Start design for the interim renovations and for Phase I of the Building 10 Revitalization Program.	Target partially met and extended to FY 2003. The developer manager has been selected and NIH expects to award the contract early in calendar year 2003.	
d) Maintain the quality of the NIH Animal Care Program and construct a Central Vivarium/Animal Research Center (ARC).	**FY 2003** Complete 35% of design documents for the facility and 95% of construction documents for site work and building foundation.	Target extended because it will not be met in FY03.	D – 249

INTRAMURAL MODERNIZATION AND IMPROVEMENTS			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference[1]**
	FY 2002 Award a Developer Manager contract for the overall execution of the project and obtain the services of an Architect-Engineering firm to perform the design. *Also see FY 2001 target, which was extended to FY 2002.*	Target not met and extended.	
	FY 2001 Award a Developer Manager contract, select the design architect-engineering firm, and complete the schematic design. *Also see FY 2000 target, which was extended to FY 2001.*	Completion of programming had been scheduled for FY 2002. Selection of a development manager and design team has been postponed pending decisions on anticipated appropriations.	
	FY 2000 Begin design for the Central Vivarium/ARC site work and foundation and the programming effort for the facility.	The design began in the summer of 2001. NIH had planned to complete the design by the fall of 2002 in parallel with the facility programming effort in FY 2002.	
	FY 1999 Receive accreditation from the Association of Assessment and Accreditation of Laboratory Animal Care International.	AAALAC accreditation received.	
e) Complete the John Edward Porter Neuro-science Research Center on the NIH Bethesda campus.	**FY 2004** 1. Complete 100% of the construction for this project phase (Phase I.) 2. Begin construction for Phase II of this project.	Performance will be reported in February 2005.	D – 251
	FY 2003 Complete 30% of the construction for this project phase, as determined by actual payments against the construction payment schedule.	Performance will be reported in February 2004.	
	FY 2002 Start construction of the 200,000 gross square feet facility on the Building 35 site.	This target was met.	
	FY 2001 Assemble project team and begin demolition of Building 35.	The project team has been assembled and demolition of the facility has begun.	

INTRAMURAL MODERNIZATION AND IMPROVEMENTS			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference[1]**
f) Establish a systematic process to manage and account for NIH's Real Property Inventory.	**FY 2003** 1. Develop and test FIRMplus to augment utility of FIRM.[2] 2. Deploy FIRM plus to users.[2] 3. Identify, pilot, and launch a commercial off-the-shelf replacement to FIRM that is endorsed by HHS/OS/OFM.	Performance will be reported in February 2004.	 SP – 8 D – 254
	FY 2002 (See FY 2001 targets #2 and #3.)		
	FY 2001 1. Launch a one-year pilot program for the Foundation Information for Real Property Management (FIRM) and integrate the lessons-learned into NIH's formalized accounting and reporting procedures for real property management. 2. Validate the NIH real property inventory and populate the FIRM database with the appropriate facility descriptive information, size, function, initial cost, and replacement cost. 3. Provide FIRM online monitoring and reporting capabilities at the desk of each stakeholder involved with real property management.	1. NIH launched the pilot program. Integration of lessons-learned into internal procedures was initiated. 2. In FY 2001, all elements of this target were completed except for the requirement to document replacement cost. The target was met in December 2001. 3. The FIRM software was determined to be inadequate. NIH had planned to develop FIRMplus.	
g) Enhance NIH's ability to deter and respond to security threats by implementing campus and facility management initiatives.	**FY 2004** 1. Finalize development of a system and standards for facility security classifications that are appropriate to NIH using the Department of Justice and Interagency Security Committee (ISC) Security Design Criteria and begin assessment of all NIH facilities against established security classification standards. 2. Complete Perimeter Fence and Guardhouse construction, NIH Bethesda Campus, within the congressionally approved budget. 3. Complete design and award the construction contract for the Visitor Center, NIH Bethesda Campus. 4. Complete 50% of the initial security enhancements, Rocky Mountain Laboratories, Hamilton, Montana and the Research Triangle Park, NC Campuses.	Performance will be reported in February 2005.	SP – 2 D – 256

[2] FY 03 targets 1 and 2 for Goal f may be superseded by new target "3".

INTRAMURAL MODERNIZATION AND IMPROVEMENTS			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference[1]**
	5. Complete design and award the contract for construction of the NIH Bethesda Campus Loop Road.		
	FY 2003 1. Complete development of 50% of the standards systems for facility security classifications that are appropriate to NIH using the Department of Justice and Interagency Security Committee (ISC) Security Design Criteria. 2. Complete design and the award construction contract for the perimeter fence and Guardhouse, NIH Bethesda Campus. 3. Award the design contract of the Visitor Center, NIH Bethesda Campus. 4. Begin initial security enhancements, Rocky Mountain Laboratories, Hamilton, Montana and the Research Triangle Park, NC Campuses. 5. Award the design contract for the NIH Bethesda Campus Loop Road.	Performance will be reported in February 2004.	
	FY 2002 Begin development of a system and standards for facility security classifications appropriate to NIH using Department of Justice and Interagency Security Committee (ISC) Security Design Criteria.	The target was met.	
h) Provide biodefense research facilities by implementing design and construction actions.	**FY 2004** 1. Complete 60% of the construction for the BSL-4 Research Laboratory, Rocky Mountain Laboratories, Hamilton, Montana, as determined by actual payments against the construction payment schedule. 2. Complete 100% of the design and 50% of the construction for the BSL-3 Research Laboratory Complex, Bethesda, MD, as determined by actual payments against the construction payment schedule. [3]	Performance will be reported in February 2005.	SP – 2 D – 258

[3] The FY04 targets have been revised to include the design phase

INTRAMURAL MODERNIZATION AND IMPROVEMENTS			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference[1]**
	3. Complete 100% of the design and 35% of the construction for the BSL-4 Research Laboratory, Ft. Detrick, MD[4] as determined by actual payments against the construction payment schedule.[3]		
	FY 2003 1. Complete 40% of the construction for the BSL-4 Research Laboratory, Rocky Mountain Laboratories, Hamilton, Montana, as determined by actual payments against the construction payment schedule. 2. Complete 5% of the construction for the BSL-3 Research Laboratory Complex, Bethesda, MD, as determined by actual payments against the construction payment schedule. 3. Complete 5% of the construction for the BSL-4 Research Laboratory, National Cancer Institute-Frederick Research Center, Frederick, MD, as determined by actual payments against the construction payment schedule.	Performance will be reported in February 2004.	
i) Increase use of feasible, cost effective, environmental sustainability strategies in NIH facilities by empowering a senior management team accountable for the manage-ment and implementation of the Strategic Plan for Environmental Sustainability (SPES).	**FY 2004** 1. Complete review of facility planning, design and construction guidelines, and revise to include sustainability features and systems as a standard business practice. 2. Complete review of facility operating procedures and revise to maximize use of sustainability procedures to reduce reliance and the drain on natural resources.	Performance will be reported in February 2005.	D – 259
	FY 2003 1. Formalize an NIH SPEC to focus on achieving energy and environmental sustainability goals. 2. Establish an NIH Advisory group to develop an appropriate Environmental Management System responsive to EO 13148.	Performance will be reported in February 2004.	

[3] The FY04 targets have been revised to include the design phase

[4] The "BSL-4 Research Laboratory, Ft. Detrick, MD" was referred to as the BSL-4 Research Laboratory. "National Cancer Institute, Fredrick Research Center, Fredrick, MD" in the FY 2003 target.

INTRAMURAL MODERNIZATION AND IMPROVEMENTS			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference[1]**
	3. Review land use guidelines and modify to reflect the sustainability philosophy. 4. Begin review of facility planning, design and construction guidelines and revise to include sustainability features and systems as a standard business practice. 5. Begin review of facility operating procedures and revise to maximize use of sustainability procedures to reduce reliance and the drain on natural resources.		

EXTRAMURAL ASSISTANCE			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference**[1]
a) Approve an optimal percent of construction designs by the end of the third year that are in compliance with Federal and NIH design regulations and guidelines, and with other relevant local, national, and international codes and standards. NOTE: This goal is part of the normal extramural facilities process and will not be continued after reporting on FY2002 targets.	**FY 2002** 1. Final construction design documents approved for 75% of grants awarded in FY 1999. *Also see FY 2001 targets #1 and #3 and FY 2000 target #1,which were extended to FY 2002.*	Final construction design documents were approved for 77% of grants awarded three years earlier (in FY 1999).	D – 263
	FY 2001 Final construction design documents approved for: 1. 100% of grants awarded in FY 1998. 2. 50% of grants awarded in FY 1999. 3. 25% of grants awarded in FY 2000.	1. Of the 22 construction awards made in FY 1998, the Division of Engineering Services has approved 20 (91%) construction design documents. The final drawings from the two remaining institutions are expected in FY 2003. 2. Of the 31 construction awards made in FY 1999, the Division of Engineering Services has approved 16 (52%) construction design documents. 3. Of the 45 construction awards made in FY 2000, the Division of Engineering Services approved 10 (22%) construction design documents in FY 2001. During the first month of FY 2002 the Division of Engineering Services approved an additional 2 for a total of 12 (26%) construction approved design documents, and the target was met.	
	FY 2000 Final construction design documents approved for: 1. 100% of grants awarded in FY 1997. 2. 50% of grants awarded in FY 1998.	1. Target met in FY 2002. 2. Final construction design documents were approved for 77% of grants awarded.	

[1] —Indicates that the goal is part of the President's Management Agenda
SP—Indicates the DHHS Strategic Plan goal to which each GPRA goal pertains
HP #—Indicates the Chapter of "Healthy People 2010" to which each Goal pertains
D #—Indicates the page in this report at which details on the goal can be found.

EXTRAMURAL ASSISTANCE			
Performance Goals	**FY Targets**	**Actual Performance**	**Reference**[1]
	3. 25% of grants awarded in FY 1999.	3. Final construction design documents were approved for 23% of grants awarded. By the third quarter of FY 2001, the documents were approved for 40% of grants awarded.	
	FY 1999 100% of awarded construction projects meet Federal and NIH safety and architectural design regulations and are in compliance with the scope of the application.	The earliest completion date for this target is FY 2003. 89% of awarded projects received final construction design approval by April 2002.	

GPRA Research Facilities Program						
Budget (dollars in thousands)	**FY 1999 Actual**	**FY 2000 Actual**	**FY 2001 Actual**	**FY 2002 Actual**	**FY 2003 Amended President's Budget**	**FY 2004 Request**
	$239,343	$251,747	$248,386	$429,553	$1,273,739	$83,208

2.3.3 Program Performance Analysis

2.3.3.1 Intramural Modernization and Improvements

The Intramural Modernization and Improvements Program (IMIP) constructs, renovates, and maintains research facilities at the National Institutes of Health's Bethesda; MD, Frederick, MD; Research Triangle Park, NC; and Hamilton, MT campuses. This program provides the facility infrastructure critical to the conduct and management of NIH research.

The IMIP is responsible for the following:

1) Providing facilities that are code compliant, safe, well maintained, energy-efficient, and functionally adequate for the conduct of state-of-the-art biomedical research.
2) Ensuring that NIH facilities comply with research and accreditation guidelines and will be deemed appropriate/adequate in peer reviews of the programs they house.
3) Expanding and improving the campus-wide utility systems to eliminate interruptions in service and to keep pace with forecast utility demand.
4) Continuing facility renovation, improvement, and new construction projects in order to modernize or replace facilities that are failing and/or are inadequately organized and equipped to support current and emerging research requirements.
5) Maintaining a complete and accurate Real Property inventory in conformance with the requirements of the Chief Financial Officers Act.
6) Providing the physical security infrastructure that enables the NIH Security Program to protect staff, visitors, patients, intellectual assets, and property from a variety of threats.

NIH is tracking the performance of the IMIP by measuring accomplishment against nine explicit goals:

a) Improve the operating conditions and environment of intramural facilities and the availability and reliability of NIH utility distribution systems to support intramural research.

b) Complete the Mark O. Hatfield Clinical Research Center.

c) Complete the Warren Grant Magnuson Clinical Center Revitalization Program.

d) Maintain the quality of the NIH Animal Care Program and construct a Central Vivarium/ARC.

e) Complete the John Edward Porter Neuroscience Research Center on the NIH Bethesda campus.

f) Establish a systematic process to manage and account for NIH's Real Property Inventory.

g) Enhance NIH's ability to deter and respond to security threats by implementing campus and facility management initiatives.

h) Provide research facilities to support biodefense by implementing design and construction actions.

i) Increase incorporation of feasible, cost-effective, environmental sustainability strategies into NIH planning, development, and operations by empowering a senior management team accountable for the management and implementation of the Strategic Plan for Environmental Sustainability (SPES).

Performance Goal Details - Intramural Modernization and Improvements

Goal a) Improve the operating conditions and environment of intramural facilities and the availability and reliability of NIH utility distribution systems to support intramural research.[1]

The physical conditions of NIH's intramural research facilities influence the efficiency and effectiveness of the research program. Inadequate environmental controls, insufficient utilities and services, system breakdowns and malfunctions due to inadequate maintenance, and outmoded technology at a minimum impair the orderly conduct of research. In the more serious cases, system failures and the consequential loss of research materials set research back by months and, in some cases, years.

As a result, NIH strives to ensure that all modernization and improvements to intramural research facilities keep pace with research and patient care demands, rapidly changing technological advancements, and research priorities. NIH's efforts to improve the reliability and quality of the intramural research environment are focused on the following:

1. Reducing the number of deferred maintenance and repair projects.
2. Completing the expansion of utility system capacity on schedule.

Across the Federal Government, under-funding of routine and preventive maintenance is systemic; NIH is no exception. The National Research Council of the National Academy of Sciences recommends that agencies annually invest two to four percent of the replacement value of their capital facilities in maintenance. Over the past generation, the NIH has not funded maintenance at this level. NIH's intramural facilities include buildings and utility systems that range in age from 20 to 40 years. Many of these systems are nearing their useful life expectancy. As a result, an extensive backlog of maintenance and repair work (including architectural, structural, mechanical, plumbing, and electrical system repairs) exists.

To systematically reduce the backlog, NIH established procedures to identify, track, monitor, and correct the numerous building deficiencies. In addition, NIH strives to achieve adequate funding levels for routine, preventive, and deferred building and infrastructure maintenance repairs. However, only by eliminating the backlog and by funding maintenance in the future at the levels recommended by the NAS will the NIH be able to meet this goal.

The NIH also has to increase the capacity of the utility systems that serve these facilities and new facilities in the pipeline. Despite its outstanding record in energy management, utility consumption at NIH facilities is growing. Current research tools and techniques utilize more electric power than techniques of the past. Current environmental control standards generate

[1] Note: This goal is part of normal operations and will not be continued past FY 2003.

higher utility loads. Moreover, NIH is building new facilities. To keep pace with this increase in demand, NIH must expand utility capacity.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Complete the most important projects involving repair and maintenance of NIH facilities:						
Continue projects to correct building and utility system deficiencies and to improve the interior and exterior of buildings and off-campus facilities consistent with standard industry practices and as necessary to ensure safe and efficient operations of NIH intramural research facilities. (Note to reader: Interpret to mean complete projects planned for FY 1999).	→	◆				
Continue projects to correct building and utility system deficiencies and to improve the interior and exterior of buildings and off-campus facilities consistent with standard industry practices and as necessary to ensure safe and efficient operations of NIH intramural research facilities. (Note to reader: Interpret to mean complete projects planned for FY 2000).		→	◆			
Continue projects to correct building and utility system deficiencies and to improve the interior and exterior of buildings and off-campus facilities consistent with standard industry practices and as necessary to ensure safe and efficient operations of NIH intramural research facilities. (Note to reader: Interpret to mean complete projects planned for FY 2001).			→	◆		
Complete at least 90% of planned projects to correct building and utility system deficiencies and to improve the interior and exterior of buildings and off-campus facilities consistent with standard industry practices and as necessary to ensure safe and efficient operations of NIH intramural research facilities. (Note: target was extended, then superceded by the target that immediately follows).				→	◇	
Complete at least 90% of projects planned for each fiscal year to correct building and utility system deficiencies and to improve the interior and exterior of buildings and off-campus facilities consistent with standard industry practices and as necessary to ensure safe and efficient operations of NIH intramural research facilities.					◇	
Complete construction of new NIH utility systems on schedule:						
Complete construction of the Utility Tunnel Extension Project and the relocation of underground utilities to support the Power Plant Expansion Project.	→	◆				
Complete construction of 90% of planned utility systems for the west and north sections of the campus; complete utility systems supporting the southeast, south, and southwest sections of the campus.		◆				
Complete construction of campus-wide utility distribution systems, renovation and modernization of existing boilers, and extension of the power plant to provide the necessary equipment to support the heating and cooling requirements of facilities on the NIH Bethesda campus.			→	◆		
Complete construction of a Third-Party-Financed 23 megawatts Cogeneration Plant.					◇	

◆ **Target Met**	◇ **Target Active**	→ **Target Not Met and Extended**	✕ **Target Not Met**

Summary of Performance Results

- **NIH made progress in reducing the number of deferred projects involving the repair and maintenance of its facilities.** In FY 1999, NIH completed all but 8% of the exterior and interior repairs and all but 14% of the campus utility systems projects that were part of the backlog slated to be addressed. The balance of this work was completed in February 2000 and June 2000, respectively. In FY 2000, NIH completed all but 7% of the interior and exterior building systems projects that were in the backlog being addressed and the balance of the work was completed in February 2001.

  Substantial progress in reducing the number of deferred projects was made in FY 2001. Specifically, 100% of the roof repairs and off-campus facility repair were completed on schedule. Due to funding constraints, however, only 80% of the projects to correct building and utility system deficiencies and only 86% of the exterior and interior repair projects were completed by the end of the fiscal year; the balance of the work in these categories was completed in the third quarter of FY 2002.

  The NIH achieved its FY2002 objective of completing 90% or greater of all work scheduled to correct campus utility deficiencies, roof repairs and off-campus facility repairs. In the exterior and interior facility repair category, the NIH completed 88% in lieu of 90% of its targeted goal. The balance of this work will be completed in the 4th quarter of FY2003 when funds become available to complete this effort. Projects that were carried over from FY2001 were completed in the 3rd quarter of FY2002 as scheduled.

FY 2002 ASSESSMENT SUMMARY

IMPROVE THE MAINTENANCE OF INTRAMURAL FACILITIES

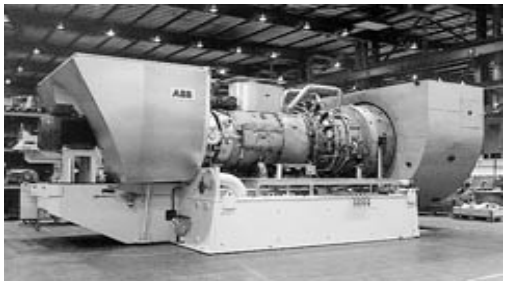
Backlog Areas	Scheduled Projects	Completed Projects	Projects in Progress	Completion Percentage
Campus Utilities	20	18	2	90%
Roof Repairs	7	7	0	100%
Exterior and Interior Repairs	17	15	2	88%
Off Campus Facility Repairs	10	10	0	100%
Total	54	50	4	93%

Note: Campus Utilities and the Exterior/Interior projects under this goal will be completed in the fourth quarter of FY 2003.

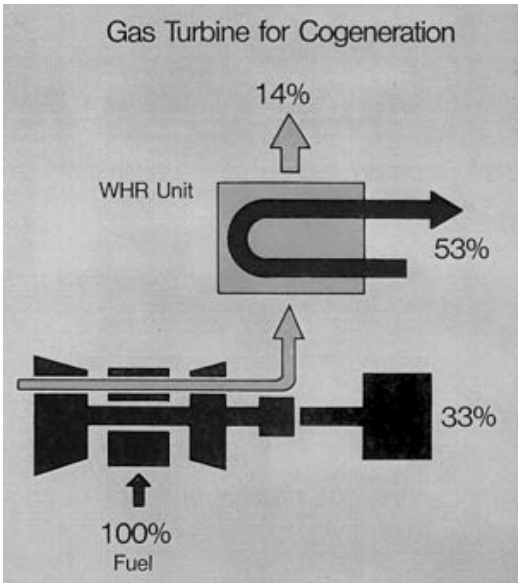
- **NIH made substantial progress in constructing new utility systems.** NIH achieved its FY 1999 target to complete construction of the Utility Tunnel Extension Project. NIH also completed 98% of the work required to relocate the underground utilities for the Power Plant Expansion Project by the end of the fiscal year; the balance of the work was completed in FY

2000. In FY 2000, NIH completed 100% of the planned utility system projects required to support buildings in five sections of the NIH campus, exceeding its targeted goal of a 90% completion percentage.

During FY 2001, construction of the campus-wide utility distribution systems was completed on schedule, and the project to renovate and modernize boilers was completed ahead of schedule. The power plant extension project was 95% complete by the end of the fiscal year; its completion was delayed by unforeseen rock conditions that were encountered during the excavation phase of the project. A revised completion date of January 2002 was set. The target was met in May 2002. The delay in meeting the re-set target was attributable to chiller unit performance issues, which have been resolved. The delay did not impact NIH's research mission.



The gas combustion turbine that is part of the cogeneration system as installed in the Utility Plant is shown above.



This gas turbine cogeneration system diagram illustrates the heat combustion process that results in the generation of electricity and steam.

Goal b) Complete the Mark O. Hatfield Clinical Research Center.

The Mark O. Hatfield Clinical Research Center (CRC) will provide 78,965 gross square meters (850,000 gross square feet) for a 250-bed research hospital, allied clinical facilities, and adjacent laboratories. The CRC will become the new heart of the intramural research program.

A congressional review of the importance of NIH's intramural research program and the vital role that clinical research plays in the success of its mission revealed the need for a new clinical research center. Design began in 1997 and construction in 1999.

Construction has been progressing steadily; however, the cost of the CRC has significantly increased, which has led to a change in the Construction Manager. NIH has undertaken steps to obtain a firm price and construction completion schedule commitments from a new Construction Manager. In November 2001, NIH received the guaranteed maximum price (GMP) proposal for the balance of the uncompleted work. The GMP exceeded the budget by $144.5 million and extended the construction schedule to March 2004. A plan was developed to fund the increase in the costs to the project.

The new Clinical Research Center under construction as of October 2002.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Complete the design and first phase of site work.	→	→	◆			
Complete the superstructure and exterior wall system.		→	→	◆		
Complete 50% of the construction.			◆			
Complete 75% of the construction.				◆		
Complete 95 % construction of the facility, as determined by actual payments against the construction payment schedule.					◇	
Complete the project within the congressionally approved, revised budget.						◇

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- **Construction of the Hatfield Clinical Research Center moves forward.** During FY 1999, NIH completed the first phase of the site work on schedule (in January 1999) and nearly completed the building design on schedule, with 95% of the work achieved by the end of the fiscal year. NIH completed the design in FY 2001, following finalization of programmatic issues involving the optimal use of space.

  During FY 2000, the construction project progressed on schedule until NIH encountered unforeseen conditions during placement of the utility distribution systems on the site. As a result, NIH completed only 65% of the superstructure by the end of FY 2000. Because construction of the exterior wall system depends on completion of the superstructure, its construction had to be delayed. The target was met in December 2001.

  In FY 2001, NIH successfully met its target to complete 50% of the construction. However, due to changes in market conditions and termination of the construction manager for breaches in the terms of his contract, NIH revised the FY 2002 target to complete 75% of the construction. NIH completed 75% of the construction for the Mark O. Hatfield CRC project, as scheduled, in FY 2002.

Goal c) Complete the Warren Grant Magnuson Clinical Center Revitalization Program.

NIH is planning to renovate space in the fifty-year-old Warren Grant Magnuson Clinical Center (WGMCC, Building 10) into flexible, "modern," laboratories. Building 10 has undergone numerous renovations and expansions in the past. Studies indicate that the major building systems providing critical electrical power, lighting, heating, ventilation, air conditioning, and plumbing services in Building 10 vary in age and condition, do not have the capacity to meet the current demands of research, and are at the end of their service life. As a result, routine maintenance and system repairs cannot keep pace with the vast number of utility infrastructure deficiencies and operating inefficiencies in the facility.

The project will replace failing, obsolete building systems with new, state-of-the-art systems sized to meet anticipated demands. These new systems will eliminate existing potentially unsafe environmental and life safety conditions. The new systems will also be less costly to operate and maintain than the existing systems.

Success of the Building 10 Revitalization Program depends on completing the interim construction efforts on various floors of the facility, and doing so within the approved budget.[1] This interim construction will provide space for administrative and laboratory programs that will remain in Building 10 after the CRC is completed. Without this action, NIH will not be able to provide safe, efficient, and code-compliant space for programs not included in the new CRC and programs that are integral to and support the Building 10 Revitalization Program. Phase I of the Building 10 Revitalization Program can begin once NIH completes the interim renovations.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Start design for the interim renovations and for Phase I of the Building 10 Revitalization Program.				→	◇	
Complete design for and start construction of the interim renovations and complete 80% of the design effort for Phase I of the Building 10 Renovation Program.					◇	
Complete 25% of the construction for the interim renovations, as determined by actual payments against the construction payment schedule, and 100% of the design effort for Phase I of the Building 10 Renovation Program.						◇

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- In FY 2002, discussions began with the DHHS concerning the development concepts and options proposed for Phase I of this project. The developer manager that will handle the

[1] The target to bring the project in within budget will appear in the final project year.

management, design and construction acquisition process for this project has been selected. Contract award is pending DHHS guidance in the January-February 2003 timeframe. Design for the interim renovations associated with this project is underway. This FY 2002 target was partially met and will slip to FY 2003.

Goal d) Maintain the quality of the NIH Animal Care Program and construct a Central Vivarium.

A quality laboratory animal research program is an essential part of the Intramural Research Program. In order to sustain its laboratory animal research program, NIH must maintain high quality, adaptive animal care facilities that comply with the standards for accreditation set by the Association of Assessment and Accreditation of Laboratory Animal Care International (AAALAC). These facilities must be suitable for research using the range of current animal models and be adaptable to changing research demands.

An important part of the NIH strategy for maintaining excellence in its laboratory animal research program is the construction of a modern, compact, and state-of-the-art Central Vivarium/Animal Research Center (ARC). The ARC will replace existing animal facilities; consolidate ongoing animal programs into an efficient, effective, and well-functioning space; and provide space for emerging research needs.

Construction will provide central utilities; site work, animal holding, receiving, and quarantine areas; procedure rooms; specialized laboratories; administrative support spaces; and the necessary mechanical, electrical, and other utility systems needed to comply with AAALAC accreditation guidelines and other applicable building codes and regulations. The other aspect of the NIH strategy is vigilant maintenance of its existing facilities so they remain in compliance with AAALAC standards.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004[1]
Maintain high quality animal care facilities for the NIH intramural research program:						
Receive accreditation from the Association of Assessment and Accreditation of Laboratory Animal Care International.	◆					
Complete construction of the Central Vivarium/Animal Research Center on schedule:						
Begin design for the Central Vivarium site work and foundation and the programming effort for the facility.		→	→	→	→	→
Award a Developer Manager contract, select the design architect-engineering firm, and complete the schematic design.			→	→	→	→
Award a Developer Manager contract for the overall execution of the project and obtain the services of an Architect-Engineering firm to perform the design.				→	→	→
Complete 35% of design documents for the facility and 95% of construction documents for site work and building foundation.					→	→

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

[1] Capital facility planning scenarios indicate that funding for the Animal Research Center may not be available until 2005 and beyond.

Summary of Performance Results

- **NIH maintains high quality animal care facilities for the intramural research program.** NIH met the FY 1999 target to receive accreditation from AAALAC on schedule. AAALAC surveyors conducted a site visit during the week of July 26, 1999. NIH received confirmation of "Deferred Continued" AAALAC accreditation based on the recommendations of the site visit team.

- **Construction of the Central Vivarium/ARC has been delayed.** NIH partially achieved the FY 2000 target to begin the facility programming effort, with discussions underway at the end of the fiscal year. However, before commencing with design work, NIH administrators felt it was prudent to examine, in concert with the update of the Bethesda campus master plan, which programs and functions would be optimal for placement in the ARC. Facility programming discussions, planning activities, and analyses were conducted to ensure that the facility would support NIH's science mission, complement future development in the south quadrant of the campus, meet NIH's strategic animal research needs, and reflect newly emerging animal research modeling requirements.

  Completion of programming had been scheduled for December 2002. However, capital facility planning scenarios indicate that funding for the Animal Research Center is not currently available. Selection of a development manager and design team has been postponed pending decisions on anticipated appropriations for the project.

Goal e) Complete the John Edward Porter Neuroscience Research Center on the NIH Bethesda campus.

The John Edward Porter Neuroscience Research Center (NRC) on the Bethesda campus will provide a collaborative environment for state-of-the-art neuroscience research with biomedical research laboratories, research support, a vivarium (for both small animals and primates), lab offices, conference facilities, a cafeteria, interaction areas, and shared public spaces. When completed, the NRC will be a multi-level facility with approximately 53,500 gross square meters (575,000 gross square feet), designed and constructed to be sensitive to the site and the adjacent structures.

Creating the NRC is crucial to plans for improving the efficiency and effectiveness of the neuroscience research program at NIH. This goal is very important because nearly all of the space currently housing NIH neuroscience programs is unsuitable by today's research standards. During the past 30 years, NIH was unable to renew buildings that house neuroscience research and, as a result, the facilities available to NIH neuroscientists did not keep pace with technological breakthroughs in genetics and imaging.

In addition to using outdated facilities, the neuroscience program at NIH is dispersed among several Institutes and Centers. Scientists are segregated in one or more preclinical departments and removed from colleagues in the clinical departments of neurology, psychiatry, neurosurgery, or anesthesiology. Furthermore, NIH conducts most of its cellular and molecular neuroscience in Building 36, where most of the laboratories in the facility are partitioned into small modules, separated by concrete walls. Eliminating these artificial barriers would create an environment where scientists could focus more intently on fundamental and clinical research, collaborate more productively, and translate their scientific findings more quickly into effective therapies for neurological and psychiatric disorders.

An artist's rendering of the Neuroscience Research Center

Completing construction of the NRC efficiently and on schedule requires a phased approach. The initial phase includes demolishing an existing older building (Building 35) and constructing the first 23,500 gross square meters (250,000 gross square feet) of the NRC. The neuroscience research program housed in Building 36 will then be relocated into this new facility. Building 36 will then be demolished and the remaining 30,250 gross square meters (325,000 gross square feet) of the NRC will be constructed.

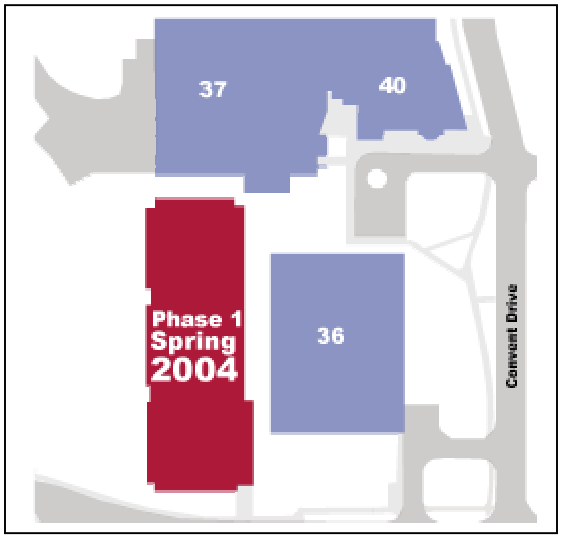
Keeping the project within the congressionally approved budget is an integral element of plan to complete the NRC. A target addressing that aim will appear for the final project year.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Assemble project team and begin demolition of Building 35.			◆			
Start construction of the 200,000 gross square feet facility on the Building 35 site.				◆		
Complete 30% of the construction for this project phase (Phase I), as determined by actual payments against the construction payment schedule.					◇	
Complete 100% of the construction for this project phase (Phase I).						◇
Begin construction for Phase II of this project.						◇

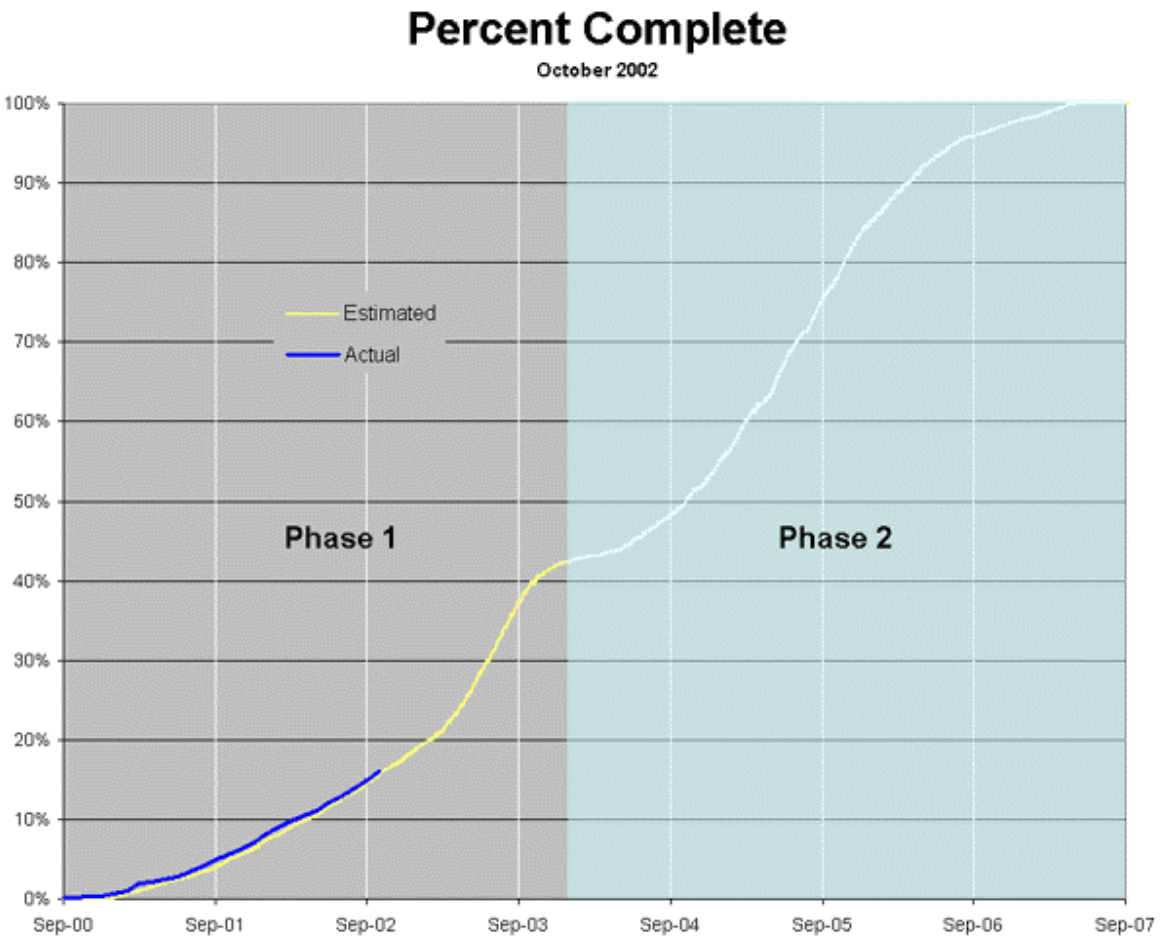
◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- **Construction of the Porter Neuroscience Research Center is on schedule.** The project team responsible for overseeing the construction of the NRC was established and met during FY 2001, and the demolition of Building 35 began on schedule. Construction for this project started in FY 2002 as scheduled.



The Phase I construction project to replace building 35 is shown in the darkened silhouette with the scheduled completion date.


Percent Complete
October 2002
100%
90%
80%
70%
60%
50%
40%
30%
20%
10%
0%
Estimated
Actual
Phase 1
Phase 2
Sep-00
Sep-01
Sep-02
Sep-03
Sep-04
Sep-05
Sep-06
Sep-07

Goal f) Establish a systematic process to manage and account for NIH's Real Property Inventory.

Accounting for real property as capital assets is required under the Chief Financial Officers Act (CFO). In 1999, during the first audit under the CFO Act, NIH worked diligently to assemble the records necessary to pass the audit. This was a challenge as the NIH had no integrated system of records that captured ownership data, depreciation, construction in progress (CIP), and lease information for its expanding portfolio of owned and leased facilities.

Recognizing the need to establish a systematic process to manage and account for NIH's real property inventory, NIH began a one-year pilot program in FY 2001 to test the suitability of the DHHS mandated Foundation Information for Real Property Management (FIRM) system being developed by the General Services Administration (GSA). If the pilot program indicated that the system was comprehensive and could satisfy NIH's needs, it would be implemented.

The pilot revealed that FIRM is inadequate. While it can be used to record and track legal property descriptive information, the size, function, and initial cost of capital assets, the system does not handle depreciation, CIP, or estimated out-year lease payments. In response NIH had planned to develop FIRMplus, an application that would have augmented the utility of FIRM.

In July GSA announced that it is discontinuing the support of FIRM as of the beginning of FY04. Upon receiving this news NIH began to investigate the viability of Peregrine's Facility Center (now called TRIRIGA FacilityCenter™) as the real estate data management platform for NIH real property records. NIH is also defining the role of the NIH Business and Research Support System (NBRSS) property module in real property record management. Finally, with the creation of the HHS/OS/OFM, the question of a Department-wide real property management data system is also being considered. While a final decision is being made, NIH will temporarily replace FIRM with a commercial off-the-shelf system that is endorsed by the Department.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Test and implement the Foundation Information for Real Property Management (FIRM) system:						
Launch a one-year pilot program for FIRM and integrate the lessons-learned into NIH's formalized accounting and reporting procedures for real property management.			◆			
Validate the NIH real property inventory and populate the FIRM database with the appropriate facility descriptive information, size, function, initial cost and replacement cost.			→	◆		
Provide FIRM online monitoring and reporting capabilities at the desk of each stakeholder involved with real property management.			→	×		

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Augment FIRM with FIRMplus:						
Develop and test FIRMplus to augment utility of FIRM. [1]					◇	
Deploy FIRMplus to users. [1]					◇	
Replace FIRM with a Department endorsed commercial off-the-shelf system:						
Identify, pilot, and launch a commercial off-the-shelf replacement to FIRM that is endorsed by HHS/OS/OFM.					◇	

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- **NIH made progress in testing and implementing the FIRM system**. In FY 2001, NIH successfully launched a one-year pilot program to test and implement the FIRM system. NIH also populated the FIRM database in FY 2001 and validated all of the information in the inventory except facility replacement costs, which were validated in December 2001. Weaknesses were identified during the pilot and NIH decided to explore alternative means to manage its capital assets. NIH had planned to develop FIRMplus to augment the shortfalls of the FIRM. However, in July 2002, based on an independent verification and validation of the FIRM product and agency input that included the NIH assessment, GSA determined that FIRM is inefficient and could not be economically modified to meet real property management needs.

  Because GSA is discontinuing support of FIRM, NIH did not provide FIRM online monitoring and reporting capabilities at the desk of each stakeholder involved with real property management. However, key people do have this capacity. Also, because a decision has been made to utilize a Department-endorsed commercial off-the-shelf system, to temporarily replace FIRM, NIH hopes to replace the previous FY 2003 GPRA targets (set only in FY 2002) if such a change during the targeted year is possible.

[1] Targets will be superseded by new FY 2003 target if possible to make such a change during the targeted fiscal year.

Goal g) Enhance NIH's ability to deter and respond to security threats by implementing campus and facility management initiatives.

In response the terrorist attacks of September 11, 2001, and in line with recommendations of several security studies, including a report by the DHHS Inspector General, the NIH is taking coordinated action on several fronts to bolster its security program. The main elements of this initiative include the following:

- Development and promulgation of easily understood and implementable standards that define NIH operations at each of the national alert levels (green through red).
- Completion of risk assessment and risk classification of each of NIH's facilities.
- Design and construction of facility improvements that will facilitate access control and harden facilities against penetration and attack.

Integral to each initiative, is the aim of adhering to the congressionally approved budget. Targets addressing that aim will appear for the final year of each initiative.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Improve facility security classification and assessment:						
Begin development of a system and standards for facility security classifications that are appropriate to NIH using the Department of Justice and Interagency Security Committee (ISC) Security Design Criteria.				◆		
Complete development of 50% of the standards systems for facility security classifications that are appropriate to NIH using the Department of Justice and Interagency Security Committee (ISC) Security Design Criteria.					◇	
Finalize development of a system and standards for facility security classifications appropriate to NIH using Department of Justice & Interagency Security Committee (ISC) Security Design Criteria and begin assessing NIH facilities against the standards.						◇
Plan, design, and construct projects that address site security requirements.						
Complete design and begin construction of the perimeter fence and Guardhouse, NIH Bethesda Campus.					◇	
Complete Perimeter Fence and Guardhouse construction, NIH Bethesda Campus within the congressionally approved budget.						◇
Begin design of the Visitor Center, NIH Bethesda Campus.					◇	
Complete design and begin construction of the Visitor Center, NIH Bethesda Campus.						◇
Begin initial security enhancements, Rocky Mountain Laboratories, Hamilton, Montana and the Research Triangle Park, NC Campuses.					◇	
Complete 50% of the initial security enhancements, Rocky Mountain Laboratories, Hamilton, Montana and the Research Triangle Park, NC Campuses.						◇
Award the design contract for the NIH Bethesda Campus Loop Road.					◇	
Complete design and begin construction of the NIH Bethesda Campus Loop Road.						◇

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

In FY 2002, the NIH met the target to begin development of a system and standards for facility classifications. A Security Operations Advisory Committee (SOAC) consisting of senior level members of the NIH and some outside expert consultants has been established. This committee considers the guidance received from the Office of Homeland Security and the DHHS in addressing security policy decisions and interim guidelines, until NIH specific security criteria are developed. As part of its efforts, the SOAC is developing facility security criteria applicable to owned and leased facilities, taking into consideration current Department of Justice and the Interagency Security Committee (ISC) Security Design Criteria.

Goal h) Provide biodefense research facilities by implementing design and construction actions.

Since the anthrax attacks in the fall of 2001, the need for specialized containment facilities to support research on highly infectious pathogens and their toxins has escalated. New facilities to conduct safe, state-of-the-art research on the transmission, pathogenesis, and prevention of infectious diseases are necessary. Biocontainment laboratories designated as Biosafety Level (BSL)-3 and BSL-4 are required to fulfill research goals and objectives related to bioterrorism preparedness. The aim to keep each project within its congressionally approved budget will be embodied in targets for the final year of each project.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Design and construct biodefense research support facilities:						
Complete 40% of the construction for the BSL-4 Research Laboratory, Rocky Mountain Laboratories, Hamilton, Montana, as determined by actual payments against the construction payment schedule.					◇	
Complete 60% of the construction for the BSL-4 Research Laboratory, Rocky Mountain Laboratories, Hamilton, Montana, as determined by actual payments against the construction payment schedule.						◇
Complete 5% of the construction for the BSL-3 Research Laboratory Complex, Bethesda, MD, as determined by actual payments against the construction payment schedule.					◇	
Complete 100% of the design and 50% of the construction for the BSL-3 Research Laboratory Complex, Bethesda, MD, as determined by actual payments against the construction payment schedule.[1]						◇
Complete 5% of the construction for the BSL-4 Research Laboratory, National Cancer Institute, Fredrick Research Center, Fredrick, MD, as determined by actual payments against the construction payment schedule.					◇	
Complete 100% of the design and 35% of the construction for the BSL-4 Research Laboratory, Ft. Detrick, MD,[2] as determined by actual payments against the construction payment schedule.[1]						◇

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	✕ Target Not Met

Summary of Performance Results

- NIH established this goal in FY 2002. The first targets are for FY 2003; therefore, no performance has been reported to date. Initial reporting will begin in February 2004.

[1] The FY04 targets have been revised to include the design phase.
[2] "The BSL-4 Research Laboratory, Ft. Detrick, MD" was referred to as "the BSL-4 Research Laboratory, National Cancer Institute, Fredrick Research Center, Fredrick, MD" in the FY 2003 target.

Goal i) Increase use of feasible, cost effective, environmental sustainability strategies in NIH facilities by empowering a senior management team accountable for the management and implementation of the Strategic Plan for Environmental Sustainability (SPES).

NIH has made significant strides toward the establishment of a formalized Strategic Plan for Environmental Sustainability (SPES) as a tool to manage compliance with Executive Order 13123, Greening the Government Through Efficient Energy Management, Executive Order 13148, Greening the Government Through Leadership in Environmental Management, and the April 1, 2002, memorandum from the Chairman, Council on Environmental Quality, Executive Office of the President. Since 1992, NIH has implemented an aggressive Transportation Management Plan (TMP) that has resulted in a reduction of peak-hour traffic flow, energy conservation, and reduction in green house gas production.

NIH's Energy Management and Conservation Program includes short-and long-term goals and objectives to manage energy use and consumption and to reduce emission of greenhouse gasses that are harmful to the environment. Energy conservation efforts include use of alternative fuel vehicles, retrofit of facilities with energy saving devices including ballasts and lamps, integration of energy saving technologies into new construction, and the construction of a Co-generation Plant. Planning and design criteria for land use, facility design, construction, and operations are being modified to place greater emphasis on sustainability from an initial and Life Cycle Cost standpoint.

To help facilitate this effort, the NIH plans to bring together under a single leadership/ management team several previously independent efforts: transportation management, recycling, building energy conservation, and sustainable development.

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Provide responsive and accountable management of natural resources:						
Formalize an NIH SPES to focus on achieving energy and environmental sustainability goals.					◇	
Establish an NIH Advisory Group to develop an appropriate Environmental Management System responsive to EO 13148.					◇	
Review land-use guidelines and modify to reflect the sustainability philosophy.					◇	
Begin review of facility planning, design and construction guidelines and revise to include sustainability features and systems as a standard business practice.					◇	
Complete review of facility planning, design, and construction guidelines and revise to include sustainability features and systems as a standard business practice.						◇
Begin review of facility operating procedures and revise to maximize use of sustainability procedures to reduce reliance and the drain on natural resources.					◇	

Performance Targets Note: Annual targets are grouped by activity.	**FY 1999**	**FY 2000**	**FY 2001**	**FY 2002**	**FY 2003**	**FY 2004**
Complete review of facility operating procedures and revise to maximize use of sustainability procedures to reduce the reliance and drain on natural resources.						◇

◆ **Target Met**	◇ **Target Active**	→ **Target Not Met and Extended**	✕ **Target Not Met**

Summary of Performance Results

- NIH established this goal in FY 2002. Therefore, no performance has been reported to date. Initial reporting will begin in February 2004.

2.3.3.2 Extramural Assistance

Biomedical research facilities are a critical component of the nation's science and engineering research infrastructure. The availability and condition of biomedical research space directly affects the scope and quality of the biomedical research conducted at the nation's colleges, universities, medical schools, hospitals, and other research organizations.

NIH's extramural research facilities construction programs work to address this important need for more biomedical research facilities. The broad priorities for these programs are the following:

- Respond to requests from the extramural research community for financial assistance in undertaking research facility modernization and construction.
- Conduct critical reviews to ensure that the construction of such facilities is safe and appropriately designed to enable the conduct of high quality research.

NIH is authorized under the Public Health Service Act, Title IV, Section 481A "Modernization and Construction of Facilities" to "make grants to public and non-profit private entities to expand, remodel, renovate, or alter existing research facilities or construct new research facilities" for biomedical and behavioral research and research training.

Under the NIH extramural research facilities construction programs, construction grants for extramural research facilities support the costs of design, renovation, and construction of non-federal basic and clinical research facilities. These grants address the needs of an institution or a research area at an institution for biomedical and behavioral research, research training, and/or research support. Facility construction that may be supported under this program includes construction of new facilities, additions to existing buildings, completion of uninhabitable "shell" space in new or existing buildings, and major alterations and renovations. This "bricks & mortar" modernization and replacement of existing research facilities provides new capabilities that can open areas to innovative research activity.

NIH collaborates with the National Science Foundation in conducting a biennial survey to assess the condition of existing facilities nationwide and to identify needs for new and refurbished research facilities. The survey provides the major source of objective data for national research infrastructure policy and planning needs. The most recent comprehensive survey, which was published in October 2000 using data from 1998, indicated that more than half of the nation's 908 biomedical research performing institutions had inadequate research space to meet their research commitments and that $5.6 billion in needed construction and renovation had to be deferred because of insufficient funds. In recognition of these findings, NIH offers funding opportunities on a competitive basis.

The number of extramural research facility construction awards that NIH makes varies from year to year. The number of awards is dependent on the level of funds provided by Congress as well as the number of applications received that are deemed scientifically meritorious. For example, in FY 2002, 58 awards totaling $113.1 million were made, and in FY 2001, 44 awards totaling $87.3 million were made.

Each construction grant application undergoes a two-tiered peer review. First, an NIH scientific and technical peer review group evaluates applications for scientific and technical merit. Next, the National Advisory Council or Board of the funding Institute or Center conducts a second level of review. Reviewers evaluate applications to determine how the proposed change in the research environment would facilitate the applicant institution's ability to conduct, expand, improve, or maintain biomedical research. It is through this two-tiered peer review process that NIH ensures that awarded construction grants have high scientific and technical merit and meet the changing needs of the research environment.

Applicants must ensure the availability of matching funds for the construction project. Then, when a grant is awarded, NIH must approve the construction designs before construction may begin. The designs are reviewed by engineers at NIH and must meet applicable codes before approval. Review by the engineers, who have expertise in the design of biomedical facilities, also helps to ensure that the facility will be designed in a way that maximally supports biomedical research.

In order to encourage project completion and to review whether the construction is following the approved designs, NIH may conduct site visits during construction and/or after project completion.

Performance Goal Details - Extramural Assistance

Goal a) Approve an optimal percent of construction designs by the end of the third year that are in compliance with Federal and NIH design regulations and guidelines, and with other relevant local, national, and international codes and standards.[1]

NIH construction grants for extramural research facilities support the costs of design, renovation, and construction of non-Federal basic and clinical research facilities. Although NIH provides funding for these construction projects, it does not have oversight responsibility over a grantee's completion of a construction project. However, if the grantee does not complete the project within the designated timeframe, generally five years, the funds awarded by NIH to the recipient institution revert to the Federal Government.

The time between award and construction is contingent on the grantee submitting satisfactory designs. Depending on the nature of the project, the grantee may be required to submit up to three sets of designs: schematic, development, and final construction designs. NIH reviews the designs to ensure they are in accordance with Federal and NIH design regulations and guidelines, as well as being in accordance with other related local, national, and international codes and standards.

If the grantee does not obtain approval for its final construction designs by the end of the third year, the building may not be completed successfully. Therefore, NIH focuses on ensuring that grantees' final construction designs are reviewed and approved in a timely manner. A third year approval rate of 65% or less is considered unacceptable, 66% to 69% is considered fair, 70% to 74% is considered good, and 75% and above is considered excellent. Previously, NIH also set performance targets regarding the approval of final construction designs in the first and second years of construction, but these targets were dropped after FY 2001 to focus on the third year, which is considered the most significant year for construction grants.

[1] NOTE: This goal is part of the normal extramural facilities process and will note be continued after reporting on FY2002 targets.

Performance Targets Note: Annual targets are grouped by activity.	FY 1999	FY 2000	FY 2001	FY 2002	FY 2003	FY 2004
Ensure that grantees' final construction designs are reviewed and approved in a timely manner:						
100% of awarded construction projects meet Federal and NIH safety and architectural design regulations and are in compliance with the scope of the application.	→	→	→	→	◇	
Final construction design documents approved for 100% of grants awarded in FY 1997.		→	→	◆		
Final construction design documents approved for 100% of grants awarded in FY 1998.			→	→	◇	
Final construction design documents approved for 75% of grants awarded in FY 1999.				◆		
Approve 50% of final construction designs by the end of the projects' second year: [1]						
Final construction design documents approved for 50% of grants awarded in FY 1998.		◆				
Final construction design documents approved for 50% of grants awarded in FY 1999.			◆			
Approve 25% of final construction designs by the end of the projects' first year: [1]						
Final construction design documents approved for 25% of grants awarded in FY 1999.		→	◆			
Final construction design documents approved for 25% of grants awarded in FY 2000.			→	◆		

◆ Target Met	◇ Target Active	→ Target Not Met and Extended	× Target Not Met

Summary of Performance Results

- **Progress has been made in ensuring that grantees' final construction designs are reviewed and approved in a timely manner.** For the FY 1999 target, 100% of the construction grants awarded by NIH in the previous three years (FY 1996-1999) received scientific peer review and concurrence, but only 43% received engineering design review and approval of their final construction designs during FY 1999. By September 2002, 99% had been approved and it is anticipated that the 100% target for final construction design approval of these grants should be met in FY 2003. NIH met the FY 2000 target in FY 2002, when 100% of the final construction design documents for grants awarded three years earlier (in FY 1997) were approved. For the FY 2001 target, NIH approved 96% of the final

[1] The tracking of the percent of final construction designs approved during the projects' first year and second year was discontinued after FY 2001 in order to focus on the third year, which is considered the most significant year for construction grants.

construction design documents for grants awarded three years earlier (in FY 1998) and the 100% target should be met in FY 2003. For the FY 2002 target, NIH met the target as final construction design documents were approved for 77% of grants awarded three years earlier (in FY 1999).

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PART III
APPENDIX TO THE PERFORMANCE PLAN

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Appendix 1

Linkage to HHS and Agency Strategic Plans

The ways in which the NIH Performance Plan links to the HHS Strategic Plan and other Administration planning documents are indicated in the Summaries of Performance for each Program in Part II of the Plan. The far right column of each summary is used for reference. Codes indicate linkage to various HHS Strategic Plan goals and linkage with other important priority setting activities including, the Department's 10-year health objectives for the Nation known as "Healthy People 2010," and the President's Management Agenda.

At the request of the Department, NIH reviewed the goals and measures in the GPRA Plans of all other HHS Agencies to check for inconsistencies with the NIH Plan. NIH is pleased to report that no inconsistencies were found. However, NIH did identify several similar goals and measures. Those similarities also are noted in the summary tables in Part II. Where NIH goals link to goals of other HHS Operating Divisions, a code has been placed in the reference column and the link is discussed below.

Similar Measures in Plans of Other Agencies	NIH Goal/Target	Comments
Agency for Healthcare Research and Quality (AHRQ)		
AHRQ 1, Research and Training Key Outcome - Increase the number of minority researchers trained as health services researchers	NIH Training Support and Outreach Goal d - Increase the participation of underrepresented racial and ethnic groups, women, and individuals with disabilities in NIH training and research programs.	The aims do not conflict. The NIH goal address the need for biomedical and behavioral researchers, while the AHRQ goal addresses training for *health services* researchers.
AHRQ 2, Research and Training Key Outcome - Support training programs for junior-level researchers and mid-career scientists to emerging and innovative research methods – establish baseline number of programs	NIH Training Support and Outreach Goal b - Maintain adequate application and award rates in key training support areas.	The aims do not conflict. The NIH goal address the need for biomedical and behavioral researchers, while the AHRQ goal addresses training for *health services* researchers.
Centers for Disease Control and Prevention (CDC)		
CDC 1 Chronic Diseases: Community-Based Prevention Research – Support prevention research to develop sustainable and transferable community-based behavioral interventions.	NIH Research Outcomes Goal 9c - By FY 2010, identify culturally appropriate, effective stroke prevention programs for nationwide implementation in minority communities.	The aims are complementary.

Similar Measures in Plans of Other Agencies	NIH Goal/Target	Comments
CDC 2 Chronic Diseases; Heart Disease and Stroke; Performance Goal 2 - Reduce death and disability due to heart disease and stroke and eliminate disparities. Measure - Reduce the proportion of heart disease and stroke deaths that occur before transport to emergency services.	NIH Research Outcomes Goal 9c - By FY 2010, identify culturally appropriate, effective stroke prevention programs for nationwide implementation in minority communities. NIH Communication of Results Goal c - Increase awareness of NIH-sponsored research results among the general public. *Target* – Extend the impact of the "Know Stroke: Know the Signs. Act in Time." campaign.	The aims are complementary.
CDC 3 Environmental Health; Biomonitoring; Performance Goal 1 – Develop laboratory capacity to monitor human exposure to chemicals in the environment.	NIH Research Outcomes Goal 6c – By 2012, develop a knowledge base on Chemical Effects in Biological Systems using a "systems toxicology" or toxicogenomics approach.	The aims are complementary.
CDC 4 HIV/AIDS Prevention; Overarching Performance Goal; Reduce the number of new HIV infections	NIH Research Outcomes Goal 2d - By 2007, develop an HIV/AIDS vaccine.	The aims are complementary.
CDC 5 HIV/AIDS Prevention; International HIV/AIDS; Performance Goal – Working with other countries, USAID, and international and U.S. government agencies, reduce the number of new HIV infections among 15- to 24-year-olds in sub-Saharan Africa from an estimated 2 million by 2005.	NIH Research Outcomes Goal 2d - By 2007, develop an HIV/AIDS vaccine.	The aims are complementary.

Similar Measures in Plans of Other Agencies	NIH Goal/Target	Comments
CDC 6 Tuberculosis; Performance Goal – Eliminate Tuberculosis in the United States	NIH Research Outcomes Goal 3b - By 2010, develop one universal antibiotic effective against multiple classes of biological pathogens.	The aims are complementary.
CDC 7 Infectious Disease Control; Performance Goal 1 – Protect Americans from infectious diseases	NIH Research Outcomes Goal 3b - By 2010, develop one universal antibiotic effective against multiple classes of biological pathogens. Goal 4a – By 2005, develop two new animal models to use in research on at least one agent of bioterror. Goal 8a - By 2007, determine the genome sequence of an additional 45 human pathogens and three invertebrate vectors of infectious diseases.	The aims are complementary.
CDC 8 Eliminating Racial and Ethnic Disparities; Performance Goal 1 - Improve the lives of racial and ethnic populations who suffer disproportionately from the burden of disease and disability and develop tools and strategies that will enable the nation to eliminate these health disparities by 2010.	NIH Research Outcomes Goal 9c - By FY 2010, identify culturally appropriate, effective stroke prevention programs for nationwide implementation in minority communities. NIH Communication of Results Goal b – Targets -	The aims are complementary.

Similar Measures in Plans of Other Agencies	NIH Goal/Target	Comments
CDC 9 Terrorism; Preparedness and Response Capacity; Performance Goal – Enhance the capacity of CDC and state and local health departments to prepare for and respond to biological, chemical, radiological, and mass trauma hazards related to terrorism.	NIH Research Outcomes Goal 3b - By 2010, develop one universal antibiotic effective against multiple classes of biological pathogens. Goal 4a – By 2005, develop two new animal models to use in research on at least one agent of bioterror. Goal 8a - By 2007, determine the genome sequence of an additional 45 human pathogens and three invertebrate vectors of infectious diseases.	The aims are complementary.
Food and Drug Administration (FDA)		
FDA 1 2.3 Human Drugs, Strategic Goal 1, Performance Goal 6- Facilitate development and availability of medical countermeasures to limit the effects of the intentional use of biological, chemical, or radiologic/nuclear agents.	NIH Research Outcomes Goal e – Develop new or improved methods for treating disease and disability. Goal 5c – Expand the range of available methods used to create, analyze, and utilize chemical libraries, which can be used to discover new medicines. Specifically, use these chemical libraries to discover 10 new and unique chemical structures that could serve as the starting point for new drugs. Goal 3b – By 2010, develop one universal antibiotic effective against multiple classes of biological pathogens. Goal 4a – By 2005, develop two new animal models to use in research on at least one agent of bioterror. Goal 8a – By 2007, determine the genome sequence of an additional 45 human pathogens and three invertebrate vectors of infectious diseases.	The aims are complementary.

Similar Measures in Plans of Other Agencies	NIH Goal/Target	Comments
FDA 2, Human Drugs 2.3 Human Drugs, Strategic Goal 1, Performance Goal 8 - Facilitate the initiation of research in a non-human primate model of pneumonic plague.	NIH Research Outcomes Goal a – Add to the body of knowledge about normal and abnormal biological functions and behavior. Goal 4a – By 2005, develop two new animal models to use in research on at least one agent of bioterror.	The aims are complementary.
FDA 3 2.7 NCTR, Strategic Goal 1, Performance Goal 1 - Introduce the knowledge of new genetic systems and computer-assisted toxicology (toxicoinformatics) into the risk management process.	NIH Research Outcomes Goal b - Develop new or improved instruments and technologies for use in research and medicine. Goal f - Develop critical genomic resources, including the DNA sequences of the human genome and the genomes of important model organisms and disease-causing microorganisms. Goal 6c – By 2012, develop a knowledge base on Chemical Effects in Biological Systems using a "systems toxicology" or toxicogenomics approach.	The aims are complementary.
FDA 4 2.7 NCTR, Strategic Goal 1, Performance Goal 2 - Develop, with other organizations, gene chip and gene array technology.	NIH Research Outcomes Goal b - Develop new or improved instruments and technologies for use in research and medicine. Goal f - Develop critical genomic resources, including the DNA sequences of the human genome and the genomes of important model organisms and disease-causing microorganisms.	The aims are complementary.

Similar Measures in Plans of Other Agencies	NIH Goal/Target	Comments
FDA 5 2.7 NCTR, Strategic Goal 3, Performance Goal 7- Use new technologies (bioinformatics, imaging, proteomics, and metabonomics) for diagnosis of risk. FY 04: Compile scientific knowledge and expertise in new technologies to determine risk and develop appropriate regulatory authority. FY 03: Evaluate, for use Agency-wide, one new technology such as proteomics or genomics for determining liver damage by regulated products.	NIH Research Outcomes Goal b - Develop new or improved instruments and technologies for use in research and medicine. Goal 6a – Identify the genes that control the risk for the development of age-related macular degeneration and glaucoma in humans. Goal 6c – By 2012, develop a knowledge base on Chemical Effects in Biological Systems using a "systems toxicology" or toxicogenomics approach. Goal 7c – By 2005, create the next generation map of the human genome, a so called "haplotype map" (HapMap), by identifying the patterns of genetic variation across all human chromosomes. Goal 8a – By 2007, determine the genome sequence of an additional 45 human pathogens and three invertebrate vectors of infectious diseases.	The aims are complementary.

Appendix 2

Changes and Improvements Over Previous Year

NIH's FY 2004 Annual Plan retains the essential features of the documents submitted in the previous fiscal years. This includes organization of the agency's activities into three aggregated (trans-NIH) Core Programs, including Research, Research Training & Career Development, and Research Facilities. It also includes performance goals that address both program outcomes and the means (processes) by which NIH's programs are implemented.

Summary of Goal Status – FY 1999 to FY 2004

Performance information was updated through November 1, 2002. A summary of the number of goals and targets for FY 1999 through FY 2004 is provided in the table below. This summary is provided only as a descriptive indicator of the status of our targets from one year to the next. Because goals and targets vary with regard to degree of importance to the NIH mission, summary data does not accurately reflect NIH's overall success.

Fiscal Year	Goals	Targets	Level of Achievement							
			Program (# of Goals)	Number of Targets	End of Targeted FY			As of November 1, 2002		
					Met	Extended	Not Met	Met	Extended	Not Met
1999[1]	46	86	Research (36)	62	55	5	2	60	0	2
			Training (4)	16	10	4	2	13	0	3
			Facilities (6)	8	3	5	0	7	1	0
2000[1]	44	88	Research (32)	65	49	14	2	59	3	3
			Training (6)	14	6	5	3	10	0	4
			Facilities (6)	9	3	6	0	9	0	0
2001[1]	36	90	Research (23)	63	52	9	2	61	0	2
			Training (6)	15	10	4	1	11	2	2
			Facilities (7)	12	5	7	0	11	0	1
2002	40	80	Research (26)	58	53	5	0	54	4	0
			Training (6)	15[2]	8	4	2	8	4	2
			Facilities (8)	7	4	3	0	4	3	0

[1] Goals that were completed in FY 1999, FY 2000 were included in this table, but are not reported in this plan.
[2] Data are not yet available for reporting on performance for one target.
[3] Targets (or milestones) for the new research outcome goals are yet to be determined. One placeholder target has been counted for each goal (1a-9c).
[4] FY 2004 targets met early include 1 Technology Transfer target under Goal a and 1 Research Training target under Goal e.
[5] A new target is suggested to supercede 2 existing targets. If this is permitted, the number of facility targets would be 19 and the total number of targets would be 92.

Fiscal Year	Goals	Targets	Level of Achievement							
			Program (# of Goals)	Number of Targets	End of Targeted FY			As of November 1, 2002		
					Met	Extended	Not Met	Met	Extended	Not Met
2003	60	93[3,5]	Research (45)	62[3]	To be reported in February 2004.[4]					
			Training (6)	10						
			Facilities (9)	21[5]						
2004	54	78[3]	Research (42)	54[3]	To be reported in February 2005.[6]					
			Training (5)	10						
			Facilities (7)	14						

[3] Targets (or milestones) for the new research outcome goals are yet to be determined. One placeholder target has been counted for each goal (1a-9c).
[6] FY 2004 targets met early include 1 Technology Transfer target under Goal a and 1 Research Training target under Goal e.

Goal/Target Changes Over the Prior Year

The tables that follow provide a general appraisal of the changes in this year's Annual Plan.

<table>
<tr><th>Section</th><th>General Appraisal of Goal Changes in the FY 2004 Annual Plan
(for specifics, see the detailed goal statements in Part II)</th></tr>
<tr><td>Research Outcomes

Page 63</td><td>
<table>
<tr><th></th><th colspan="6">Fiscal Year</th></tr>
<tr><th></th><th>1999</th><th>2000</th><th>2001</th><th>2002</th><th>2003</th><th>2004</th></tr>
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<tr><td>Number of Targets</td><td>13</td><td>13</td><td>18</td><td>17</td><td>28</td><td>28</td></tr>
</table>
<u>Section Comments</u>
• In the current document, the stage is set for a complete turnover in NIH's Research Outcome Goals. A set of 28 research outcome goals is presented. These 28 goals replace all of NIH's previous research outcome goals for FY 2003 and beyond.
• Goals a-h are retained in this document for FY 2002 reporting purposes.
• In the FY 2001 GPRA report (issued February 2002) four goals (a-d of the FY 2002/2003 plan) were replaced by eight more specific goals (a-h) as *representative* of NIH research outcomes. Those more representative goals were subgoals of specific aspects of the earlier goals. The FY 2003/2004 GPRA plan (issued in September 2002), retained the eight subgoals but noted that they were placeholders pending resolution of discussions with OMB and the Department. At that time, one new goal (k) was added to address NIH's increased emphasis on biodefense. For a short period (in the FY 2002/2003 plan), the original five goals were reduced to four by combining goals d and e.)
<u>Goal-by-Goal Comments</u>
• Previous goals a-h were replaced by goals a-e.
• Goal i) was renumbered to goal f). In a FY 2002 target "and" was changed to "and/or" to conform with original intent. The targets for FY 2003 and FY 2004 were deleted because the goal is being superceded by two of the new goals (7c and 8a).
• Goal j) was renumbered to goal g). The targets for FY 2003 and FY 2004 were deleted because the goal is being superceded by new goal 2d.
• Previous Goal k) on biodefense research, with six targets, was cut now that new goals 3b, 4a, and 8a are in place. That goal was new in the FY 2003/2004 Plan issued this September.
• Goals 1a-9c were added to the plan for FY 2003 and beyond.
</td></tr>
</table>

<table>
<tr><th>Section</th><th>General Appraisal of Goal Changes in the FY 2004 Annual Plan
(for specifics, see the detailed goal statements in Part II)</th></tr>
<tr><td>Communication of Results

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<table>
<tr><th></th><th colspan="6">Fiscal Year</th></tr>
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<tr><td>Number of Targets</td><td>14</td><td>24</td><td>27</td><td>10</td><td>6</td><td>6</td></tr>
</table>
<u>Goal-by-Goal Comments</u>
• Goal b) –A FY99 target on the “Back to Sleep Campaign was edited to delete extraneous (redundant) text that preceded the articulation of the target. The deleted text read “Develop and implement NIH information, education, and outreach programs on specific health issues.”
Three new targets that are follow-ons to the FY99 Back to Sleep campaign target were added.
• Goal e) – The goal remains in the plan only for reporting purposes.
</td></tr>
<tr><td>Technology Transfer

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<table>
<tr><th></th><th colspan="6">Fiscal Year</th></tr>
<tr><th></th><th>1999</th><th>2000</th><th>2001</th><th>2002</th><th>2003</th><th>2004</th></tr>
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<tr><td>Number of Targets</td><td>5</td><td>6</td><td>7</td><td>6</td><td>1</td><td>1</td></tr>
</table>
<u>Goal-by-Goal Comments</u>
• Goal a) – During FY 2002, NIH also met the FY 2004 target of making completion of the NIH On-Line Technology Transfer Training an ongoing requirement for employees. With completion of all targets, this goal has been met and will be dropped from future GPRA plans.
</td></tr>
<tr><td>Grants Administration and Peer Review

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<table>
<tr><th></th><th colspan="6">Fiscal Year</th></tr>
<tr><th></th><th>1999</th><th>2000</th><th>2001</th><th>2002</th><th>2003</th><th>2004</th></tr>
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<tr><td>Number of Targets</td><td>8</td><td>7</td><td>7</td><td>8</td><td>9</td><td>5</td></tr>
</table>
</td></tr>
</table>

Management and Administrative Support

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	Fiscal Year					
	1999	2000	2001	2002	2003	2004
Number of Performance Goals	11	11	3	6	7	6
Number of Targets	16	13	3	17	18	14

Section Comments

- To better align the plan with the President's Management Agenda, two new goals were added that address strategic management of human capital.

Training Support and Outreach

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	Fiscal Year					
	1999	2000	2001	2002	2003	2004
Number of Performance Goals	4	6	6	6	6	5
Number of Targets	16	14	15	15	10	10

Intramural Modernization and Improvements

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	Fiscal Year					
	1999	2000	2001	2002	2003	2004
Number of Performance Goals	5	5	6	7	9	7
Number of Targets	7	6	9	6	21	14

Goal-by-Goal Comments

- Goal f) – A new FY 2003 target is suggested to supersede two existing but no longer relevant FY 2003 targets.
- Goal h) – The FY 2004 targets have been adjusted to reflect changes in the design and construction schedule.

Extramural Assistance

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	Fiscal Year					
	1999	2000	2001	2002	2003	2004
Number of Performance Goals	1	1	1	1	0	0
Number of Targets	1	3	3	1	0	0

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Appendix 3

Partnerships and Coordination

NIH activities complement the efforts of sister HHS agencies in many ways and NIH participates actively in endeavors to coordinate across HHS. In addition, many initiatives are undertaken in partnership with other HHS agencies.

Correlation and Coordination

As a research agency, NIH's relationship with sister HHS agencies is bi-directional, that is, NIH both receives information from, and contributes information to, other operating divisions/agencies (OPDIVs). Information collected by other HHS agencies helps to inform NIH priority-setting processes in important ways. For example, the extensive data on disease prevalence and incidence collected by the Centers for Disease Control and Prevention (CDC) is a key source of knowledge about the burden of illness. In turn, NIH often is an important source of expertise for sister agencies. For example, prior to issuing regulations, the Food and Drug Administration (FDA) often seeks comment from NIH.

Research Coordination Council. In terms of coordination, the recent establishment of the Research Coordination Council is a significant development. The Research Coordination Council (RCC)—an element of the Secretary's "One Department" initiative—was established by Secretary Thompson in October 2001 to streamline research and evaluate Department-wide research priorities to ensure greater efficiencies in research, demonstration, and evaluation. The RCC will continue to provide a forum for developing new ideas to enable HHS components, including the NIH, to take advantage of every opportunity for efficiency in the support and conduct of the Department's research programs.

Healthy People 2010. The coordination activities of the RCC build on a history of department-wide planning. Perhaps the most significant trans-HHS planning effort is the decadal articulation of health objectives, the latest of which is known as Healthy People 2010. NIH is an active participant in this process, lending expertise and vision to the Department's aims for the health status of the Nation.

Additional examples of correlation and coordination include:

- *Adverse Event Reporting*. NIH is working closely with FDA to harmonize human subjects protection regulations and the handling of reports of adverse events. In this regard, NIH has developed a national database for gene transfer clinical research, which includes a reporting format accepted by both the NIH and FDA. The database is called the Genetic Modification Clinical Research Information System, or GeMCRIS. GeMCRIS provides a standardized means for reporting, organizing, and analyzing data in order to enable systematic analysis of data across all clinical studies and to enhance communication and application of knowledge gained from the studies.

- *Autoimmune Disease Research Plan*. A comprehensive research plan to fight autoimmune diseases was prepared by the NIH Autoimmune Diseases Coordinating Committee, a body of government and outside experts. This committee, established in 1998, facilitates collaboration among the NIH institutes, other federal agencies such as the CDC and the FDA, and private organizations. The plan will foster research to identify genetic, environmental and infectious causes of autoimmune diseases and to develop new treatments and prevention strategies.
- *Early Notification System*. The NIH Early Notification System (ENS) facilitates the dissemination of information on upcoming research initiatives. To improve coordination of the Department's research activities, each Operating Division (OPDIV) will identify a contact person for the ENS who will determine if their proposed solicitations should be entered into the ENS and coordinate review within their OPDIV. To assist in this effort, the NIH will offer training sessions of the ENS to the various agency contacts.

Trans-HHS Collaborations

NIH institutes and centers collaborate with other HHS agencies in order to efficiently maximize resources and expertise, advance scientific discoveries, and translate these discoveries into policies and programs that benefit the Nation. Just a few examples of these numerous efforts are described below.

Selected collaborations involving more than one other OPDIV:

- *Next-generation smallpox vaccine initiative:* The National Institute of Allergy and Infectious Diseases (NIAID) is leading a trans-HHS initiative to develop a next-generation smallpox vaccine that can be administered to a broader population than existing smallpox vaccines, which pose substantially increased risks for people with eczema or immune deficiencies and for pregnant women. An intradepartmental task force, consisting of representatives from the Office of Public Health Policy, CDC, FDA, and NIH, is rapidly implementing a research and development plan intended to demonstrate the efficacy and safety of modified vaccinia Ankara, and then license it for use in these and other populations at risk. The work of this task force is of the very highest priority to the NIH and the HHS, and represents an excellent and important example of post-9/11 collaboration.
- *HIV/AIDS Treatment Guidelines*. The Panel on Clinical Practices for the Treatment of HIV Infection meets regularly in order to make reliable up-to-date information on treatment of HIV/AIDS available to healthcare providers. The Panel is a joint effort of the HHS and the Henry J. Kaiser Family Foundation. Co-chaired by the Director of the NIH's National Institute of Allergy and Infectious Diseases, the Panel includes participants from CMS, FDA, HRSA, CDC, and SAMSA. Initially published in 1998, the *Guidelines for the Use of Antiretroviral Agents in HIV-Infected Adults and Adolescents* were constructed as a "living document" and are updated by the Panel as new data emerge.

Selected Collaborations with the Centers for Disease Control and Prevention (CDC):

- *National Health and Nutrition Examination Survey*. Several NIH institutes and centers are collaborating with the CDC on the National Health and Nutrition Examination Survey (NHANES). NHANES is the only national source of objectively measured health data capable of providing accurate estimates of both diagnosed and undiagnosed medical

conditions in the population. NHANES represents a unique collaboration between CDC, NIH, and others to obtain data for biomedical research, public health, tracking of health indicators, and policy development.

- *The National Diabetes Education Program*. The National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) and the Division of Diabetes Translation of the Centers for Disease Control and Prevention jointly sponsor this initiative that involves over 200 public and private partners. The goal of the program is to reduce the illness and deaths associated with diabetes and its complications by increasing public awareness and understanding, improving the knowledge of health care providers, and promoting health care policies that improve the quality of and access to care.

Selected Collaborations with the Food and Drug Administration (FDA):

- *Rhesus Breeding Colony*. The National Institute of Allergy and Infectious Diseases (NIAID) collaborates with the FDA on a colony for breeding Rhesus monkeys for research.
- *Toxicological Assessments*. The National Institute of Environmental Health Sciences (NIEHS) collaborates with the FDA on the conduct of comprehensive toxicological assessments.
- *Workshop on Antioxidants*. The National Center for Complementary and Alternative Medicine (NCCAM) is collaborating with the FDA on a workshop that will address the pros and cons of antioxidants including the role of antioxidants in cancer prevention and tumor biology, and their interactions with conventional chemotherapy and radiotherapy.

Selected Collaborations with the Indian Health Service (IHS):

- *Diabetes in Indian Populations*. The National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) collaborates extensively with the Indian Health Service on research and education regarding diabetes.

Selected Collaborations with the Substance Abuse and Mental Health Services Administration (SAMHSA):

- *Mental Health Services Collaboration:* The National Institute of Mental Health (NIMH) works closely with SAMHSA on a number of specific critical issues. For example, NIMH's services research program sponsors research on topics including the cost-effectiveness of specific mental health treatments, the economic impact of mental disorders, innovative models for assessing and treating mental illness in primary care settings, and research on ways to increase the adoption of appropriate mental health services in every day clinical settings. NIMH has collaborated with SAMHSA to fund research on its demonstration projects. With this collaboration, NIMH pays for the research component while SAMHSA funds the delivery of services; this close relationship provides SAMHSA's programs with a strong research base and ensures that research findings are translated and disseminated to the field.

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Appendix 4

Data Verification and Validation

Types of Data Sources

Data for Quantitative and Other Goals with Definitive Endpoints

Most of NIH's performance goals contain quantitative or otherwise objective targets. Accordingly, in most cases, the basis for performance assessment involves data that are uncontroversial, credible, and open to independent public scrutiny (e.g., material readily available through NIH's website). The data for assessing objective/quantitative performance goals comes from a variety of NIH sources:

Completion of Studies/Actions - Where a goal is to complete an action (e.g., respond to a recommendation), documenting evidence will be provided that confirms the completion or status of the project. Studies and reports developed by and for the use of peer review and advisory councils and other distinguished independent panels and committees are examples of the information useful for this type of GPRA reporting.

Program Evaluation - Objective evaluation studies and analyses are already a well-established component of NIH's regular planning and management activities for its programs. Such studies are used to provide basic data on program performance, identify avenues for program improvement, and consider the implications of emerging issues on program operation. NIH also conducts various special evaluation studies in association with such agencies as the National Academy of Sciences and the National Science Foundation – such as large scale, long-term studies of scientific personnel and training needs, research facilities, and research instrumentation.

Data Tracking and Collection Systems - Most performance comparisons for quantitative goals will be based on data from information systems that are designed to track a particular operation. NIH has established and maintains a number of large-scale databases to meet its ongoing management needs (such as IMPAC – see below) or with other federal agencies (such as Edison – see below). These databases play a role in the agency's GPRA performance assessment process. In general, these are public databases, created over a number of years through competitive proposals and subject to outside review by knowledgeable experts, and are maintained through standard database quality protocols. These data are widely regarded, within and outside of NIH, as providing a credible picture of various aspects of the nation's biomedical research enterprise.

The table below identifies some of the data systems that are currently used at NIH to track and develop data for performance comparisons.

System	Purpose	Types of Data	Goals Verified
IMPAC (Information for Management, Planning, Analysis, and Coordination)	IMPAC is a comprehensive database system covering NIH's extramural research activities.	• Records of research contracts • Records of in-process grant applications • Inter- and intra-agency agreements	• Grants Administration goal d • Agency Management goal d • Training Support goals b, c, d, e, and f
CRISP (Computer Retrieval of Information on Scientific Projects)	CRISP is a searchable database (maintained by NIH) of federally funded biomedical research projects conducted at universities, hospitals, and other research institutions.	• Abstracts and indexing terms for funded research projects	• Research outcome goal g
DCIS (Departmental Contracts Information System)	DCIS provides data collection and reporting capabilities needed to enable DHHS to comply with the reporting requirements mandated by Public Law 93-400.	• Contract actions for awards with an anticipated award value over $25,000	• Agency Management goal b
CMMS (Computerized Maintenance Management System) **PIN** (Project Information Network)	Together, these systems are used to manage and monitor the acquisition, design, construction, modernization, replacement, and/or enhancement of NIH's capital assets.	• Acquisition strategy • Project Status • Proposed schedules • Actual schedules • Proposed costs • Actual costs • Management Reports	• Intramural Modernization goals a, b, c, d, and e
World Wide Web	Worldwide sharing of data, information, images, and sound posted and transmitted electronically.	• Genomic sequences • NIH policy and procedure documents • Clinical Trial databases • Reports on use of web sites	• Communication of Results goal e • Grants Administration goals b and e • Research outcome goal f • Training Support goal a

Data for Descriptive Goals

The "Alternative Form" assessment approach used for many of NIH's research outcome goals poses some unique issues for data validation and verification. Nonetheless, virtually all of the outside advisory groups that have looked at this issue over the last several years (e.g., the White House Office of Science and Technology Policy, National Academy of Sciences panels and committees, the Office of Naval Research, and various other science agencies) have affirmed the centrality of peer review by technical experts in preparing findings about the productivity of basic research programs. (See, for example, the National Academy of Science's 1999 report

Evaluating Federal Research Programs: Research and the Government Performance and Results Act and their 2001 report *Implementing The Government Performance and Results Act for Research*.)

The approach NIH uses to prepare these annual assessments of its research goals relies chiefly on such a peer review process (see Appendix 7 – Approach to Performance Assessment). The most prominent sources of data are science advances validated through the verification process inherent in the course of publication.

Goal-by-Goal Verification and Validation

Research Program

Research Outcomes

***Goal a**) Add to the body of knowledge about normal and abnormal biological functions and behavior.*
***Goal b**) Develop new or improved instruments and technologies for use in research and medicine.*
***Goal c**) Develop new or improved approaches for preventing or delaying the onset or progression of disease and disability.*
***Goal d**) Develop new or improved methods for diagnosing disease and disability.*
***Goal e**) Develop mew or improved methods for treating disease and disability.*

These research outcome goals will be superceded in fiscal year 2003 and beyond. A set of replacement goals that are representative and more specific have been developed.

NIH's progress toward meeting these goals in FY 1999-2001 goals was assessed by a working group of the Advisory Committee to the Director (ACD), NIH. The GPRA Assessment Working Group was composed of members of the ACD, the NIH's Council of Public Representatives (COPR), and selected members of the Institute and Center (IC) national advisory councils.

The assessment was based on data provided by the Institutes and Centers that describes the new findings and theories forthcoming from the research that NIH conducts and supports. Key references were provided for all science advances, science capsules, and stories of discovery. NIH also provided copies of full articles to the Assessment Working Group whenever requested. (For a further discussion of this assessment process and the members of the Working Group, see Appendix 7).

While the Assessment Working Group drew on narrative information as the source of data for determining performance, the adequacy of performance was judged based on specific assessment criteria developed by the Working Group. Specific criteria were developed for each descriptive goal. For example, for the goal to add to the body of knowledge about normal and abnormal biological functions, the criteria were as follows:

- The NIH biomedical research enterprise *has successfully met this goal* when its research yields new findings related to biological functions and behavior, and the new findings are published and/or disseminated.
- The NIH biomedical research enterprise *has substantially exceeded this goal* when, in addition to fulfilling the criteria under *successfully met*, any of the following applies:
  - Discoveries result in significant new understanding of a particular biologic or behavioral process. Such new understanding may open up new avenues of research or be applicable to other disciplines, other areas of research, or other diseases.
  - Research yields answers to long-standing, important biological and behavioral questions, or provides novel investigative approaches for addressing such questions.
  - Genomic information about humans, model organisms, and/or disease-causing agent is translated into new understanding of the role of genes and/or environment in human health, disease, and behavior.
  - Discoveries have potential for translation into new or improved technologies, diagnostics, treatments, and preventive strategies.
- The NIH biomedical research enterprise *has not met this goal* when its research fails to yield new findings related biological functions and behavior, or when new findings are not published and/or disseminated.

Another example of criteria is those the Assessment Working Group developed for the goal to develop new or improved instruments and technologies for use in research and medicine:

- The NIH biomedical research enterprise *has successfully met this goal* when its research yields new or improved instruments and technologies for use in research and medicine, and the instruments and technologies are published and/or disseminated or made available to appropriate populations.
- The NIH biomedical research enterprise *has substantially exceeded this goal* when, in addition to fulfilling the criteria under *successfully met*, any of the following applies:
  - Instruments and technologies improve quality of life. This includes new or improved ways to ameliorate/manage symptoms, relieve suffering, and restore/increase physical function/activity.
  - Technical barriers are overcome so that investigations that were previously impossible are now possible.
  - Instruments and technologies enable novel approaches to answering important biological and behavioral questions.
  - Instruments and technologies are applicable to other disciplines, areas of research, or diseases.
  - New/improved methods for generating, organizing, and disseminating genomic and other biological and behavioral information are developed.
- The NIH biomedical research enterprise *has not met this goal* when its research fails to yield new findings related biological functions and behavior, or when new findings are not published and/or disseminated.

Because research outcome goals a–e are being replaced, for FY 2002, OMB excused NIH from reporting on the soon-to-be obsolete goals. Accordingly, NIH did not arrange for conduct of an independent assessment of the performance it reported. Nonetheless, as required under GPRA,

NIH has assembled reporting materials that can be assessed and thus is in compliance with GPRA reporting requirements (see http://www1.od.nih.gov/osp/ospp/gpra/gpra_nih.htm).

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

Both finished and draft sequence data for human and mouse genomes are deposited into the public database, GenBank. Totals and weekly production rates for the human genome are reported on an NIH Website. Since September 1, 1999 sequence information submitted to GenBank by the major participants in the Human Genome Project has included quantitative, 'per nucleotide' quality estimates provided by appropriate analytical software; this quality information is also available publicly. Independent assessment of the quality of the sequence data produced under NHGRI funding will be done by a quality assessment process. Evaluations will be publicly available through a Web site and publication.

Raw and assembly data for the human, mouse, and rat genomes are available at the following links

Genome	Assembly	Raw Data
Human	http://genome.cse.ucsc.edu/cgi-bin/hgGateway?org=human	http://www.ncbi.nlm.nih.gov/genome/seq/weekly_report.html
Mouse	http://www.ensembl.org/Mus_musculus/	http://www.ncbi.nlm.nih.gov/Traces/trace.cgi?
Rat	http://hgsc.bcm.tmc.edu/projects/rat/	http://www.ncbi.nlm.nih.gov/Traces/trace.cgi?

The NIH is still in the process of developing the partnerships that will be necessary in order to establish and fund the Haplotype map project and meet the proposed timetable. The timing and achievement of the present haplotype goals and targets are thus contingent on these partnerships being solidified and may be adjusted accordingly.

Single nucleotide polymorphisms (SNPs) are deposited into a public database, dbSNP, and totals are reported on an NIH web site: http://www.ncbi.nlm.nih.gov/SNP/index.html

Sequence data for the full-length cDNA clones will be deposited into GenBank and identified as part of the Mammalian Gene Collection (http://mgc.nci.nih.gov/). cDNA Clones will be available to researchers from a central repository.

The sequences of disease-causing microorganisms are deposited in public databases that have Intranet sites and are published. Citations are available for all publications. In addition to sequence data that are deposited in GenBank, http://www.ncbi.nlm.nih.gov/GenBanfk/, depositions frequently are made to other public databases, including:

Databases for Completed Sequencing Projects

- *Anopheles gambiae* – http://www.niaid.nih.gov/dmid/genomes/anopheles.htm
- *Bacillus anthracis* (Ames) – http://www.tigr.org/tdb/
- *Bacillus cereus* (10987) – http://www.tigr.org/tdb/mdb/mdbinprogress.html
- *Brucella suis* (1330) – http://www.tigr.org/tdb/mdb/mdbinprogress.html
- *Chlamydia pneumoniae* (AR39) – http://www.tigr.org/tdb/mdbcomplete.html

- *Chlamydia trachomatis* (mouse pneumonitis) – http://www.tigr.org/tdb/mdb/mdbcomplete.html
- *Chlamydia trachomatis* (serovar D) – http://www.stdgen.lanl.gov/
- *Coxiella burnetti* (Nine Mile phase I RSA 493) – http://www.tigr.org/tdb/mdb/mdbinprogress.html
- *Enterococcus faecalis* (V583) – http://wwwtigr.org/tdb/mdb/mdbinprogress.html
- *Escherichia coli* (O157:H7) – http://www.genome.wisc.edu
- *Escherichia coli* (CFT073) – http://www.genome.wisc.edu
- *Haemophilus ducreyi* (35000HP) – http://www.microbial-pathogenesis.org
- *Klebsiella pneumoniae* (MGH 78578) – http://genome.wustl.edu/gsc/Projects/bacteria.shtml
- *Leishmania major* (Friedlin Chromosome 1) – http://genedb.org/genedb/leish/index.jsp
- *Mycobacterium avium* (104) – http://www.tigr.org/tdb/mdb/mdbinprogress.html
- *Mycobacterium tuberculosis* (CSU 93) - http://www.tigr.org/tdb/mdb/mdbinprogress.html
- *Neisseria gonorrhoeae* (FA1090) – http://www.genome.ou.edu/gono.html
- *Plasmodium falciparum* (3D7) – http://PlasmoDB.org
- *Salmonella paratyphi A* (ATCC 9150) – http://genome.wustl.edu/projects/bacteria.shtml
- *Salmonella typhimurium* (LT2) – http://genome.wustl.edu/projects/bacterial/styphimurium
- *Shigella flexneri* (Serotype 2a) – http://www.genome.wisc.edu
- *Staphylococcus aureus* (COL) – http://www.tigr.org/tdb/mdb/mdbinprogress.html
- *Staphylococcus aureus* (8325) – http://www.genome.ou.edu/staph.html
- *Staphylococcus epidermidis* (RP62A) – http://www.tigr.org/tdb.mdb/mdb/inprogress.html
- *Streptococcus pneumoniae* (Serotype 4) – http://genome.microbio.uab.edu//strep/
- *Streptococcus pyogenes* (M1 GAS) – http://www.genome.ou.edu/strep.html
- *Treponema pallidum* (Nichols) – http://www.tigr.org/tdb/mdb/mdbcomplete.html; http://www.stdgen.lanl.gov
- *Ureaplasma urealyticum* (Serovar 3) – http://genome.microbio.uab.edu/uu/uugen.htm; http://stdgen.lanl.gov
- *Vibrio cholerae* (N16961 Serotype 01, biotype El Tor) – http://www.tigr.org/tdb/mdb
- *Wolbachia spp.* – http://www.tigr.org/cgi-bin/BlastSearch/blast.cgi
- *Yersinia pestis* – http://www.genome.wisc.edu

Databases for Ongoing Sequencing Projects

- *Anopheles gambiae* – http://konops.imbb.forth.gr/AnoDB
- *Aspergillus fumigatus* (Af293) – http://www.aspergillus.man.ac.uk
- *Brugia malayi* (NIAID/TRS) – http://www.tigr.org/tdb/e2k1/bma1
- *Burkholderia mallei* (ATCC 23344) – http://www.tigr.org/tdb/mdbinprogress.html
- *Clostridium perfringens* (ATCC 13124) – http://www.tigr.org/tdb/mdb/mdbinprogress.html
- *Coccidioides posadasii* (C735) – http://www.tigr.org/tigr-scripts/ufmg/ReleaseDate.pl
- *Cryptococcus neoformans* (JEC21 serotype D)) – http://www.sequence.stanford.edu/group/C.neoformans/index.html
- *Cryptococcus neoformans* (JEC 21 serotype D) – http://www.tigr.org/tdb/e2k1/cna1/
- *Cryptosporidium parvum* (Human isolate) – http://www.parvum.mic.vcu.edu
- *Cryptosporidium parvum* (Iowa) – http://www.cbc.umn.edu/ResearchProjects/AGAC/Cp/index.html

- *Ehrlichia spp*. – http://riki-lb1.vet.ohio-state.edu/ehrlichia
- *Entamoeba histolytica* (HM1:IMSS) – http://www.tigr.org/tdb/e2k1/eha1
- *Escherichia* coli (K1 RS218 meningitis strain) – http:///www.genome.wisc.edu
- *Giardia lamblia* (WB5 chromosomes) – http://hermes.mbl.edu/baypaul/Giardia-HTML/index2.html
- *Legionella pneumophila* (Philadelphia 1) – http://genome3.cpmc.columbia.edu/~legion/
- *Leishmania major* (Friedlin) – http://genedb.org/genedb/leish/index.jsp
- *Mycobacterium smegmatis* (MC2155) – http://www.tigr.org/tdb/mdb/mdbinprogress.html
- Nematode EST project – http://www.nematode.net
- *Pneumocystis carinii* (Rat and human isolates) – http://pneumocystis.uc.edu
- *Rickettsia typhi* (Wilmington) – http://www.hgsc.bcm.tmc.edu
- *Salmonella typhi* (TY2) – http://www.genome.wisc.edu
- *Schistosoma mansoni* (Puerto Rican Strain) – http://www.tigr.org/tdb/e2k1/sma1
- *Streptococcus pneumoniae* (serotype 6) – http://genome.microbio.uab.edu//strep/
- *Toxoplasma gondii* (ME49) - http://toxodb.org
- *Trypanosoma brucei* (TREU 92714) – http://www.tigr.org/tdb/e2k1/tba1
- http://www.genedb.org/genedb/tryp/index.jsp
- *Trypanosoma cruzi* (CL Brenner) – http://www.tigr.org/tdb/e2k1/tca1

Other NIH-funded genome sequence databases include the Poxvirus Bioinformatics Resource Center (http://www.poxvirus.org) and the Sexually Transmitted Diseases Database (http://www.stdgen.lanl.gov). NIH also funds sequencing of microbes associated with oral disease (http://www.nidr.nih.gov/research/dbts/microbial_lrg_scale_DNA_prjs.asp).

Access to new technologies will be documented through electronic archives of initiatives, e.g., archives of the NIH Guide, and databases including the NIH Computer Retrieval of Information on Scientific Projects (CRISP) that contain objective data on grant and contract awards.

The citation for the tuberculosis genome paper is: R.D. Fleishmann *et. Al.* "Whole Genome Comparison of *Mycobacterium tuberculosis* Clinical and Laboratory Strains," *Journal of Bacteriology*, Oct 2002, Vol.184 5479-5490. The citations for the malaria parasite and vector papers are M. J. Gardner *et al*. "Genome sequence of the human malaria parasite *Plasmodium falciparum," Nature* 419: 498-511(2002) and RA Holt *et al*. "The genome sequence of the malaria mosquito *Anopheles gambiae," Science* 297: 129-49 (2002).

Goal g) *Develop an HIV/AIDS vaccine by 2007.*

Précis of science advances will be used to document progress in vaccine strategies, delivery/production technologies, and animal models. The progress in collaborating with scientists in developing countries and with industry will be inferred on the basis of activities to promote such interaction. NIH and NIAID databases provide documentation of these activities. Information from the Statistical Center for HIV/AIDS Research and Prevention (SCHARP) at the Fred Hutchinson Cancer Research Center, Seattle, Washington will be used to compile a record of the completion of ongoing vaccine trials and the initiation of new trials.

Citations will be available for all science advances considered in the assessment process. NIH and NIAID databases, including Computer Retrieval of Information on Scientific Projects (CRISP) and Scientific Information Management System (SIMS), which are used to track research awards and initiatives, and the contract archives contain objective data on grant and contract awards and on the establishment of initiatives. The electronic archive of the NIH Guide also contains objective data on establishment of initiatives. The SCHARP database on the status of NIH HIV/AIDS vaccine trials maintains data from each of the clinical trial sites.

Communication of Results

Goal a) *Increase awareness of NIH-sponsored research among health care providers to promote research application.*

Performance was measured by the successful completion of planned evaluation and dissemination activities and development of model case studies and other training programs for health care providers. NIH uses several means to verify performance data, including statistical reports showing the number of health professionals who have received information materials from NIH, the number of completed and graded tests for CME Category I credit, and analyses of NIH website usage, including downloads of specific NIH tools.

Continuing Medical Education (CME) is part of NHLBI's Asthma Management Model System, a special professional education section on NHLBI's site. Many more physicians have visited the CME site, accessed the course, and possibly registered for Category II credit. However, this use cannot be tracked since Category II does not require the user to return a test for grading.

Goal b) *Increase awareness of NIH-sponsored research results among racial/ethnic minorities and high risk, underserved, and/or affected publics***.**

Performance was measured by the successful development of specific health education materials and the development and implementation of planned communication campaigns. NIH uses several means to verify performance data, including statistical reports showing the number of public service announcements (PSA) distributed to and broadcast by television stations, cable stations, and cable systems. Other data sources include reports regarding newspaper placements, NIH website usage, and inquiries to different NIH information clearinghouses.

Goal c) *Increase awareness of NIH-sponsored research results among the general public***.**

Performance was measured by the successful implementation of improved NIH operations, evidence of stronger collaboration with other organizations, and the development and implementation of planned communication campaigns. NIH uses several means to verify performance data and estimate the reach of communications campaigns by analyzing website usage and the number of media requests for interviews, hotline calls, media impressions, and radio and television stations airing campaign messages.

Goal d) *Increase awareness of clinical research and support participation in clinical trials.*

Performance was measured by evidence of stronger collaborations with other organizations, the development of web-based tools and other materials, and the number of inquiries about NIH-sponsored intramural clinical trials and their availability. NIH uses several means to verify performance data, such as statistical reports on NIH website usage, including downloads of specific NIH tools; the percent of minorities and other underrepresented groups enrolled in NIH-sponsored clinical trials; and the number of telephone inquiries about participating in clinical trials.

PDQ is a database that contains the world's most comprehensive list of cancer clinical trials (more than 1,800 open to patient accrual as of 2001) and is used to report and exchange clinical trials information.

Goal e) *Establish a Clinical Trials Database, as required by the FDA Modernization Act.*

Performance was measured by successful completion of the planned implementation study, the number of clinical trials in the database sponsored by non-NIH organizations, and evidence of effective promotion of the database. NIH uses several means to verify performance data, including statistical reports analyzing the number of hits on the Clinical Trials Database website (ClinicalTrials.gov).

Technology Transfer

Goal a) *Increase the number of scientists who have received training in technology transfer.*

Prior to FY 2002, performance was measured by the number and percent of NIH scientists who attended seminars/retreats focused on technology transfer. NIH used participation information collected from seminar attendance sheets to verify these performance data. Post FY 2002, performance was measured by electronically monitoring the number of scientists who completed the online training module. Documentation of the fact that training is mandatory for all Principal Investigators, Fellows, Graduate Students, and Staff Scientists/Staff Clinicians can be found at http://137.187.206.145/cbttng_techxfer/cbts/tesweb/login.asp. Performance data can be verified by contacting the training monitor for the NIH On-Line Technology Transfer Training module http://tttraining.od.nih.gov/.

Goal b) *Develop a system to identify and measure the health outcomes of technologies licensed by NIH.*

Performance is measured by completion of the recommendations on potential outcome measures and data sources, by completion of the pilot-test, and by application of the methodology to a sample of licensed technologies. Performance data can be verified through records maintained by the NIH Office of Technology Transfer (OTT). The 2 pilot studies are available on the OTT website at http://ott.od.nih.gov/NewPages/techdev.pdf.

Goal c) *Maintain oversight and protection of the public investment in NIH research through increased monitoring of licensee activities.*

Performance is measured by the number of licensees audited, by the number and percent reduction in delinquent royalty payments and terminated licenses, and by the implementation of a process for pursuing litigation against those who infringe on NIH intellectual property rights. NIH uses several means to verify performance data, including records maintained by the NIH Office of Technology Transfer regarding licensee audits, delinquent payments, terminated licenses with outstanding balances, and court actions. During FY 2002 the Office of Technology Transfer (OTT) established a new system, called TechTracS, to improve tracking of activities associated with patenting licensing and royalties billing and collection activities. A module in TechTracS for monitoring benchmarks and milestones is being designed.

Grants Administration and Peer Review

Goal a) *Improve electronic Research Administration (eRA) technology by developing capability for end-to-end electronic research administration by 2004.*

Performance will be measured by the extent to which information can be submitted to NIH through the NIH Commons system and by the number of FDP institutions registered, R01 applications scanned, and progress reports and R01 applications received each year. Performance data will be verified through eRA status reports posted on the OER website. Deployment schedules, announcements, and eRA project milestones for all electronic research administration systems are available at era.nih.gov.

Goal b) *Ensure proper stewardship of public funding for research.*

Performance will be measured by the establishment of the Division of Grants Compliance and Oversight in NIH Manual Chapter 1123 by the NIH Office of Management Assessment (OMA). The number of proactive compliance site visits can be verified through the site visit schedule and the *Proactive Compliance Site Visits 2000: A Compendium of Findings and Observations,* both of which are posted on the Office of Extramural Research (OER) website at http://grants.nih.gov/grants/oer.htm.

Goal c) *Ensure that the NIH peer review process keeps pace with current advances in research and that the expertise of peer reviewers is appropriate for the needs of modern science.*

Performance was measured by the number of Steering Committees, SSB Teams, and external IRG Advisory Groups in operation at the end of each fiscal year, and by completion of modifications to specific IRGs and their study sections. Performance data can be verified through postings on the CSR website describing progress in implementing Phase 2 and dissemination for public comment of the proposed IRG and study section modifications (www.csr.nih.gov/events/updatephase2.htm). Information regarding SSB teams is available at www.csr.nih.gov/review/reorgact.asp. A summary of IRG Advisory Group activities and comments are available at www.csr.nih.gov/EVENTS/IRG_WG_Summary.htm. Approved minutes of the January 2002 CSR Advisory Committee meeting are available at www.csr.nih.gov/drgac/jan02min.doc.

Goal d) *Develop innovative business practices to facilitate government/public interactions.*

Performance was measured by the percent of Type 1 awards expedited, the early notification of Type 2 awards, and the number of months it took the most meritorious grant applicants to receive funding or a notice of award. Performance data can be verified through comparisons of grant application data in the IMPAC database system with accounting data maintained by the NIH Office of Financial Management.

By employing expedited en bloc Council concurrence, NIH has found that certain factors limit the expediting of awards: (1) applicants may decline the offer of an early Type 1 award to ensure that additional staff are hired and in place, and appropriate collaborations are fully established; (2) Type 2 applications are tied to an existing end date, so that the start date for making the Type 2 award is fixed; and (3) the Federal budget process, including the practice of using Continuing Resolutions, prohibits the NIH from using expedited procedures for a least one of the three Council rounds (October). In some years, the budget process may also affect the January round.

One-third of the Institutes use their websites to inform the community of the percentile they will assure funding for research project grants. Consequently, as soon as applicants receive their scores from peer review (approximately 4-5 months after application submission), they know they will be funded if they are within that percentile payline. For these Institutes, this percentile payline is usually above the 20th percentile, thus successfully extending the definition of "most meritorious awards" past the originally anticipated 15th percentile.

Status of the streamlining of the competing and non-competing application requirements, including the status of SNAP, may be found on the NIH Office of Extramural Research website at https://commons.era.nih.gov/commons/index.jsp.

Goal e) *Improve grantee reporting of inventions developed with Federal funds.*

Performance data for the target on educating constituents can be verified on the NIH grants compliance website at http://grants.nih.gov/grants/compliance. Schedule and findings for NIH proactive compliance site visits are discussed

The status of Edison is documented on the website for Interagency Edison http://www.iedison.gov/. Performance data for the target on improvement of quality of historical invention reporting data can be retrieved from the Edison database.

Management and Administrative Support

Goal a) *Improve the efficiency of the simplified acquisition process by continuing to expand the Purchase Card Program.*

When this goal was initiated, NIH decided to measure performance by the annual dollar volume of orders, the total number of staff trained to use the purchase cards, and the total number of purchase card holders. NIH used several means to verify the performance data, including

Automated Data Base reports on purchase card transactions and records maintained by the NIH Office of Administration on the number of individuals trained and issued purchase cards.

Because the number of purchase cardholders has increased dramatically since the inception of this goal, future increases in the number of card holders may not be as significant in the future as it was in the past five years. Furthermore, it is difficult to estimate future requests for purchase cards. A request for a purchase card is at the discretion of each IC. Some IC's have internal directives on the usage of the purchase card.

At this point in the Purchase Card Program, the most meaningful measures of success focus on the annual dollar amount of orders and the annual number of orders/transactions generated. Performance data for these measures could be verified through Automated Data Base reports on purchase card transactions and dollar volume. Reports are on file in the Division of Acquisition Programs (Building 6011, Room 547B).

***Goal b**) Expand the use of Performance Based Contracting (PBC).*

Performance was measured by the amount of contract dollars awarded. Performance data was verified through reports obtained from the Departmental Contracts Information System (DCIS).

***Goal c**) Identify and pilot new approaches to providing human resource services which increase manager satisfaction with personnel system flexibility and ease of use.*

Performance was measured by the completion of National Academy of Public Administration (NAPA) 2001 survey of NIH managers, the analysis of the data gathered, and the issuance of the final report in 2002. Performance data can be verified through the survey and interview data collected in the 2001 survey (maintained by NAPA) and the final report, "Results of Research Conducted to Ascertain Personnel Delegations for Supervisors and Managers at the National Institutes of Health," NAPA, December 2001.

***Goal d**) Recruit and retain highly qualified extramural investigators to biomedical/behavioral research through the use of student loan repayment programs.*

Performance with respect to recruitment of researchers will be measured by the percent of applicants who are early in their research careers, the percent of awardees that are also first time recipients of an NIH grant or other award, the percent of eligible sites using the programs for recruitment, the percent of applicants seeking the loan repayment programs, and the percent of awardees from specific populations. Performance with respect to retention of researchers will be measured by the percent of awardees that receive additional training and are encouraged to apply for small grant support, and the percent of participants retained in specific fields of study. The data will be drawn from the application and review processes. Performance data will be verified through reports from the Information for Management, Planning, Analysis, and Coordination (IMPAC) system and through reconciliation records maintained by the loan repayment program area.

Goal e) *Implement government-wide initiative on delayering management levels and streamlining organization.*

Performance data will be verified through organizational charts showing management layers before implementation, compared with organizational charts showing management layers after streamlining.

Research Training and Career Development Program

Training Support and Outreach

Goal a) *Respond to the National Academy of Sciences quadrennial report on the future needs for health-related researchers.*

Performance was demonstrated by NIH's submission to Congress of its formal response to the NAS quadrennial report and the development of GPRA performance targets for four of the most challenging NAS recommendations. Verification of performance is available through public documents, including the online posting of NIH's formal response to the NAS quadrennial report (http://grants.nih.gov/training/nas_report/NIHResponse.htm).

National Research Service Award (NRSA) stipend increases were published in the January 25, 2002 issue of the NIH Guide (http://grants1.nih.gov/grants/guide/index.html).

Goal b) *Maintain adequate application and award rates (success rates) in key training support areas.*

Performance was measured by the annual application and success rates for T32, F32, K01, and K08 awards and by assessments of the information needs of potential applicants. Performance data were verified through reports generated from the IMPAC database system and information obtained using WebTrends site analysis software.

Goal c) *Increase the pool of clinician researchers trained to conduct patient-oriented research.*

Performance was measured by the number of annual K23 and K24 applications and awards. Performance data were verified through reports generated from the IMPAC database system.

Goal d) *Increase the participation of underrepresented racial and ethnic groups, women, and individuals with disabilities in NIH training and research programs.*

Performance was measured by tracking the participation of individuals of different races and ethnicities in NIH research and training programs. However, because only 75-85 percent of applicants generally respond to questions about their race and ethnicity, accurate measures of participation are difficult to obtain. NIH is working to improve the response rates by redesigning grant and fellowship application forms and providing applicants with better explanations of why they should complete this voluntary portion of the application. The quality of these data is key to the ability of NIH to accurately monitor and report on diversity. NIH uses several means to

verify performance data, including reports on the year-end status of new procedures for identifying race/ethnicity, records maintained by the Research Training and Career Development Program, and reports generated from the IMPAC database system. Data collection for a given fiscal year is usually only completed one to two years later.

Goal e) *Expand capabilities for electronic administration of research training and career development activities.*

Performance will be measured by the number of appointment forms and termination notices received electronically and by the year-end status of evaluation plans for NIH research training programs. All electronic appointment forms received via X-Train will be used to establish a professional profile that NIH will use for tracking career outcomes. A temporary database will hold each electronic form received until the NIH official responsible for the training grant has reviewed and approved its contents, after which it will be added to the IMPAC database system. Performance data will be verified through records maintained by the Research Training and Career Development Program and through reports generated from the IMPAC system.

Goal f) *Improve the capabilities for career outcome tracking for NIH training and career development programs.*

Performance was measured by the year-end status of current and planned evaluations and reports of NIH research training programs and by the year-end status of the X-Train system. Verification of performance is available through records maintained by the Research Training and Career Development Program and through reports generated from the IMPAC database system.

Research Facilities Program

Intramural Modernization and Improvements

Goal a) *Improve the operating conditions and environment of intramural facilities and the availability and reliability of NIH utility distribution systems to support intramural research***.**

Performance was measured using the number and percentage of completed projects at the end of the fiscal year. Project and milestone accomplishments were validated using data maintained by the Chief, Resources Management Section, Division of Engineering Services, Public Works Branch until the newly created Facility Assessment Database (FAB) is fully operational. This new comprehensive system will quantify, track and monitor project activities applicable to this goal. The database for the NIH Bethesda Campus is scheduled to come on line first in the spring of 2003 exclusive of the Warren Grant Magnuson Clinical Center (Building 10), which is slated for major renovations. The database for the remainder of the NIH will be on line by the summer of 2003.

Goal b) *Complete the Mark O. Hatfield Clinical Research Center.*

Performance was measured by the completion of the design work, superstructure, and exterior wall system for the CRC, and the percent of construction completed. Process and milestone data were obtained from the CMMS and PIN systems. Performance data were verified through quality control systems that track, monitor, verify, and validate project goals consistent with internationally recognized standards of operations. Performance was measured as a percentage of the scheduled duration of the construction. Performance data was verified using monthly construction progress reports that are maintained by the Project Officer.

Goal c) *Complete the Warren Grant Magnuson Clinical Center Revitalization Program.*

Performance was measured as a function of the design continuing and/or starting for the interim renovation phases and Phase I of this project respectively. Performance data was verified using project tracking and monitoring systems that have been established for this project.

Goal d) *Maintain the quality of the NIH Animal Care Program and construct a Central Vivarium.*

Performance was measured by the receipt of AAALAC accreditation, initiation of the design for the Central Vivarium/ARC, and awarding of contracts. Process and milestone data were obtained from the CMMS and PIN systems. Performance data were verified through quality control systems that track, monitor, verify, and validate project goals consistent with internationally recognized standards of operations. The need to stop performance is documented in the annual Buildings and Facilities (B&F) budgeting plan, which identifies facility requirements and a funding strategy to support them. The plan is part of the Strategic Facilities Planning process that considers the constraints of NIH existing capital assets, the recommendations of the NIH Facilities Planning Advisory Committee, NIH research priorities and DHHS guidance.

Goal e) *Complete the John Edward Porter Neuroscience Research Center on the NIH Bethesda campus.*

Performance under this goal was measured and data verifiable and available from the ProLog and Primavera Project Management System being used in the management of this project. Project status information is also available via the NIH intranet that follows http://des.od.nih.gov/eWeb/construction/building35/progress.htm.

Goal f) *Establish a systematic process to manage and account for NIH's Real Property Inventory.*

Performance was measured by the system assessment input from the stakeholders assigned to conduct the pilot test of the Foundation Information for Real Property Management (FIRM). Output from the FIRM database, in the form of management reports, documents the database was populated. The GSA decision to discontinue support of FIRM is documented in a July 26, 2002 letter from Mr. David Bibb, Deputy Associate Administrator, Office of Real Property,

GSA, which is on file in the NIH Office of Research Services. The decision also is documented at the following web site: http://www.gsa.gov/Portal/content/offerings_content.jsp?contentOID=114355&contentType=1004&PMPR=1&S505=1.

Goal g) *Enhance NIH's ability to deter and respond to security threats by implementing campus and facility management initiatives.*

Performance for design and construction projects will be measured by evidence that the project teams have been assembled and design and construction awards are made. Process and milestone data will be obtained from contracting and project management systems. Performance data will be verified through quality control systems that track, monitor, verify and validate the project goals with internationally recognized standards of operations.

There is an ORS committee that has general oversight for developing, monitoring, and reviewing security classification standards and policies. Also, a database of security classification data has been developed and is reviewed by the Office of the Deputy Chief Security Officer. A list of committee members and confidential meeting minutes are on file in the ORS Division of Public Safety.

Goal h) *Provide research facilities to support biodefense by implementing design and construction actions.*

Performance for each design and construction project will be measured by evidence that the project teams have been assembled and design and construction awards are made. Process and milestone data will be obtained from contracting and project management systems. Performance data will be verified through quality control systems that track, monitor, verify and validate the project goals with internationally recognized standards of operations.

Goal i) *Increase incorporation of feasible, cost effective, environmental sustainability strategies into NIH planning, development, and operations by empowering a senior management team accountable for the management and implementation of the Strategic Plan for Environmental Sustainability (SPES).*

An Environmental Management Progress plan will be developed to track, monitor, verify, and validate project goals consistent with recognized standards of operations. Performance will be based on increases to energy efficiencies and percent development of an environmental management system that incorporates initial review of planning, design and construction and operating features.

Extramural Assistance

Goal a) *Approve an optimal percent of construction designs by the end of the third year that are in compliance with Federal and NIH design regulations and guidelines, and with other relevant local, national, and international codes and standards.*

Performance will be measured by the issuance of approval letters by the engineers who review the construction designs. Performance data will be verified through these letters, which are maintained as part of the formal record of the official grant files.

Appendix 5

Performance Measurement Linkages with Budget, Cost Accounting, Information Technology Planning, Capital Planning, and Program Evaluation

Budget

NIH links performance measures to budget and accounting, as appropriate, to meet the requirements of GPRA and other management reporting such as under the Chief Financial Officers (CFO) Act and the Government Management and Reform Act (GMRA).

NIH organizes its activities into three core programs for purposes of planning and performance assessment under GPRA (see Section 1.2). Those programs are Research, Research Training and Career Development, and Research Facilities. NIH's GPRA goals are arrayed under these programs. Under NIH's current budget and cost accounting system, dollars are not directly associated to each goal (such association is not required under GPRA). However, NIH has developed a "crosswalk" for how each budget mechanism (e.g., Research Project Grant, Research Management and Support, Construction, etc.) links to the core programs. In this way, NIH distributes its total budget authority by GPRA Core Program.

Cost Accounting

NIH develops and reports the cost of its three programs on its audited Statement of Net Costs, as required by the CFO Act, the GMRA, and the Office of Management and Budget. These reported costs are derived using an accrual basis of accounting as required by federal accounting standards and the Federal Financial Management Improvement Act. These amounts differ from the reported obligations or budgetary resources included in budget documents that use an obligation basis of accounting.

NIH includes cost measures for performance goals, as appropriate, in its service and supply fund activities. NIH finances these activities using a fee for service cost recovery method. NIH develops cost per unit of good or service and benchmarks these unit costs with other providers of similar or complementary goods and services. Also, NIH strives to increase stakeholder value by reducing the cost per unit of good or service wherever possible.

Information Technology Planning

Information Technology (IT) had a discrete set of goals in the NIH GPRA Performance Plan through 2000. These goals focused the IT activities of the agency on the NIH mission and institutionalized a corporate-wide perspective in the management of the IT function. (A summary of the accomplishment of these goals follows.) In addition, IT has been woven throughout the NIH research program and linked to goals identified in all three of the core

programs in the NIH Annual Performance Plan. Although, the IT-specific goals have been accomplished and dropped from this Plan, the performance goals that reflect how IT is utilized by the NIH research community will continue to be found in all three of the core Programs. For example, the electronic research administration goals under the Research Program and under the Research Training and Career Development Program and the real property inventory goal under the Research Facilities Program.

The accomplishment of the IT-specific goals began in 1996, when the NIH Director began activities for managing selected elements of IT from a corporate-wide perspective. His first step addressed the organizational structure by hiring a Chief Information Officer (CIO) and the second established the Center for Information Technology (CIT). In addition, two advisory groups were established: the NIH Director formed NIH's IT Board of Governors (BOG), composed of selected senior management from across NIH, and the NIH CIO established the NIH IT Management Committee (ITMC), composed of senior Institute and Center (IC) IT representatives.

Since then, the CIO and its advisory groups have developed a process for managing IT from a corporate-wide perspective to make it more effective in supporting the mission of NIH and in providing integrated systems that support the variety of NIH business processes. They accomplished the following:

- Strengthened the investment review process
- Established a formal project management structure for enterprise IT
- Refined and implemented the strategic, corporate "IT vision" for NIH
- Developed a NIH-wide information security program
- Developed interoperability standards.

In addition, guidance was developed to assist the ICs in establishing performance measures and evaluating IT programs based on performance measures, (which can be found at http://www.cit.nih.gov/mgmt-pol.html). Discussions of performance measures were woven throughout the Investment Review process described at http://irm.cit.nih.gov/itmra/invreview.html and were also incorporated in the IT Management Guide, http://irm.cit.nih.gov/itmra/mgtprocess.html. Now, when IC program managers conduct a business case analysis, they are advised to address IT performance measures among others. Resources and tools were made available to facilitate this process and can be found at http://irm.cit.nih.gov/itmra/perfmeasure.html. In addition, the Office of the CIO initiated a recurring class in performance measures, to increase the number of IT and program managers familiar with the creation and use of performance measures.

Having set these organizations, processes, guidelines and tools in place, NIH has focused its Information Technology planning on pursuing the mission of the NIH as described in this Plan. This accomplishment has also enhanced our ability to accomplish the IT-related goals within our core programs in conformance with the performance measurement principles of GPRA.

Capital Planning

NIH's planning for capital projects is woven throughout the annual performance plan, notably in the functional area of "Intramural Modernization and Improvements." Additional information on capital projects can be found in the detailed budget tables prepared by NIH's Office of Financial Management.

Evaluation

Evaluation is the foundation of managing for results. Inevitably, program managers and other decision-makers gather information about a program and make judgments about its worth or value. The quality of those judgments depends on the quality of the information upon which they are based. For that reason, NIH program managers depend on two complementary evaluation activities, *performance measurement* and *program evaluation*, to establish reasonable performance goals and to accurately assess progress toward those goals.

Performance measurement refers to regular monitoring of program accomplishments. Program accomplishments include the activities conducted (process), products produced or services delivered (outputs), and the results of those products and services (outcomes). Performance measurement is conducted by program managers to gauge how well the program is progressing toward its intended goals. The information gained from such on-going tracking systems may alert program managers to emerging problems and may spur a program evaluation to provide more information on why the program is not achieving anticipated results.

Program evaluation refers to systematic investigations or studies that involve assessing the worth and/or performance of particular programs. In most cases, the underlying purpose of a program evaluation is to help program mangers answer specific questions about a program, such as whether it is being implemented as planned or is achieving its intended purpose. Managers typically use the information obtained from program evaluations to understand why certain results are or are not being achieved and to make adjustments in program strategies or activities. The four types of program evaluations conducted by NIH are needs assessments, feasibility studies, process evaluations, and outcome evaluations. Needs assessments and feasibility studies are usually conducted as preliminary studies (e.g., to improve the design of a more complex process or outcome evaluation). Experts external to the program often conduct program evaluations, but program managers may also conduct them.

Purposes of Program Evaluation Under GPRA

At NIH, program evaluation serves two important purposes under GPRA: to support program planning and to support program performance assessment.

Support Program Planning. Program evaluations provide useful information to NIH's program managers regarding the appropriateness of established performance goals, annual targets, and implementation strategies. For example, needs assessments are typically conducted to identify systematically whom a program is serving and the extent to which their needs are being addressed. They may also explain why certain needs are not being met and how the program

could be revised to address the unmet needs. Using the information gained from such evaluations as a foundation for program planning, NIH program managers develop and modify performance goals and targets to more effectively direct their programs toward the desired outcomes. In addition, the strategies used to implement NIH programs are often adjusted based on evaluation findings.

Support Program Performance Assessment. Program evaluations support program performance assessment activities at NIH primarily by providing insight regarding the relationship between NIH activities and the results NIH seeks to achieve. Outcome evaluations are often conducted to obtain methodologically sound information about the effectiveness of a program and to measure the program's progress towards goal achievement. In addition, this information is critical to determining the extent to which a program's activities contributed to any measured progress toward the desired end result or outcome.

NIH managers also use process evaluations to examine program progress (as evidenced primarily by program outputs) and to determine whether programs are being implemented as planned. The information gleaned from these evaluations allows managers to make mid-course corrections and improve program administration. Finally, feasibility studies are used to develop better ways to measure program performance. Examples include developing databases to track information over time, identifying ways to more effectively access existing data sources, developing new data collection instruments, and validating/verifying data sources.

Appendix 6

NIH Institutes and Centers

Institute/Center	Mission
National Cancer Institute	NCI conducts and supports programs to understand the causes of cancer; prevent, detect, diagnose, treat, and control cancer; and disseminate information to the practitioner, researcher, patient, and public. The Institute's efforts are directed at reducing the burden of cancer morbidity and mortality and, ultimately, at preventing the disease.
National Heart, Lung, and Blood Institute	NHLBI's research program is directed at diseases of the heart, blood vessels, lungs, and blood, and at transfusion medicine. Its activities encompass innovative basic, clinical, population-based, and health education research.
National Institute of Dental and Craniofacial Research	NIDCR's research program is directed at understanding, treating, and ultimately preventing the infectious and inherited craniofacial-oral-dental diseases and disorders that compromise millions of human lives.
National Institute of Diabetes and Digestive and Kidney Diseases	NIDDK conducts and supports research, training, health information dissemination, and other programs with respect to diabetes millitus and endocrine and metabolic diseases; digestive diseases and nutritional disorders; and kidney, urologic, and hematologic diseases.
National Institute of Neurological Disorders and Stroke	NINDS conducts and supports research and training on the normal and diseased nervous system to reduce the burden of neurological diseases. The research program is ultimately directed at improving the prevention, diagnosis, and treatment of the hundreds of disorders affecting the nervous system. These include stroke; epilepsy; demyelinating disorders such as multiple sclerosis; tumors; pain; traumatic injury of the brain and spinal cord; degenerative disorders such as Parkinson's disease; movement disorders; developmental disorders such as autism, the myasthenias and muscular dystrophies; and numerous autoimmune, metabolic, and genetic disorders.
National Institute of Allergy and Infectious Diseases	NIAID conducts and supports research that strives to understand, treat, and ultimately prevent the myriad infectious, immunologic, and allergic diseases that threaten millions of human lives.
National Institute of General Medical Sciences	NIGMS supports basic biomedical research that is not targeted to specific diseases, but increases understanding of life processes and lays the foundation for advances in disease diagnosis, treatment, and prevention. NIGMS attempts to ensure the vitality and continued productivity of basic biomedical research, while producing the next generation of scientific breakthroughs and training the next generation of scientists.

Institute/Center	Mission
National Institute of Child Health and Human Development	NICHD conducts and supports research on fertility, pregnancy, growth, development, and medical rehabilitation. The Institute's broad purpose is to ensure that every child is born healthy and wanted, and grows up free from disease and disability.
National Eye Institute	NEI conducts and supports research, training, health information dissemination, and other programs directed at blinding eye diseases, visual disorders, mechanisms of visual function, preservation of sight, and the special health problems and requirements of the blind.
National Institute of Environmental Health Sciences	NIEHS conducts and supports research on how environmental exposures, genetic susceptibility, and age interact to affect an individual's health. Its overall purpose is to reduce the burden of human illness and dysfunction from environmental causes.
National Institute on Aging	NIA conducts and supports research on the biomedical, social, and behavioral aspects of the aging process; the prevention of age-related diseases and disabilities; and the promotion of a better quality of life for all older Americans.
National Institute of Arthritis and Musculoskeletal and Skin Diseases	NIAMS conducts and supports research, training, and information dissemination directed at understanding the normal structure and function of bones, muscles, and skin, as well as the numerous and disparate diseases that affect these tissues.
National Institute on Deafness and Other Communication Disorders	NIDCD conducts and supports basic and clinical research and research training in the normal and disordered processes of hearing, balance, smell, taste, voice, speech, and language. These diseases and disorders currently affect about 46 million Americans. Basic and clinical research focused on understanding the normal processes and disorders of human communication are motivated both by intrinsic scientific interest and importance to the health of the nation.
National Institute of Mental Health	NIMH conducts and supports research on the brain and behavior – basic, clinical, epidemiological, and health services research. The Institute's activities are broadly dedicated to understanding, treating, and preventing mental illnesses.
National Institute on Drug Abuse	NIDA conducts and supports research across a broad range of disciplines that bear on drug abuse and addiction, and disseminates information about its research findings. The Institute's broad purpose is to help reduce drug abuse and to improve the options for addiction prevention and treatment.
National Institute on Alcohol Abuse and Alcoholism	NIAAA conducts research directed at improving the treatment and prevention of alcoholism and alcohol-related problems. The Institute's broad objective is to reduce the enormous health, social, and economic consequences of this disease.

Institute/Center	Mission
National Institute of Nursing Research	NINR has a broad mandate to sponsor research on the clinical care of individuals and their responses to health problems. Scientists supported by the Institute seek to understand and mitigate the effects of acute and chronic illness and disability, promote healthy behaviors and prevent the onset or worsening of disease, and improve the healthcare environment.
National Human Genome Research Institute	NHGRI supports NIH's participation in the Human Genome Project, a worldwide research effort directed at analyzing the structure of human DNA and determining the location of the estimated 100,000 human genes. At the intramural level, NHGRI develops technology for understanding, diagnosing, and treating genetic diseases.
National Institute of Biomedical Imaging and Bioengineering	The NIBIB promotes fundamental discoveries, design and development, and translation of technological capabilities in biomedical imaging and bioengineering, enabled by relevant areas of information science, physics, mathematics, materials science, and computer sciences. NIBIB plans, conducts, fosters, and supports an integrated and coordinated program of research and research training that can be applied to a broad spectrum of biological processes, disorders and diseases and across multiple organ systems.
National Center for Research Resources	NCRR advances biomedical research and improves human health through research projects and shared resources that create, develop, and provide a comprehensive range of human, animal, technological, and other resources. There are four main areas of concentration: biomedical technology, clinical research, comparative medicine, and research infrastructure.
National Center for Complementary and Alternative Medicine	NCCAM conducts and supports basic and applied research and training and disseminates information on complementary and alternative medicine to practitioners and the public.
National Center for Minority Health and Health Disparities	NCMHD serves as the focal point within the National Institutes of Health for planning and coordinating minority health and other health disparities research. The Center coordinates the development of a comprehensive health disparity research agenda that identifies and establishes priorities, budgets, and policy that govern the conduct and support of NIH-sponsored minority health and other health disparities research and training activities.
Fogarty International Center	FIC leads NIH's efforts to advance the health of the American public and citizens of all nations through international cooperation on global health threats.
Warren Grant Magnuson Clinical Center	CC is the clinical research facility of the NIH. It provides patient care, services, training, and the environment in which NIH clinician-scientists creatively translate emerging knowledge into better understanding, detection treatment, and prevention of human diseases.

Institute/Center	Mission
Center for Scientific Review	CSR carries out initial peer review of the majority of research and research training applications submitted to the NIH. Peer review is the foundation of the NIH grant and award process. The Center also serves as the central receipt point for all Public Health Service applications and makes referrals to scientific review groups for scientific and technical merit review and to funding components for potential award.
National Library of Medicine	NLM is one of three national medical libraries. It collects, organizes, and makes available biomedical science information to investigators, educators, and practitioners. It also carries out programs to strengthen medical library services in the United States. NLM's electronic databases, such as MEDLINE, are used extensively throughout the world.
Center for Information Technology	CIT provides, coordinates, and manages information technology and seeks to advance computational science.

Appendix 7

Approach to Performance Assessment

NIH's Annual Performance Plans include performance goals that can be assessed through objective/quantitative measures and performance goals based on descriptive achievement criteria.

The vast majority of NIH's performance goals are objective/quantitative. In these cases performance assessment is a process, principally, of comparing data on actual achievement with the target levels stated by the Annual Program Performance Plans.

Where such quantitative measures are not available or not useful, GPRA also provides a means for an agency to define performance goals that rely on qualitative criteria. Provision for this "Alternative Form" is in the Act (P.L. 103-62) at Sec. 1115 (b). The use of descriptive measures has been central to the approach taken with many of the outcomes goals for NIH's research activities.

Further details on these assessment approaches are discussed below.

Objective/Quantitative Performance Goals

As noted above, most of the performance goals in NIH's Annual Plans have objective/quantitative targets. For these goals, data submitted for the assessment process permits a comparison between the actual achievement level and that targeted by the performance goal. In many cases, the performance data are quantitative, drawn from one or more of NIH's databases that support the agency's normal management processes. Or, where the goal is to complete an action or reach an intermediate milestone, data are provided that objectively documents the status of the progress. (See Appendix 4 on Data Verification and Validation for further discussion of the data sources used for quantitative performance targets.)

In the FY 2002 Performance Report, NIH used the following codes in each performance goal chart for quantitative goals.

◆ **Target Met** – Indicates that NIH's actual performance met or surpassed the stated target for quantitative/objective goals.

◇ **Target Active** – Indicates when NIH plans to meet the target.

→ **Target Not Met and Extended** – Indicates that actual performance fell short of the target and that NIH extended the timeframe for meeting the target.

× **Not Met** – Indicates that actual performance fell short of the target and that the target was specific to a particular fiscal year. Therefore, no further action can be taken to achieve the target.

TBD To Be Determined – Indicates that the data needed to determine whether the goal was met are not yet available.

Descriptive Performance Goals

Agencies whose missions include basic and clinical research face unique challenges in developing the objective/quantitative performance goals preferred under GPRA. NIH has concluded that strictly numeric goals and measures are neither feasible nor sufficient to capture the breadth and impact of NIH's Research Program.

As already noted, the GPRA legislation anticipated that such situations could arise for some agencies and provides the "Alternative Form" as a way for an agency to identify performance goals based on criteria that are chiefly descriptive in nature. In such situations, GPRA requires an agency to develop an assessment process that is *systematic* and *independent* and can provide *objective evaluation* of the agency's achievements relative to the stated performance goals.

NIH Use of Descriptive Performance Goals. NIH utilized the qualitative approach for five Research Program outcome goals over fiscal years 1999 through 2001.

- Add to the body of knowledge about normal and abnormal biological functions.
- Develop new or improved instruments and technologies for use in research and medicine.
- Develop new or improved approaches for preventing or delaying the onset or progression of disease and disability.
- Develop new or improved methods for diagnosing disease and disability.
- Develop new or improved approaches for treating disease and disability.

In FY 2002, those five goals were superseded by eight narrower, but also qualitative, goals. Those goals were to:

- Discover innovative approaches for identifying and measuring genetic and environmental factors that contribute to complex diseases across populations.
- Develop model systems (animal models, cell lines, etc.) that will advance our understanding of disease processes.
- Develop new technologies to enable greater understanding of genomic and proteomic information.
- Develop biocompatible materials for use in replacing or repairing damaged and non-functioning or missing tissue.
- Identify modifiable risk factors for disease/disability.
- Identify, develop, and test new/improved medications for the prevention of disease/disability.
- Develop and apply powerful new imaging, genetic, and biological technologies to enable early and more precise diagnosis and intervention.
- Identify and apply knowledge about factors, including gender, race, ethnicity, and socioeconomic status, to improve diagnostic reliability and treatment response.

In the current consolidated FY 2003/2004 Plan and FY 2002 Report, NIH presents a set of representative, specific research outcome goals that will replace the earlier comprehensive, qualitative goals. The extent to which the new goals are qualitative and thus fall under the alternative format will be determined as plans for annual performance reporting on the goals are developed.

The Challenge of Measuring Research Performance. As noted by the President's Office of Science and Technology Policy (OSTP), the Committee on Science, Engineering, and Public Policy of the National Academy of Sciences, and the numerous others who have studied the processes of science, technology, and innovation, the linkages between inputs and outputs in science are complex and non-linear:

- Outcomes are usually very difficult to foresee with any degree of accuracy.
- The full value of any given research finding is usually only barely visible at the time of discovery, and reaches a state of fruition often only after many years or in combination with other advances.
- Moreover, the downstream impact of basic research usually is dependent on substantial further development of new knowledge by private industry, other public sector researchers, or other economic factors.

Scientific advances are generally incremental, and iterative. Each advance builds on previous discoveries and findings frequently redirect a line inquiry back to more basic questions. The importance of a particular discovery may not be apparent immediately. Its significance and impact upon human health and quality of life may become evident only after years of continued research. Accordingly, any assessment process looking at research performance must recognize these difficulties and strive to present an analysis that accounts for the full picture of accomplishment.

Intermediate vs. Ultimate Outcomes. The ultimate outcomes of medical research are, of course, improved health, longevity, and quality of life for all Americans. Each year the NIH can document a number of major medical "culminations" that are visible as practical health benefits, and are often accompanied by economic benefits. For example:

- NIH-supported research recently culminated in the discovery and development of a new drug known as Gleevec. Gleevec is the first anti-cancer drug specifically developed to target a molecular problem that causes a particular type of cancer—chronic myelogenous leukemia (CML). Clinical trials are being conducted to determine the long-term effectiveness of this drug.
- A simple means of diagnosing bladder cancer may become routine based on advances made in other NIH-supported research. Scientists knew that a protein called "survivin" is made by cancer cells and released into urine, but is not made by most normal cells. Based on this and other knowledge, scientists developed a screen for survivin in urine as a sign of cancer. During testing, this diagnostic proved very sensitive, detecting survivin in all of the bladder cancer patients studied. It also gave very few false-positives and was simple and cost-effective.

Nevertheless, the more numerous and immediate outcomes of the Nation's investment in medical research are the incremental findings and accomplishments that increase our knowledge of

fundamental life processes. These "intermediate" advances or "inspirations" provide building blocks for future medical culminations. For example:

- NIH funded researchers recently solved the crystal structure of one of the three toxic proteins produced by anthrax bacteria, lethal factor (LF), and discovered how it interacts with the specific proteins it destroys inside the cell. This structural information can now be used to look for therapeutic agents to block LF's deadly activity.
- With NIH support, researchers sequenced the genome of a virulent strain of *Streptococcus pneumoniae*. *S. pneumoniae* kills millions of people, especially the elderly, worldwide each year with pneumonia, blood stream infections, and meningitis. Many *S. pneumoniae* strains have become resistant to common antibiotics. The examination of this sequence and comparison with other strains should provide targets for the development of new drugs and vaccines.
- While we do not yet know exactly how the human immunodeficiency virus (HIV) causes AIDS, NIH supported scientists have discovered that HIV must attach to cholesterol-rich regions of a cell's membrane in order to enter and infect the cell. The finding provides a more detailed view of how HIV travels into and out of cells and points to possible ways to block that travel—crucial information for continued development and improvement of therapies for patients with AIDS.

None of these intermediate accomplishments directly and/or immediately improve human health. They are, however, essential research steps that enable further work that will lead to improved understanding, diagnosis, treatment, and prevention of human disease and are the expected outcomes of NIH's mission.

Independent Review Process. To assess NIH performance on research outcome goals, goals that encompass the non-linear complex activity of science, NIH drew on the Alternative Form provided under GPRA. The NIH approach has been to annually assess its descriptive research outcome goals in a way that provides an independent and objective account of the agency's science achievements. In brief, an independent review group, impaneled by NIH, examines current information provided by the agency on its recent research achievements and gauges the extent to which NIH research has yielded important discoveries, new knowledge, and improved technologies that can be applied to the development of new or improved diagnostics, treatments, and preventive measures. This review and evaluation is conducted by a working group of the Advisory Committee to the Director (ACD), NIH. The Assessment Working Group is composed of members of the ACD, the Director's Council of Public Representatives (COPR), and members of Institute and Center national advisory councils.

Sources of Data for the Independent Review Process. The principal data inputs to the Assessment Working Group's review/evaluation process are narratives that document and characterize significant research accomplishments that have recently resulted from NIH conducted or funded research. This narrative information places a specific research advance within the context of what was previously known and unknown about the topic; the scientific and/or medical significance of the research area and the accomplishment; potential applications of knowledge from the research, if known; and potential economic implications of the advance, if known. This information provides perspective for where an advance fits in within the

continuum of medical research, and its potential or direct contribution to understanding and improving human health.

This narrative information is of four principal types:

- *Science Advances* are one-page narratives that describe a specific scientific discovery published within the past year that was supported by NIH funding. The background section of the narrative places the advance in the larger context of what is known and unknown, the advance section details the discovery, and the implications section describes the significance of the finding to science, health, and/or the economy.
- *Science Capsules* are snapshots of NIH supported discoveries. The capsules consist of a short paragraph that succinctly describes an advance and its significance, as well as citations. There are obvious limitations to the sheer number of detailed, one-page science advances that Working Group members can be expected to review and assimilate. The collection of science capsules assembled for each descriptive goal facilitates an understanding of the scope of NIH Research Program outcomes.
- *Stories of Discovery* compensate for another major limitation of traditional science advances. The one-page advances address a single, incremental finding, while biomedical progress usually is achieved through long-range investments in research. Progress occurs slowly with one incremental gain in knowledge gradually building upon one another. Stories of discovery are narratives of approximately two pages. Each story traces the major developments in one area over several decades. Important connections between advances in science and improvements in the quality of life, health, and health care, as well as any resulting economic benefits also are highlighted.
- *Research Awards/Honors* demonstrate outside evaluation and recognition of the value of NIH Research Program outcomes. The award write-ups are brief descriptions of national and international scientific awards/honors received by NIH scientists and grantees within the given fiscal year. The brief narratives identify the researcher(s) and the award; describe the work being honored, and the significance/purpose of the award.

Together, these kinds of information provide an extensive, but by no means exhaustive, substantiation of research outcomes with regard to specific goals.

While the Assessment Working Group drew on the narrative material as a source of data for determining performance,[1] the adequacy of performance was judged based on specific assessment criteria developed by the group (see Appendix 4 on Data Verification and Validation). As illustrated below, the conclusions rendered by the Independent Assessment Group were displayed in the Annual Performance Reports using codes similar to those for the quantitative goals.

v **Target Substantially Exceeded** -- Indicates that NIH met certain criteria in addition to those needed to meet the target.

◆ **Target Successfully Met** – Indicates that NIH met criteria developed by an independent Research Assessment Working Group for that target.

[1] The published articles that were the basis of each narrative were not provided as part of the assessment materials, but were provided upon request and at the Working Group meeting.

◇ **Target Active** – Indicates when NIH plans to meet the target and that NIH extended the timeframe for meeting the target.

× **Not Met** – Indicates that actual performance fell short of the target and that the target was specific to a particular fiscal year. Therefore, no further action can be taken to achieve the target.