



Chapter 5
Strategic Goal 4:

Scientific Research and Development

Advance scientific and biomedical research and development related to health and human services.



Disease and injury are constant threats to humankind and are never static. Diseases, such as HIV/AIDS, SARS, pandemic influenza, obesity, and many other conditions can emerge at any time. Twenty years ago, the impact of Alzheimer's disease was not fully appreciated, and its causes were not known. Bioterrorism did not figure prominently in the scientific research and development agenda in 2001, but is now a top priority for numerous HHS divisions, including FDA, NIH, and CDC.

**STRATEGIC GOAL 4:
SCIENTIFIC RESEARCH AND DEVELOPMENT**

Strategic Objective 4.1:

Strengthen the pool of qualified health and behavioral science researchers.

Strategic Objective 4.2:

Increase basic scientific knowledge to improve human health and human development.

Strategic Objective 4.3:

Conduct and oversee applied research to improve health and well-being.

Strategic Objective 4.4:

Communicate and transfer research results into clinical, public health, and human service practice.

As a result of success in preventing and treating acute and short-term conditions such as heart attacks, stroke, cancer, and many infectious diseases, people are living longer. The increasingly older population faces the new challenge of multiple chronic conditions that now consume about 75 percent of health care expenditures. The Nation is in a continuous race against the overwhelming health and economic consequences of disease and human suffering.

Therefore, we must utilize research and development to its maximum capacity to transform health care, public health, and human service practice efforts.

The 21st century is an era of great scientific opportunity. Advances in the understanding of basic human biology allowed NIH to sequence the human genome by 2003, 2 years ahead of schedule, and to complete the haplotype map, showing the variation between individual humans, in October 2005. New advances enable new treatments that could lead to the transformation of medical treatment in this century. The hope is to usher in an era in which medicine will begin to be predictive, personalized, and preemptive. Personalized medicine has the potential to transform health care through earlier diagnosis, more effective prevention and treatment of disease, and avoidance of drug side effects.

Basic science is the foundation for improved health and human services. However, once a basic discovery is made, the findings must be applied and translated into practice for health and human service improvement to result. This continuum from basic and applied research to practice is a significant emphasis of HHS's scientific research and development enterprise.

Strategic Goal 4, *Scientific Research and Development*, seeks to connect this path from basic research to practice through four broad objectives:

- Strengthen the pool of qualified health and behavioral science researchers;
- Increase basic scientific knowledge to improve human health and development;
- Conduct and oversee applied research to improve health and well-being; and
- Communicate and transfer research results into clinical, public health, and human service practice.

A number of HHS operating and staff divisions, including the Agency for Healthcare Research and Quality (AHRQ), Centers for Disease Control and Prevention (CDC), Food and Drug Administration (FDA) and, most significantly, the National Institutes of Health (NIH), sustain and contribute to a full spectrum of scientific research and development activities.

NIH supports and conducts investigations across the full range of the health research continuum, including basic research, which may be disease oriented or related to the development and application of breakthrough technologies; observational and population-based research; behavioral research; prevention research; health services research; translational research¹⁵; and clinical research,¹⁶ as well as research on new treatments or prevention strategies.

FDA supports the research and development goal as a scientific regulatory agency. It is responsible for protecting the public health by assuring the safety, efficacy, and security of human and veterinary drugs, biological products, medical devices, and the Nation's food supply. FDA also ensures the safety of cosmetics and products that emit radiation. FDA advances the public health agenda by helping to speed innovations to market that make medicines more effective and to provide the public accurate, science-based information needed regarding medicines and foods to improve its health. FDA plays a significant role in addressing the Nation's counterterrorism capability and in ensuring the security of the food supply. FDA conducts applied and translational research that enables it to develop regulatory standards and risk assessment criteria to reach sound, science-based public health decisions on regulated products. All of these activities are conducted in collaboration with numerous public and private partners, including academic research institutions; nonprofit foundations; and vaccine, pharmaceutical, and medical device industries.

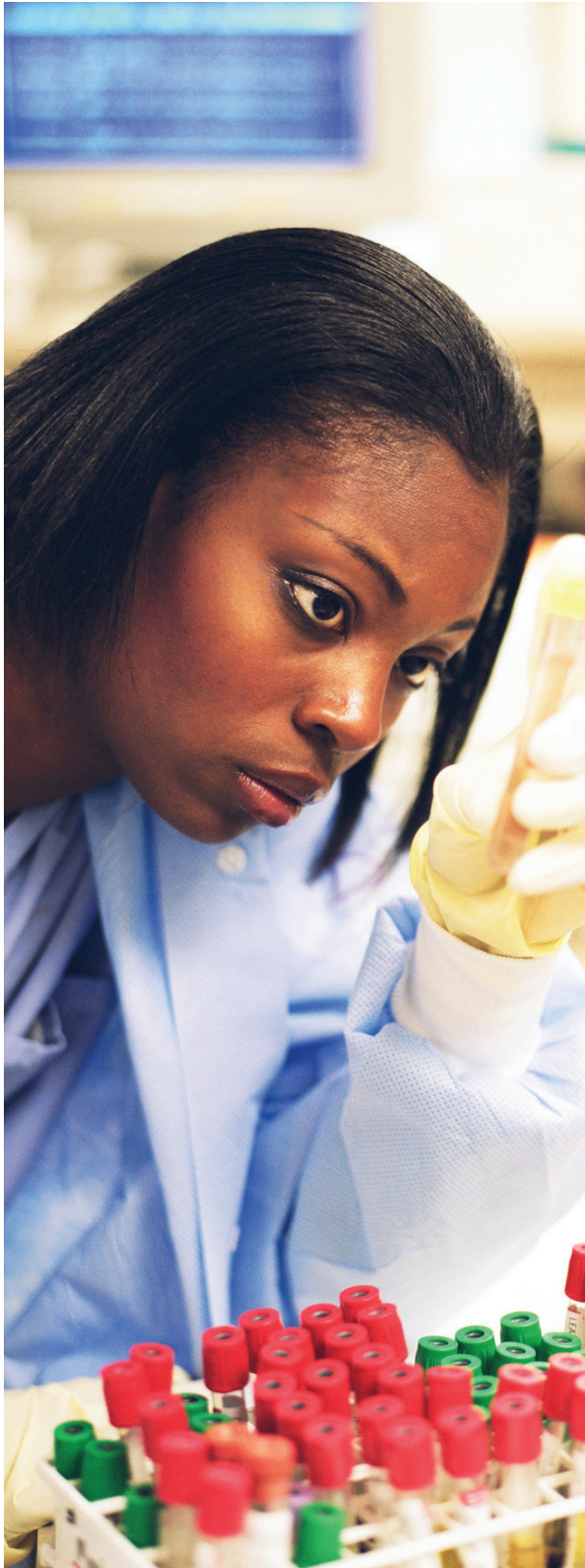
¹⁵ Translational research involves the application of laboratory findings to clinical interventions.

¹⁶ Clinical research includes research to understand both normal health and disease states.

CDC focuses primarily on epidemiological and public health practice research. AHRQ has established a broad base of scientific research and promotes evidence-based improvements in clinical practice and in the organization, financing, and delivery of health care services.

Below is a description of each strategic objective, followed by a description of the key programs, services, and initiatives the Department is undertaking to accomplish those objectives. Although HHS supports a wide array of research and development activities, these represent the major areas of the emphasis for the Department over the next 5 years. Key partners and collaborative efforts are included under each relevant objective. The performance indicators selected for this strategic goal are also presented with baselines and targets. These measures are organized by objective. Finally, this chapter discusses the major external factors that will influence HHS's ability to achieve these objectives, and how the Department is working to mitigate those factors.





Strategic Objective 4.1

Strengthen the pool of qualified health and behavioral science researchers.

The average age of first-time (new) principal investigators of research funded by NIH has risen to 42 years for Ph.D. degree holders and 44 years for M.D. and M.D./Ph.D. holders. This trend must be curtailed in order to capture the creativity and innovation of new independent investigators in their early career stages to address the Nation's health-related research needs.

The National Research Council of the National Academies of Science issued two reports in 2005 about research training and career development with calls for immediate action. NIH will continue to respond to this need to assist and mentor creative young researchers through existing programs. NIH is also developing new initiatives to complement existing efforts to strengthen the pool of qualified health and behavioral science researchers.

NIH will continue to support the *Ruth L. Kruschstein National Research Service Award Research Training Grants and Fellowships Program*. This program provides grant and fellowship funding for individual investigators with or working on a research-related or health-profession doctorate degree. Individual awards promote diversity in health-related research fields across NIH. HHS will use the retention rate of these trainees and fellows as an indicator of its success in improving the pool of qualified researchers. NIH will also support the *Pathway to Independence Award Program*. This program is an innovative and new opportunity for promising postdoctoral scientists to receive both mentored and independent research support from the same award. NIH will also continue to work with IHS to support the *Native American Research Centers for Health* to increase the number of AI/AN researchers.



Strategic Objective 4.2

Increase basic scientific knowledge to improve human health and human development.

Basic research contributes significantly to personalized health care and to increasing understanding of human makeup and biological processes. Current and future basic research projects in HHS focus on those areas with the greatest potential for reduction in excess morbidity and mortality, including brain function, human development, asthma and other respiratory diseases, cancers, dementia, influenza strain mapping, and antimicrobial resistance. The performance indicators for this strategic objective highlight research efforts related to major diseases, including cardiovascular disease and Alzheimer's, and imaging tools for the early detection of diseases, including cancer.

Brain Research

The rising public health impact of disorders of the nervous system makes neuroscience one of the most important scientific frontiers for biomedical and behavioral research in this century. Discoveries in the areas of pain, alcoholism, drug abuse, autism, schizophrenia, depression, and other mental disorders are increasing dramatically. NIH will build on these discoveries by continuing to support research to better understand the processes of the brain, including improving imaging technologies to be able to visualize brain processes as they happen. The increased understanding of the nerve circuits will pave the way for improved diagnosis and treatment of common diseases such as depression, stroke, and epilepsy and reduced burden on the Nation in terms of both suffering and health care costs. NIH will also support the *Autism Phenome Project*, which will identify various clinical characteristics and subtypes of autism to facilitate research on genetic and other potential causes of autism and to guide applied research related to treatment approaches.

Alzheimer's Disease

Alzheimer's disease, the most common cause of dementia among people older than 65, is one of the most serious threats to the Nation's health and economic well-being. Currently, 4.5 million Americans are affected by the disease; that number is expected to almost triple by 2050. Those suffering from Alzheimer's disease advance inexorably, from early, mild forgetfulness to a severe loss of mental function and inability for self-care. Existing research suggests that Alzheimer's disease pathology begins to develop in the brain long before clinical symptoms yield a diagnosis. The ability to make an accurate early diagnosis of Alzheimer's disease would allow targeted intervention before cognitive loss becomes significant. NIH is searching for valid, easily attainable biological markers that could help identify biological markers for early disease. For example, NIH will support research to examine one promising approach that involves using coated gold nanoparticles as bioprobes to measure



the concentrations of substances that correlate with Alzheimer's disease. NIH will also continue to support the *Alzheimer's Disease Neuroimaging Initiative*.

The 5-year, 50-site project represents the most comprehensive effort to date to develop neuroimaging and other biomarkers for the changes associated with mild cognitive impairments and Alzheimer's disease. The ongoing *Genetics Initiative* will also support the development of resources necessary for identifying late-onset risk factor genes, associated environmental factors such as physical activity and diet, and their interactions.

Human Development

NIH is committed to funding a diverse portfolio of basic and translational research that addresses the physical, psychological, psychobiological, language, behavioral, social-emotional, and educational development of children. For example, the National Institute of Child Health and Human Development (NICHD) at NIH has taken a leadership role in advancing scientific knowledge regarding the acquisition of reading and mathematics skills, related learning disabilities, and language development and second language acquisition, as well as child maltreatment, childhood obesity, and the attainment of school readiness skills. Additionally, understanding normative brain development and its relationship to cognitive, social-emotional, and behavioral development is important in finding the causes of myriad childhood disorders related to mental retardation, mental illness, drug abuse, and pediatric neurological diseases, which can continue into adulthood. To define the healthy ranges in brain growth and development patterns in children as they mature, NIH-funded researchers are creating the Nation's first database of Magnetic Resonance Imaging measurement of normal brain development over time in children and adolescents in the United States. NIH is bringing together a diverse array of researchers to design and support a large-scale longitudinal study that uses state-of-the-art brain-imaging technologies and that collects clinical and behavioral data, which will be used to develop analytical software tools. A special effort will be made

to disseminate these data, and as a result, the scientific community will have access to a Web-based, user-friendly resource that integrates neuroanatomical and clinical/behavioral data to examine brain-behavior relationships and relationships between physical maturation and brain development.

Cancer Research

NIH investment in cancer research is helping to make a real difference. In the United States, death rates from all cancers combined dropped 1.1 percent per year from 1991 to 2001.^{xxxviii} Yet cancer remains a major public health problem with more than 1 million Americans per year diagnosed with some form of cancer. Despite significant progress, the cancer challenge remains formidable, and NIH investment in basic cancer research remains critical. NIH will continue to support a broad range of basic research to expand the understanding of cancer. Through the *Cancer Genome Atlas Project*, NIH will expand the capacity of the cancer community to utilize information on cancer genes. NIH also will focus on a growing area of interest—understanding the reaction of the body’s immune system to a developing tumor—because chronic inflammatory immune responses are known to exacerbate certain cancers.

Asthma

NIH supports a comprehensive asthma program to develop new approaches to prevent, treat, and control asthma. Asthma exacerbations cause many of the negative effects of asthma, and management of asthma exacerbations accounts for a large proportion of the estimated annual cost to the Nation’s economy. In contrast to the understanding of the origins of asthma, little is known about the processes that occur during an acute episode; how worsening attacks are resolved; the effect of attacks on future severity and frequency; and the long-term effects on lung physiology, function, and disease progression. In order to develop new interventions to prevent and help resolve acute or worsening asthma episodes, NIH initiated a set of basic, clinical, and translational studies to determine the molecular, cellular, and

genetic causes of asthma exacerbations. The long-term goal is to identify and characterize two molecular pathways of potential clinical significance that may serve as a basis for discovering new medications for preventing and treating the progression of this disease. The studies will address diverse areas including the role of environmental triggers in enhancing airway hyperresponsiveness; the relationship of environmental factors to frequency and severity of asthma attacks; specific effects of initiating events on lung physiology and inflammation; genetic approaches to individual susceptibility for worsening attacks; and the role specific immune and lung cells play in asthma disease classification, chemistry, and physiology.

Pandemic Influenza

HHS is working intensely against influenza. The center of this work is the development of multiple vaccines against influenza virus. At the level of basic science, however, NIH is collaborating with numerous public and private partners on an influenza sequencing project. This project will determine the complete genetic sequences of thousands of influenza virus strains, providing the scientific community with data vital to development of new vaccines, therapies, and diagnostics.

Antimicrobial Resistance

Microbes once easily controlled by antimicrobial drugs are causing infections that no longer respond to treatment with these drugs. In addition, new, serious, and unforeseen infectious disease threats have emerged, including those posed by agents of bioterrorism. Because the existing repertoire of antimicrobial medications may not provide an effective defense against newly emerging and resistant organisms and bioterrorism agents in the future, there is a need to develop new treatments that may be effective against a range of pathogens. NIH is working to develop a *universal antibiotic*, a drug effective against a wide spectrum of infectious diseases, to help address these challenges. NIH also is expanding its capacity for medicinal and combinatorial chemistry, library and database resources, and screening assays for use in identifying novel antimicrobial drugs.



Strategic Objective 4.3

Conduct and oversee applied research to improve health and well-being.

The application of basic scientific findings in the health and human service areas marks the next step along the continuum from basic research to practice. Numerous divisions within HHS conduct and provide oversight of applied research. These activities range from clinical trials for promising new pharmaceuticals and vaccines to behavioral research to identify effective approaches for reducing risky behaviors associated with infectious and chronic diseases. The performance indicators highlight clinical trials focused on improving treatment to those with both cardiovascular disease and diabetes and/or chronic kidney disease.

Birth Defects/Developmental Disabilities

CDC conducts a variety of applied research studies to advance the understanding of factors associated with birth defects and developmental disabilities in both children and adults. Over the next 5 years, CDC will identify and evaluate the role of new factors for birth defects and developmental disabilities. CDC also will initiate new and continue existing studies of candidate risk and protective factors associated with birth defects and developmental disabilities to identify potential intervention strategies.

Substance Abuse Treatment

Although research has demonstrated that substance abuse treatment can be effective in reducing substance use and addiction, few science-based interventions have been developed and tested widely within the health care field. The reasons for this are, in part, related to cultural and institutional barriers. In an effort to narrow the substance abuse treatment gap, recent substance abuse treatment studies have focused on deploying interventions in communities. NIH has adapted and is testing three substance abuse treatment approaches in an effort to bring research-based treatments to communities more rapidly. These substance abuse treatment protocols,



Cardiovascular Disease

To improve the treatment of cardiovascular disease, NIH is working to develop and clinically apply one new imaging technique that will enable tracking the mobility of stem cells within cardiovascular tissues. Scientists are now devoting considerable effort to understanding the role of cytokine¹⁷ production by stem cells rather than focusing solely on assessing their differentiation state and location *in vivo*. Despite the new focus on cytokine production, the importance of understanding stem cell differentiation remains a basic, important problem in regenerative medicine. A promising new approach for assessing differentiation has recently been reported in the literature. Scientists have inserted a reporter of calcium transients into stem cells, allowing scientists to determine whether stem cells are coupled productively to the normal heart during the regeneration process. Control of differentiation will be critical for the eventual success of cardiovascular cell-based therapy. Imaging methods to detect and monitor the differentiation process are now the focus of efforts in numerous laboratories. NIH is undertaking a multimodality imaging effort to develop tools to track cardiovascular stem cells *in vivo*, and ultimately in patients.

Public Health Protection

CDC's applied research portfolio targets four interrelated areas: healthy people in every stage of life, healthy people in healthy places, people prepared for emerging health threats (infectious, occupational, environmental, and terrorist threats), and healthy people in a healthy world. In support of the goals-directed research strategy, CDC has developed the *Advancing the Nation's Health: A Guide to Public Health Research Needs, 2006-2015*. This Research Guide is a comprehensive resource of critical national and global public health research priorities that will advance science and practice toward greater health impact.

¹⁷ Cytokine is a protein secreted by cells of the lymph system that affects the activity of other cells and is important in controlling inflammatory responses.

The array of public health research priorities includes infectious diseases, public health preparedness, chronic diseases and disabilities, safety of environments, global public health, health information and communication, crosscutting research, and health disparities. Over the next 5 years, CDC will progress toward achievement of the health protection goals and will address the research themes in the Research Guide.

Within the infectious disease area, research will focus on antimicrobial resistance; bioterrorism-related environmental microbiology, and zoonotic and vectorborne diseases; vaccines and immunization programs, including vaccine supply issues; and special populations. Within the preparedness area, research will focus on vulnerable populations, including predictive strategies for risk and recovery after an extreme event, infrastructure and workforce preparedness, and detection and diagnosis of hazards and their medical consequences.

The chronic disease applied research portfolio will focus on pregnancy planning and preconception care; optimal child and adolescent development; identification of effective health promotion strategies; and reduction of the burden of, disparities in, and risk factors for, chronic disease among older adults. In order to create safe places to live, work, and play, CDC will conduct research on environmental risk factors such as lead exposure and health, safe workplace design, effective strategies to prevent injuries and violence, and risk and protective factors for interpersonal violence and suicidal behavior.

Food, Drug, and Device Safety

Under its *Critical Path Initiative*, FDA will stimulate and facilitate a national effort to modernize the scientific process through which a potential human drug, biological product, or medical device is transformed from a discovery or “proof of concept” into a medical product. FDA will continue to conduct research on resistance of foodborne pathogens to antimicrobial drugs and to provide for the safe use of antimicrobials in food animals, while ensuring that the usefulness of critical human drugs is not compromised or lost.



FDA's National Center for Toxicological Research (NCTR) will undertake applied research studies that support and anticipate current and future regulatory needs, including integrated toxicological research and support for the *Critical Path Initiative*.



Strategic Objective 4.4

Communicate and transfer research results into clinical, public health, and human service practice.

The implementation of research results in the health care Americans receive every day is the last step of productive research. The performance indicators at the end of this chapter highlight three ongoing efforts to improve prevention efforts among older adults, reduce infant mortality among minorities, and implement evidence-based practices in clinical care.

Community Preventive Services

The *Guide to Community Preventive Services* serves as a filter for scientific literature on specific health problems that can be large, inconsistent, uneven in quality, and even inaccessible. This Community Guide summarizes what is known about the effectiveness, economic efficiency, and feasibility of interventions to promote community health and prevent disease. The *Task Force on Community Preventive Services*, convened by HHS, makes recommendations for the use of various interventions based on the evidence gathered in the rigorous, systematic scientific reviews of published studies conducted by the review teams of the Community Guide. The findings from the reviews are published in peer-reviewed journals and made available on this Internet Web site. HHS will continue to support the Community Guide and will disseminate its systematic review findings via its Web site.

Clinical Preventive Services

The *Guide to Clinical Preventive Services* provides recommendations on screening, counseling, and preventive medication topics and includes clinical considerations for each topic. This new pocket guide provides general practitioners, internists, family practitioners, pediatricians, nurses, and nurse practitioners with an authoritative source for making decisions about preventive services. HHS will continue to support the *Guide to Clinical Preventive Services* and disseminate its systematic review findings via its Web site.

HHS also supports a joint *Clinical Decision Support* program/project planning and coordination effort. This project will provide recommendations and an action plan designed to advance the development, widespread adoption, and value of clinical decision support in improving health and the quality and safety of health care delivery. AHRQ, CMS, IHS, and ONC are collaborators within HHS. The project also includes non-Federal partners, including the Certification Commission for Healthcare Information Technology, Thomas Macromedia, Brigham and Women's Hospital, Partners HealthCare, American Medical Informatics Association, and Oregon Health & Science University.

Dissemination of Findings

AHRQ plays an important role in translational research and dissemination of research findings. AHRQ conducts and supports research on value-based purchasing to help meet these information needs, including articles for the field on how to design, implement, and evaluate value-based purchasing programs. AHRQ's studies and reports will expedite the cycle of research so that purchasers have quicker access to findings on value-based purchasing and will provide guidance on decisionmaking related to value-based purchasing. AHRQ's *Accelerating Change and Transformation in Organization and Networks* program will promote innovation in health care delivery accelerating the development, implementation, dissemination, and uptake of demand-driven and evidence-based products, tools, strategies, and findings.

AHRQ's *Innovations Clearinghouse*, an online searchable database and repository of innovations in health service delivery, will capture effective methods of disseminating and sustaining improvements in the delivery of health care. In addition, the *Innovations Clearinghouse* will serve as a forum for learning about innovation and change. It will provide a national-level, publicly accessible mechanism for obtaining objective, detailed information on health care innovations and tools and will promote their dissemination, replication, adaptation, and use.

Dissemination of Information

HHS also develops and disseminates information and guidelines based on applied research results. For example, NIH continues to develop and disseminate guidance related to Sudden Infant Death Syndrome (SIDS). SIDS, a syndrome of unknown cause, is defined as the sudden death of an infant younger than 1 year of age, which remains unexplained even after a thorough case investigation, autopsy, and review of the clinical history. SIDS is the leading cause of postneonatal mortality in the United States. Led by NIH in collaboration with various sponsors, the *National Back to Sleep* public health education campaign was launched in 1994 after the American Academy of Pediatrics recommended back sleeping as the safest sleep position for infants younger than 1 year of age. Since the launch of the campaign, the SIDS rate has dropped by 50 percent.

However, despite the overall success of the campaign, African-American infants are placed to sleep on their stomachs more often than are White or Caucasian infants. The SIDS rate for African-American infants is two times greater than that of White or Caucasian infants. In collaboration with African-American organizations, NIH has developed comprehensive strategies to reduce SIDS in African-American communities. First, NIH launched a multiyear project to disseminate the American Academy of Pediatrics safe sleep guidelines in Mississippi. The project has multiple components, including training public health workers to convey SIDS risk reduction messages, developing partnerships with State and



local stakeholders, and providing minigrants to community and faith-based organizations to assist with their outreach efforts. Second, a continuing education curriculum has been developed for nurses on safe sleep guidelines and effective ways to convey the risk reduction message. This curriculum will be implemented at regional and national conferences.

Evidence-Based Practices

Several HHS operating divisions support grant programs that facilitate the utilization of evidence-based approaches. SAMHSA's *Strategic Prevention Framework State Incentive Grants*, e.g., require State grantees and their subrecipients to identify their substance use-related problems and to develop and implement evidence-based programs, policies, and practices that have been proven effective in addressing these issues. AoA funds a grant program and public/private partnership to increase older people's access to programs that have proven to be effective in reducing their risk of disease, disability, and injury. The partnership involves a variety of Federal agencies and private foundations that are coordinating their efforts to support the implementation of evidence-based disease prevention programs at the State and community levels.

In addition, the President's Budget for FY 2008 requests \$10 million in new funding under the Child Abuse Prevention and Treatment Act to fund competitive grants. These grants will support the development of a statewide infrastructure to implement, monitor, and sustain high-quality, evidence-based nurse home visitation programs. Funds will be used to support and enhance collaboration and coordination across multiple State and private agencies that already receive Federal or State funding to implement various home visitation models. This new funding will be used for investments in cross-agency collaboration, program development, quality-assurance systems, training, technical assistance, workforce recruitment and retention, evaluation, and other administrative mechanisms needed to successfully implement and sustain high-quality, evidence-based home visitation programs that have strong fidelity to proven effective models.

These programs demonstrate how the results of research from HHS divisions, including NIH, CDC, and AHRQ, can be effectively translated into practice at the community level through service providers. HHS will continue its commitment to infuse evidence into practice through such discretionary programs.

National Registry

SAMHSA supports the *National Registry of Evidence-based Programs and Practices*, a Web-based system designed to disseminate timely and reliable information about interventions that prevent and/or treat mental and substance use disorders. Programs in the Registry have undergone a rigorous review. The Registry provides detailed descriptions of each intervention as well as outcome data.



Performance Indicators

		Most Recent Result	FY 2012 Target
Strategic Objective 4.1			
Strengthen the pool of qualified health and behavioral science researchers.			
4.1.1	<p>Through the National Research Service Award program, increase the probability that scientists continue participation in NIH-funded research within the following ten years:</p> <p>a) Post-doctoral fellows; and b) Pre-doctoral trainees and fellows.</p>	<p>a) 13 percentage points; and b) 13 percentage points.</p>	<p>a) 12+ percentage points; and b) 12+ percentage points.</p>
Strategic Objective 4.2			
Increase basic scientific knowledge to improve human health and human development.			
4.2.1	Develop and apply clinically one new imaging technique to enable tracking the mobility of stem cells within cardiovascular tissues.	Researchers in the NIH intramural program have developed probes that are compatible with optical microscopy techniques developed by intramural scientists.	Develop one new imaging technique that is able to be clinically applied.
4.2.2	Identify at least one clinical intervention that will delay the progression or onset of Alzheimer's disease (AD), or prevent it.	Nearly 1,000 new late-onset AD families have been identified and recruited to the AD Genetics Initiative.	Identify the next generation of compounds for testing in pilot clinical trials.
4.2.3	Develop a novel advanced pattern recognition algorithm to analyze data obtained from imaging technologies to aid clinicians in diagnosing the earliest stage of disease, e.g., brain cancer.	The prototype pattern recognition algorithm has been designed and trained to recognize anomalies in the pilot study of brain Magnetic Resonance Spectroscopic Imaging (MRS) scans.	Apply, in conjunction with a Cooperative Research and Development Agreement (CRADA) partner, a pattern recognition algorithm to identify early biomarkers of brain disease to other disease endpoints in clinical applications such as those used to identify breast cancer markers.

		Most Recent Result	FY 2012 Target
Strategic Objective 4.3			
Conduct and oversee applied research to improve health and well-being.			
4.3.1	Conduct clinical trials to assess the efficacy of at least three new treatment strategies to reduce cardiovascular morbidity/mortality in patients with type 2 diabetes and/or chronic kidney disease.	Initial findings made public at the annual American Diabetes Association meeting in June 2006.	Complete clinical trials and make results available.
Strategic Objective 4.4			
Communicate and transfer research results into clinical, public health, and human service practice.			
4.4.1	Increase the number of AoA-supported community-based sites that use evidence-based disease and disability prevention programs.	27 sites	136 sites
4.4.2	Reduce the disparity between African-Americans infants and White infants in back sleeping by 50% to reduce the risk of Sudden Infant Death Syndrome (SIDS).	The SIDS rate for African-American infants is two times greater than that of White infants.	Reduce disparity by 50%
4.4.3	Reduce the financial cost (or burden) of upper gastrointestinal (GI) hospital admissions by implementing known research findings.	\$93.46 per U.S. resident ages 65 to 85.	10% reduction

Note: Additional information about performance indicators is included in Appendix B.



Meeting External Challenges

Numerous external factors influence the Department's ability to advance its scientific research and development enterprise. The pace and uncertainty of progress in basic and applied research make it difficult to predict how and from where the next important advances will emerge. Additionally, applied research depends on advances in basic biomedical and behavioral research as a precondition of new work, the time often needed for a basic research finding to develop into a public health result, and drug testing time needed to develop animal models and move through the phases of clinical trials successfully.

Pace and Success of Research

In recent years, rapid advances in the biomedical sciences have raised expectations of similar progress in the development of products for the prevention and treatment of serious illnesses. Despite huge strides to decipher the intricacies of human biology, medicine today remains, to an unfortunate degree, an attempt to balance the risks of treatments against their uncertain potential to cure. Physicians earnestly attempting to provide the best treatments, along with their patients—who may be suffering from any of a host of debilitating, even fatal, diseases—are too often left waiting for treatments that are expensive and, ultimately, may not work for them. Compounding these problems

is the fact that the number of new drugs and other treatments approved each year for use in the United States is steadily dropping, in no small part because scientists test new discoveries using outdated and inefficient tools and techniques. The result is a slow, expensive process. It produces fewer and fewer treatments that can be approved as safe and effective, and it leaves consumers on their own to grapple with the question marks of treatment and a short list of prevention options.

One of HHS's primary strategies for reversing this trend is through the FDA *Critical Path Initiative* (CPI). The CPI identifies and prioritizes the most pressing medical product development problems and the greatest opportunities for rapid improvement in public health benefits. The goal is to stimulate the development of powerful new scientific and technical tools—such as proven biomarkers, innovative clinical trial designs, simulation models of physiology and disease processes, and manufacturing quality assessment methods—capable of rapidly predicting the safety, effectiveness, and quality of new medical products. Development of these tools will be based on an understanding of the most successful practices as well as the failures, roadblocks, bottlenecks, and missed opportunities along the way.

Business Interests

Within the research and development sector, business decisions, such as technical capabilities, competing opportunities, interest in the field to develop basic findings into next steps or the next generation of science, economic motivations, public health motivations, and other considerations, significantly influence research and development progress. For example, during the last half-century, pharmaceutical companies have been gradually abandoning the development and manufacture of vaccines. Today, fewer companies are making vaccines because of a number of factors, including the expense involved in bringing vaccines to market and the small size of the vaccine market compared to the larger drug market. There does not appear to be a single reason to explain

the decline in the number of vaccine manufacturers. High-risk research that is critical to biomedical advances must often be initiated by public agencies; because of the high risk of failures, private for-profit groups may be less likely to pursue this type of research. A larger trend in pharmaceutical company mergers and acquisitions, which has seen vaccine-only companies acquired by larger manufacturers, has also contributed to the decreases in the total number of companies making vaccines. In the 1990s, these mergers and acquisitions were largely driven by the need to cut costs. Companies are merging and acquiring other companies in order to secure enough capital and expertise to take advantage of these innovations and, at the same time, cut costs and create efficiencies. The concern remains, however, that leaving the manufacture of all vaccines in the hands of so few producers leaves the supply vulnerable to disruptions and shortages as has been observed in recent years regardless of the total capacity to produce vaccines. HHS is working with manufacturers to identify incentives to promote market reentry and capacity expansion.

Intellectual Property

Intellectual property issues also influence the advancement of science. HHS is working to devise creative incentives to promote the sharing of knowledge among researchers. HHS is also working as a member of the global community to promote knowledge sharing across countries through formal and informal channels.

Recruiting and Retaining Expertise

The scientific labor market is highly competitive. A highly competitive labor market may impact HHS's ability to recruit and retain scientific experts to conduct and oversee research activities and to review applications for medical products. HHS will work to address this challenge by developing training and fellowship programs, as well as partnerships with academia, to enhance the pool of qualified scientists.