



SUMMARY REPORT:

Shaping the Future of Research

A Strategic Plan for the National Heart, Lung, and Blood Institute



U.S. Department of Health and Human Services
National Institutes of Health
National Heart, Lung, and Blood Institute



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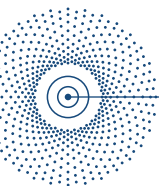


U.S. Department of Health and Human Services
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**National Heart
Lung and Blood Institute**
People Science Health

NIH Publication No. 07-6149
September 2007



Welcome from the Director



I am delighted to present the Strategic Plan of the National Heart, Lung, and Blood Institute. This plan reflects the intellectual energy of over 600 individuals representing an international spectrum of expertise in areas of relevance to the Institute's mission. We are proud of the important role that the Institute has played historically in shaping the prevention and treatment of heart, lung, and blood diseases worldwide. We look forward with great enthusiasm to building upon this tradition and broadening our impact in the years to come. We invite you to join us in this grand adventure.

With best wishes,

A handwritten signature in black ink that reads "Betsy". The signature is written in a cursive, flowing style.

Elizabeth G. Nabel, M.D.
Director



Introduction

The National Heart, Lung, and Blood Institute (NHLBI) provides global leadership for a research, training, and education program to promote the prevention and treatment of heart, lung, and blood diseases and enhance the health of all individuals so that they can live longer and more fulfilling lives.

The breadth of the Institute's programs reflects its mandate, which includes three of the four leading causes of death in the United States. To achieve its vision, the NHLBI stimulates basic discoveries about the causes of disease, speeds the translation of basic discoveries into clinical practice, fosters training and mentoring of emerging scientists and physicians, and communicates research advances to the public. The NHLBI also collaborates with international organizations to help reduce the burden of heart, lung, and blood diseases worldwide.

Heart, lung, and blood research can be expected to change dramatically over the next several decades in response to major drivers of research activity—information technologies that link scientists and their findings globally and instantaneously; accelerating health care costs; an aging population of baby boomers who can expect to live longer, more productive lives, even in the face of chronic diseases; and the development of increasingly sophisticated research tools and databases.

This strategic plan is intended to provide the NHLBI with a guide for its research and training programs over the next 5 to 10 years. It consists of a set of goals that reflects the successive movement of scientific discovery from “form to function,” “function to causes,” and “causes to cures.” The goals focus on questions and processes that are broadly applicable to the Institute's mandate, rather than on any specific disease or condition.

The plan reflects the wisdom, advice, and judgment of more than 600 individuals, including researchers, representatives of patient advocacy groups and professional societies, and other members of the scientific and lay communities. We are indebted to all participants for their commitment to the excellence and productivity of the Institute. Their further participation in the strategic plan will ensure its successful implementation and its continued evolution in response to new challenges and discoveries.



Goal 1: To increase understanding of the molecular and physiological basis of health and disease and use that understanding to improve diagnosis, treatment, and prevention.



Modern science offers outstanding opportunities to probe the innermost workings of the human body and to understand how events at the subcellular and molecular levels influence the functioning and integrity of the individual as a whole. Powerful new research approaches promise to unlock nature's fundamental mysteries and shed light on where healthy processes go awry and how they can be corrected.



Key research questions include the following:

What processes help us maintain health throughout life?

Processes of great interest include the steps involved in organ development; the mechanisms of tissue repair and regeneration; and the roles played by growth factors, intercellular communications, and connective tissue. Research in these areas will facilitate understanding of critical physiological mechanisms that operate from embryonic development to old age and will, thereby, enable progress in the development of cell-based therapeutics.

How do cells receive and process instructions?

Complex networks of biological molecules allow cells to receive and process the instructions needed to carry out normal functions. When the networks are disrupted, disease can occur. Research using new technologies will enhance understanding of how environmental factors, drugs, and even a person's own genes affect how well cellular pathways function. This work should facilitate the identification of approaches that support the proper functioning of cellular pathways as well as the environmental, genetic, and other factors that disrupt them.

What genetic factors account for susceptibility or resistance to disease?

Most common diseases have a complex cause that involves multiple genetic risk factors. Moreover, the severity may vary greatly among affected individuals depending on the presence of genes that either enhance or suppress disease manifestations. New tools are available to enable researchers to identify gene variants and modifiers that affect an individual's risk of developing a particular disease and its range of manifestations as well as the person's response to medications and other interventions—key elements in the development of personalized medicine.

How does the body respond to environmental factors?

Environment in this context includes not just toxic exposures but also diet, physical activity, sleep deprivation, and psychosocial influences, all of which may combine to alter the predisposition for developing a disease, the rapidity and degree of its progression, and the response to therapy. Determining the mechanisms by which such factors disrupt normal biology and interact with genetic susceptibility is an important objective of future research.

Can we differentiate between various subtypes of a disease?

Traditional diagnostic criteria may not reveal subtle differences in the features of a given disease that can have important prognostic or therapeutic implications. Biomarkers—measurable physical, functional, or biochemical indicators—can be used to subdivide related diseases into clinically meaningful classes to achieve early diagnosis, to predict disease progression or regression, and to anticipate the occurrence of desirable or adverse therapeutic outcomes. Moreover, the biomarkers may themselves serve as effective targets for drug development.

Can we find new ways to observe and investigate disease processes in living people?

The same knowledge base that will provide new insights into disease mechanisms and enable identification of potential therapeutic targets will also enable the design of new molecular imaging probes and provide an impetus for the development of new methods to visualize disease. Advances in imaging technology may enhance understanding of the natural history of disease and may lead to more powerful, specific, and timely treatments.



Genetics and Genomics

The NHLBI is investing substantial resources to identify genetic susceptibility factors for common and rare diseases and to uncover ways of applying the new knowledge to develop innovative approaches for diagnosis, prevention, and treatment. For example, the Institute is developing genetic data from long-standing groups of study participants, linking it with data about participants' characteristics and health indicators, and making it available—with appropriate privacy safeguards—to investigators in the community. This is just one step in a sequence of activities that will lead us from gene identification to functional studies and genomic clinical trials and, ultimately, to personalized medicine.





Goal 2: To improve understanding of the clinical mechanisms of disease and thereby enable better prevention, diagnosis, and treatment.



Remarkable progress is being made in understanding the molecular, genetic, and cellular origins of disease, and opportunities now exist to uncover practical uses for this new knowledge, particularly in the realm of personalized medicine. The application of a fundamental understanding of biological processes to the prevention and management of disease requires creative insights by investigators from diverse scientific fields. Further research is needed to identify, measure, and validate targets and pathways that have been detected in basic studies and then to develop new clinical applications and rigorously evaluate their effectiveness and safety.



Key research questions include the following:


Can we repair old body parts or build new ones?

Cell-based therapeutic approaches, including those involving the use of stem cells, offer great promise for treating heart, lung, and blood diseases. However, many questions remain concerning how to select, propagate, and administer cells and, most important, how to ensure that they remain viable and contribute safely to healing and function. Similarly, tissue engineering offers

the possibility of restoring normal function in many diseases through the creation of durable, functional, and biocompatible implants.

Can we harness the potential of genomics, imaging, and nanotechnology to develop new diagnostic and therapeutic strategies?

For example, nanotechnology (engineering on an ultra-small scale) can be expected to play a key role in such areas as drug delivery and therapeutics, molecular imaging, diagnostics and biosensors, and tissue engineering and biomaterials. Although clinical testing of nanoparticles and nanodevices awaits future



developments, less-invasive applications (e.g., diagnostic blood tests) could become available much sooner, if preclinical and clinical studies establish their safety and utility.

Is it possible to detect abnormalities and pathologies long before they cause symptoms and illness?

Many disease processes relevant to the NHLBI mission are known to progress silently over the course of decades. The use of rapidly evolving imaging technologies could shed light on mechanisms of disease initiation, progression, and reversal; detect “subclinical” disease before symptoms develop; and potentially identify targets for effective interventions.

Can we improve our ability to diagnose disease, track its progress, and predict the success of treatments?

Biomarker discovery and validation have already yielded benefits for subgroups of patients identified as being at risk for a wide variety of disorders. Studies to associate genes with disease characteristics are expected to uncover many new biomarkers that may prove useful for evaluating risk and individual responsiveness to treatments in populations and, ultimately, for identifying new therapeutic targets. Such work could potentially transform clinical

decision-making and enhance the participation of patients in health-promoting behaviors.

How can we personalize our approach to disease prevention and treatment?

Personalized medicine is based on the concept that all individuals have unique characteristics as defined by their genome and that human variability in health and disease is determined by genetic makeup in combination with developmental and environmental exposures (e.g., diet, exercise, sleep, psychosocial influences). Exploration of the interactions between genetic and environmental factors has now become essential to explain the development, progression, and outcome of many diseases and to identify the best interventions for a given patient. As the vision of personalized medicine becomes a reality, clinicians will be able to select effective medications and dosages and avoid adverse reactions, thereby improving outcomes and potentially reducing health care costs.

What are “best practices” for the medicine of today and tomorrow?

The NHLBI will continue its long and distinguished tradition of excellence in conducting clinical trials to ensure that clinical practice guidelines for disease detection, management, and prevention are based on rigorous standards of scientific evidence.



Clinical Networks

Since the early 1990s, the NHLBI has been a pioneer in developing networks to support and facilitate clinical research on a variety of diseases, including childhood and adult asthma, pediatric heart disease, heart failure, chronic obstructive pulmonary disease (COPD), thalassemia, and sickle cell disease. Recently established networks are addressing cell therapies, surgical interventions, community-based care, and resuscitation outcomes for patients with cardiovascular diseases. The NHLBI networks enable a timely response to new scientific developments by providing clinical and administrative expertise, access to patients, standardization of treatment protocols, and rapid dissemination of research findings to health care professionals and the public.





Goal 3: To translate research into practice for the benefit of personal and public health.



To realize its public health objective, the NHLBI must find ways to extend the full benefits of scientific advances to all of the diverse populations that constitute the American public. Many evidence-based approaches to prevent and treat heart, lung, and blood diseases have not been uniformly applied in clinical and community practice. Further understanding of the translation process itself is needed to expedite and expand the adoption of biomedical advances into clinical practice and individual health behaviors. Focused behavioral and social science research may help uncover effective new approaches for communicating research findings to the public and for motivating and empowering individuals and communities to take charge of their health.



Key research questions include the following:


How can we move proven therapeutic and preventive approaches into everyday practice?

Integrating behavioral and social sciences research with clinical research is crucial for developing successful strategies to improve medical practice. A better understanding of the factors that influence patient, provider, and health care system

behaviors may facilitate the development of new methods to reduce the “quality gap” between what is currently being done and what can potentially be accomplished. Research is needed to evaluate factors that are associated with care-delivery patterns; to document the benefits, risks, and costs of interventions; and to identify patient-centered approaches to clinical decision-making.

How can we ensure that medical practice is based on up-to-date scientific evidence?

The NHLBI plays a pivotal international role in continually assessing the available



evidence, serving as a knowledge broker for the development of clinical practice guidelines, and identifying areas that need additional research to support clinical decision-making. Systems approaches are needed to speed the implementation of knowledge in health care and community settings; to foster partnerships among practitioners, patients, family members, community organizations, and community health workers; and to create environments that support healthy choices and reduce known risk factors.

How can we reach high-risk communities?

Substantial evidence indicates that health, socioeconomic status, and psychosocial factors are inextricably linked and that disparities in health status are complex problems that demand multifaceted solutions. In particular, new approaches that can accommodate nontraditional family patterns and immigrant status are needed. Community groups and local health providers who share an appreciation of psychosocial issues and cultural beliefs must be involved in efforts to promote community acceptance of science-based health information. Partnerships and linkages will be supported that enhance understanding of the contributions of individual health behaviors, community-based organizational and environmental policies, and health systems innovations to reducing health disparities.

How can we engage individuals as full participants in their health care and disease prevention?

Despite widespread recognition of the importance of health behaviors, only a relatively small percentage of adults and children regularly follow relevant prevention and treatment recommendations. Influences on health behaviors are diverse—they include personal knowledge and motivation, familial expectations and role models, and environmental factors such as workplace and school policies. Accordingly, emphasis should be placed on developing and evaluating approaches within the context of complex real-world environments.

How can we communicate research advances effectively to the public?

The NHLBI will continue to develop public education programs as needed, partnering with professional societies, patient-advocacy groups, community organizations, and Federal entities to ensure that uniform health messages are disseminated. In addition to encouraging individuals and communities in health promotion and disease prevention efforts, the Institute will stress the importance of their involvement in the research process by emphasizing that new health-related information can be generated only with their cooperation and participation.



Prevention

The NHLBI places great emphasis on the translation and dissemination of research findings to health professionals, patients, and the public so that timely information can be integrated into health care practice and individual health behavior. Disease prevention through awareness and the modification of risk factors is a subject that receives considerable attention. For instance, *The Heart Truth* campaign urges women to take steps to lower their risk of developing coronary heart disease. *WeCan!* is helping households and communities around the country to prevent obesity in youngsters and to encourage lifelong habits of healthy eating and exercise.





Next Steps

The NHLBI Strategic Plan provides a broad vision of challenges that the Institute will be addressing over the next few years. A detailed strategy for its implementation will be developed by the Institute in consultation with the National Heart, Lung, and Blood Advisory Council and with other representatives from the research community and the public.

A time-honored way to advance knowledge and explore new horizons is for researchers to apply their expertise and insight to intriguing scientific questions. We anticipate that much of the plan will be realized through such investigator-initiated research, and we are disseminating the plan widely in the research community in the expectation that it will generate highly meritorious proposals.

NHLBI-initiated investments guided by this plan will be directed largely toward programs that either enable or complement investigator-initiated activities. They will call for a variety of strategies to facilitate the conduct of research; enhance interdisciplinary work; speed early-stage translation of basic discoveries; ensure the cross-fertilization of basic, clinical, and epidemiological discoveries; and maximize the resultant public health benefits. The Institute will use the following key strategies in fulfilling the objectives of the plan.

- Develop and facilitate access to scientific research resources.
- Develop new technologies, tools, and resources.

- Increase the return from NHLBI population-based and outcomes research.
- Establish and expand collaborative resources for clinical research.
- Extend the infrastructure for clinical research.
- Support the development of multidisciplinary teams.
- Develop and retain human capital.
- Establish knowledge networks to bridge the gap between research and practice.

The NHLBI also will explore ways to increase its programmatic involvement in global health issues related to chronic diseases in the developing world.

As the challenges identified in this plan are met and as new ones emerge, the NHLBI will identify and embrace new strategies. The Institute will also continue to look to its Advisory Council and to the larger research community for guidance to ensure that the plan continues to reflect the rapidly changing environments of research and public health issues.





NHLBI Resources

NHLBI Home Page

<http://www.nhlbi.nih.gov>

NHLBI Strategic Plan

<http://apps.nhlbi.nih.gov/strategicplan/>

NHLBI Information for Researchers

<http://www.nhlbi.nih.gov/resources/index.htm>

NHLBI Information for Health Professionals

<http://www.nhlbi.nih.gov/health/indexpro.htm>

NHLBI Information for Patients and the Public

<http://www.nhlbi.nih.gov/health/index.htm>

NHLBI Clinical Trial Database

<http://apps.nhlbi.nih.gov/clinicaltrials/>

NHLBI Funding Training and Policies

<http://www.nhlbi.nih.gov/funding/index.htm>

NHLBI Training and Career Development Web Site

<http://www.nhlbi.nih.gov/funding/training/>

NHLBI Research and Policy Update Listserv

<http://www.nhlbi.nih.gov/resources/listserv/index.htm>

NHLBI Fact Book

<http://www.nhlbi.nih.gov/about/factpdf.htm>

NIH Resources

NIH Home Page

<http://www.nih.gov>

NIH Center for Scientific Review

<http://cms.csr.nih.gov/>

NIH Forms and Applications

<http://grants.nih.gov/grants/forms.htm>

NIH Grants and Funding Opportunities

<http://grants1.nih.gov/grants/index.cfm>

NIH Public Involvement

<http://www.nih.gov/about/publicinvolvement.htm>

For More Information

The National Heart, Lung, and Blood Institute (NHLBI) Health Information Center is a service of the NHLBI of the National Institutes of Health. The NHLBI Health Information Center provides information to health professionals, patients, and the public about the treatment, diagnosis, and prevention of heart, lung, and blood diseases and sleep disorders. For more information, contact:

NHLBI Health Information Center

P.O. Box 30105

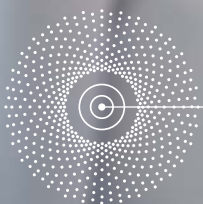
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Discrimination Prohibited

Under provisions of applicable public laws enacted by Congress since 1964, no person in the United States shall, on the grounds of race, color, national origin, handicap, or age, be excluded from participation in, be denied the benefits of, or be subjected to discrimination under any program or activity (or, on the basis of sex, with respect to any education program and activity) receiving Federal financial assistance. In addition, Executive Order 11141 prohibits discrimination on the basis of age by contractors and subcontractors in the performance of Federal contracts, and Executive Order 11246 states that no federally funded contractor may discriminate against any employee or applicant for employment because of race, color, religion, sex, or national origin. Therefore, the National Heart, Lung, and Blood Institute must be operated in compliance with these laws and Executive Orders.



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