Shaping the Future of Research: A Strategic Plan for the National Heart, Lung, and Blood Institute

Executive Summary

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

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. Investigator-initiated research has long constituted the largest share of the NHLBI research portfolio, and it is our intention to maintain that historical commitment. In fact, we expect that much of the plan will be realized through our investment in investigator-initiated research. Institute investments guided by this plan will be directed largely toward programs that either will enable or complement investigator-initiated activities.

The plan consists of a set of goals that reflects the successive movement of scientific discovery from "form to function" (Goal 1), "function to causes" (Goal 2), and "causes to cures" (Goal 3), with research challenges identified for each of the goals and a set of strategies to address the plan as a whole.

The goals are as follows:

Goal 1: To Improve Understanding of the Molecular and Physiological Basis of Health and Disease and To Use That Understanding To Develop Improved Approaches to Disease Diagnosis, Treatment, and Prevention.

<u>Challenge 1.1</u>: To delineate mechanisms that relate molecular events to health and disease.

- 1.1.a. Develop a detailed understanding of the molecular, cellular, and physiological mechanisms that maintain health from embryonic development to the end of the human lifespan.
- 1.1.b. Identify intracellular targets of key signaling and transcriptional pathways in normal and pathological states.
- 1.1.c. Determine key genetic variants that are associated with specific diseases and delineate the molecular mechanisms that account for susceptibility or resistance to disease.
- 1.1.d. Define molecular, cellular, and organ-specific responses to environmental challenges and the mechanisms by which heritable and non-genetic factors interact in disease initiation and progression and in therapeutic response.
- 1.1.e. Determine the role of systemic pathological processes, such as inflammation, immunity, and infection, in the development and evolution of disease.

- <u>Challenge 1.2</u>: To discover biomarkers that differentiate clinically relevant disease subtypes and that identify new molecular targets for application to prevention, diagnosis—including imaging, and therapy.
 - 1.2.a. Identify molecular signatures that allow complex disease phenotypes to be stratified into clinically relevant categories.
 - 1.2.b. Develop *in vivo* molecular imaging methods and probes for investigating the biology of disease processes.

Goal 2: To Improve Understanding of the Clinical Mechanisms of Disease and Thereby Enable Better Prevention, Diagnosis, and Treatment.

- <u>Challenge 2.1</u>: To accelerate the translation of basic research findings into clinical studies and trials and to promote the translation of clinical research findings back to the laboratory.
 - 2.1.a. Integrate advances in regenerative biology to develop clinically feasible applications.
 - 2.1.b. Apply discoveries in nanotechnology to the development of new diagnostic and therapeutic strategies.
 - 2.1.c. Integrate, analyze, and share extant and emerging genotypic and phenotypic data.
- <u>Challenge 2.2</u>: To enable the early and accurate risk stratification and diagnosis of cardiovascular, lung, and blood disorders.
 - 2.2.a. Exploit noninvasive imaging methods to detect and quantify subclinical disease.
 - 2.2.b. Apply new discoveries in biomarkers to improve risk assessment, diagnosis, prognosis, and prediction of response to therapy.
- <u>Challenge 2.3</u>: To develop personalized preventive and therapeutic regimens for cardiovascular, lung, and blood diseases.
 - 2.3.a. Improve the understanding of interactions between genetic and environmental factors that influence disease development and progression and response to therapy.
 - 2.3.b. Identify and evaluate interventions to promote health and treat disease in genetically defined patient subgroups by altering developmental or environmental exposures including drugs, diet and exercise, sleep duration and quality, and infectious agents and allergens.
- <u>Challenge 2.4</u>: To enhance the evidence available to guide the practice of medicine and improve public health.
- Goal 3: To Generate an Improved Understanding of the Processes Involved in Translating Research into Practice and Use That Understanding To Enable Improvements in Public Health and To Stimulate Further Scientific Discovery.
 - <u>Challenge 3.1</u>: To complement bench discoveries and clinical trial results with focused behavioral and social science research.
 - 3.1.a. Develop and evaluate new approaches to implement proven preventive and lifestyle interventions.

- 3.1.b. Develop and evaluate policy, environmental, and other approaches for use in community settings to encourage and support lifestyle changes.
- 3.1.c. Develop and evaluate interventions to improve patient, provider, and health care system behavior and performance in order to enhance quality of care and health outcomes.

<u>Challenge 3.2</u>: To identify cost-effective approaches for prevention, diagnosis, and treatment.

- 3.2.a. Evaluate the risks, benefits, and costs of diagnostic tests and treatments in representative populations and settings.
- 3.2.b. Develop research designs, outcome measures, and analytical methods to assess prevention and treatment programs in community and health care settings across populations and lifespan.

<u>Challenge 3.3</u>: To promote the development and implementation of evidence-based guidelines in partnership with individuals, professional and patient communities, and health care systems and to communicate research advances effectively to the public.

- 3.3.a. Establish evidence-based guidelines for prevention, diagnosis, and treatment and identify gaps in knowledge.
- 3.3.b. Develop personalized and community- and health care system-oriented approaches to increase the use of evidence-based guidelines by individuals, communities, health care providers, public institutions, and, especially, by populations that experience a disproportionate disease burden.
- 3.3.c. Communicate research advances effectively to the public.

The strategies that will be used to address the preceding goals and challenges are listed below:

- Strategy 1: Develop and facilitate access to scientific research resources.
- Strategy 2: Develop new technologies, tools, and resources.
- Strategy 3: Increase the return from NHLBI population-based and outcomes research.
- Strategy 4: Establish and expand collaborative resources for clinical research.
- Strategy 5: Extend the infrastructure for clinical research.
- Strategy 6: Support the development of multidisciplinary teams.
- Strategy 7: Develop and retain human capital.
- Strategy 8: Bridge the gap between research and practice through knowledge networks.